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

Form 10-Q

(Mark One)

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

For the quarterly period ended September 30, 2020

OR

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

Commission File Number: 001-33038

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

Delaware	**84-1475642**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**

One First Avenue, Parris Building 34, Navy Yard Plaza
Boston, Massachusetts 02129
(617) 259-1970
(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

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	**ZIOP**	**The Nasdaq Stock Market LLC**

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

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T 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 the 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 October 29, 2020, the number of outstanding shares of the registrant's common stock, $0.001 par value, was 214,291,057 shares.

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This Quarterly Report on Form 10-Q contains forward-looking statements that are based on our current beliefs and expectations. These forward-looking statements may be accompanied by such words as “anticipate,” “believe,” “estimate,” “expect,” “forecast,” “intend,” “may,” “plan,” “project,” “target,” “will” and other words and terms of similar meaning, although not all forward-looking statements contain these identifying words. These statements involve known and unknown risks, uncertainties and other factors that may cause our actual results, levels of activity, performance or achievements to be materially different from the information expressed or implied by these forward-looking statements. Although we believe that we have a reasonable basis for each forward-looking statement contained in this Quarterly Report, we caution you that these statements are based on a combination of facts and factors currently known by us and our expectations of the future, about which we cannot be certain.

The forward-looking statements in this Quarterly Report include, but are not limited to, statements about:

- our ability to raise substantial additional capital to fund our planned operations in the near term;
- estimates regarding our expenses, use of cash, timing of future cash needs and anticipated capital requirements;
- the development of our product candidates, including statements regarding the initiation, timing, progress and results of our preclinical clinical studies, clinical trials and research and development programs;
- our ability to advance our product candidates through various stages of development, especially through pivotal safety and efficacy trials;
- the risk that final trial data may not support interim analysis of the viability of our product candidates;
- our expectation regarding the safety and efficacy of our product candidates;
- the timing, scope or likelihood of regulatory filings and approvals from the U.S. Food and Drug Administration or equivalent foreign regulatory agencies for our product candidates and for which indications;
- our ability to license additional intellectual property relating to our product candidates from third parties and to comply with our existing license agreements;
- our ability to enter into partnerships or strategic collaboration agreements, our ability to achieve the results contemplated and the potential benefits to be derived from relationships with collaborators;
- our ability to maintain and establish collaborations and licenses; developments and projections relating to competition from other pharmaceutical and biotechnology companies or our industry;
- our estimates regarding the potential market opportunity for our product candidates;
- the anticipated rate and degree of commercial scope and potential, as well as market acceptance of our product candidates for any indication, if approved;
- the anticipated amount, timing and accounting of contract liability (formerly deferred revenue), milestones and other payments under licensing, collaboration or acquisition agreements, research and development costs and other expenses;
- our intellectual property position, including the strength and enforceability of our intellectual property rights;
- our ability to attract and retain qualified employees and key personnel;
- the impact of government laws and regulations in the United States and foreign countries;
- our expectations regarding the impact of the ongoing coronavirus disease 2019, or COVID-19, pandemic, included the expected duration of disruption and immediate and long-term impact and effect on our business and operations;
- the diversion of healthcare resources away from the conduct of clinical trials as a result of the ongoing COVID-19 pandemic, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;

- the interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel, quarantines or social distancing protocols imposed or recommended by federal or state governments, employers and others in connection with the ongoing COVID-19 pandemic; and
- other risks and uncertainties, including those listed under Part II, Item 1A, “Risk Factors”.

Any forward-looking statements in this Quarterly Report on Form 10-Q reflect our current views with respect to future events or to our future financial performance and involve known and unknown risks, uncertainties and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. Factors that may cause actual results to differ materially from current expectations include, among other things, those listed under Part II, Item 1A. “Risk Factors” and elsewhere in this Quarterly Report on Form 10-Q. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

NOTE REGARDING COMPANY REFERENCES

Throughout this Quarterly Report on Form 10-Q, “Ziopharm,” the “Company,” “we,” “us” and “our” refer to ZIOPHARM Oncology, Inc. and its subsidiaries.

NOTE REGARDING TRADEMARKS

All trademarks, trade names and service marks appearing in this Quarterly Report on Form 10-Q are the property of their respective owners.

ZIOPHARM Oncology, Inc.

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Part I - Financial Information

Item 1. Financial Statements

ZIOPHARM Oncology, Inc.

BALANCE SHEETS
(unaudited)

(in thousands, except share and per share data)

	September 30, 2020	December 31, 2019
ASSETS		
Current assets:		
Cash and cash equivalents	$ 135,471	$ 79,741
Receivables	5,428	3,330
Prepaid expenses and other current assets	14,172	22,421
Total current assets	155,071	105,492
Property and equipment, net	7,577	1,110
Right of use asset	2,370	2,272
Deposits	130	130
Other non-current assets	746	110
Total assets	$ 165,894	$ 109,114
LIABILITIES AND STOCKHOLDERS' EQUITY		
Current liabilities:		
Accounts payable	$ 2,619	$ 906
Accrued expenses	15,836	10,846
Lease liability - current portion	866	774
Total current liabilities	19,321	12,526
Lease liability - noncurrent portion	1,655	1,578
Total liabilities	20,976	14,104
Commitments and contingencies (Note 6)		
Stockholders' equity:		
Common stock, $0.001 par value; 250,000,000 shares authorized; 214,165,690 and 181,803,320 shares issued and outstanding at September 30, 2020 and December 31, 2019, respectively	214	182
Additional paid-in capital	886,033	778,953
Accumulated deficit	(741,329)	(684,125)
Total stockholders' equity	144,918	95,010
Total liabilities and stockholders' equity	$ 165,894	$ 109,114

The accompanying notes are an integral part of the unaudited interim financial statements.

ZIOPHARM Oncology, Inc.

STATEMENTS OF OPERATIONS
(unaudited)

(in thousands, except share and per share data)

	For the Three Months Ended September 30,		For the Nine Months Ended September 30,	
	2020	2019	2020	2019
Operating expenses:				
Research and development	$ 13,968	$ 8,641	$ 38,725	$ 28,115
General and administrative	6,353	4,807	18,862	13,707
Total operating expenses	20,321	13,448	57,587	41,822
Loss from operations	(20,321)	(13,448)	(57,587)	(41,822)
Other income, net	6	203	383	523
Non-cash inducement warrant expense	—	(60,751)	—	(60,751)
Net loss	$ (20,315)	$ (73,996)	$ (57,204)	$ (102,050)
Basic and diluted net loss per share	$ (0.10)	$ (0.43)	$ (0.27)	$ (0.62)
Weighted average common shares outstanding used to compute basic and diluted net loss per share	212,837,367	170,613,712	208,497,410	164,053,029

The accompanying notes are an integral part of the unaudited interim financial statements.

ZIOPHARM Oncology, Inc.

STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY
For the Three and Nine Months Ended September 30, 2020
(unaudited)

(in thousands, except share and per share data)

For the Three Months Ended September 30, 2020

	Common Stock		Additional Paid In Capital Common Stock	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount			
Balance at June 30, 2020	214,150,940	$ 214	$ 884,214	$ (721,014)	163,414
Stock-based compensation	—	—	1,792	—	1,792
Exercise of employee stock options	14,750	—	27	—	27
Net loss	—	—	—	(20,315)	(20,315)
Balance at September 30, 2020	214,165,690	$ 214	$ 886,033	$ (741,329)	$ 144,918

For the Nine Months Ended September 30, 2020

	Common Stock		Additional Paid In Capital Common Stock	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount			
Balance at December 31, 2019	181,803,320	$ 182	$ 778,953	$ (684,125)	$ 95,010
Stock-based compensation	—	—	5,393	—	5,393
Exercise of employee stock options	22,916	—	43	—	43
Restricted stock awards	555,900	1	(1)	—	—
Cancelled restricted common stock	(141,230)	—	—	—	—
Issuance of common stock in connection with a public offering, net of commissions and expenses of $5.9 million	29,110,111	29	88,632	—	88,661
Issuance of common stock in connection with an at the market offering, net of commissions and expenses of $0.4 million	2,814,673	2	13,013	—	13,015
Net loss	—	—	—	(57,204)	(57,204)
Balance at September 30, 2020	214,165,690	$ 214	$ 886,033	$ (741,329)	$ 144,918

The accompanying notes are an integral part of the unaudited interim financial statements.

ZIOPHARM Oncology, Inc.

STATEMENTS OF CHANGES IN STOCKHOLDERS' EQUITY…continued
For the Three and Nine Months Ended September 30, 2019
(unaudited)

(in thousands, except share and per share data)

For the Three Months Ended September 30, 2019

	Common Stock		Additional Paid In Capital Common Stock	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount			
Balance at June 30, 2019	162,477,963	$ 162	$ 656,216	$ (594,383)	$ 61,995
Stock-based compensation	—	—	1,486	—	1,486
Exercise of employee stock options	94,584	—	420	—	420
Restricted stock awards	15,000	—	—	—	—
Issuance of common stock upon exercise of warrants, net	17,803,031	18	52,481	—	52,499
Issuance of inducement warrants	—	—	60,751	—	60,751
Issuance of common stock in connection with at the market offering, net	639,442	1	2,970	—	2,971
Net loss	—	—	—	(73,996)	(73,996)
Balance at September 30, 2019	181,030,020	$ 181	$ 774,324	$ (668,379)	$ 106,126

For the Nine Months Ended September 30, 2019

	Common Stock		Additional Paid In Capital Common Stock	Accumulated Deficit	Total Stockholders' Deficit
	Shares	Amount			
Balance at December 31, 2018	161,066,136	$ 161	$ 651,732	$ (566,329)	$ 85,564
Stock-based compensation	—	—	4,741	—	4,741
Exercise of employee stock options	352,652	—	1,020	—	1,020
Restricted stock awards	1,408,536	1	999	—	1,000
Issuance of common stock upon exercise of warrants, net	17,803,031	18	52,481	—	52,499
Issuance of inducement warrants	—		60,751	—	60,751
Cancelled restricted common stock	(74,599)	—	—	—	—
Restricted stock buy-back at vesting to cover taxes	(165,178)	—	(370)	—	(370)
Issuance of common stock in connection with at the market offering, net	639,442	1	2,970	—	2,971
Net loss	—	—	—	(102,050)	(102,050)
Balance at September 30, 2019	181,030,020	$ 181	$ 774,324	$ (668,379)	$ 106,126

The accompanying notes are an integral part of the unaudited interim financial statements.

ZIOPHARM Oncology, Inc.

STATEMENTS OF CASH FLOWS
(unaudited)

(in thousands)

	For the Nine Months Ended September 30, 2020	2019
Cash flows from operating activities:		
Net loss	$ (57,204)	$ (102,050)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation	708	453
Stock-based compensation	5,393	5,741
Non-cash inducement warrant expense	—	60,751
Change in operating assets and liabilities		
(Increase) decrease in:		
Receivables	(2,098)	(1,577)
Prepaid expenses and other current assets	8,249	(3,047)
Right of use asset	(98)	—
Deposits	—	(2)
Other noncurrent assets	(636)	9,432
Increase (decrease) in:		
Accounts payable	1,713	219
Accrued expenses	3,828	810
Deferred rent	—	17
Lease liabilities	168	7
Net cash used in operating activities	(39,977)	(29,246)
Cash flows from investing activities:		
Purchases of property and equipment	(6,012)	(184)
Net cash used in investing activities	(6,012)	(184)
Cash flows from financing activities:		
Repurchase of common stock	—	(370)
Proceeds from exercise of stock options	43	1,020
Issuance of common stock in connection with a public offering, net	88,661	2,971
Issuance of common stock in connection with at the market offerings, net	13,015	52,499
Net cash provided by financing activities	101,719	56,120
Net increase in cash and cash equivalents, and restricted cash	55,730	26,690
Cash and cash equivalents, and restricted cash, beginning of period	79,741	61,729
Cash and cash equivalents, and restricted cash, end of period	$ 135,471	$ 88,419
Supplementary disclosure of cash flow information:		
Compensation paid in restricted stock, gross	$ —	$ 1,000
Accounts included in accounts payable and accrued expenses related to property and equipment	$ 1,163	$ —

The accompanying notes are an integral part of the unaudited interim financial statements.

ZIOPHARM Oncology, Inc.

NOTES TO FINANCIAL STATEMENTS
(unaudited)

1. Business

Overview

ZIOPHARM Oncology, Inc., which is referred to herein as "ZIOPHARM," or the "Company," is a biopharmaceutical company seeking to develop, acquire, and commercialize, on its own or with partners, a diverse portfolio of immuno-oncology therapies.

The Company's operations to date have consisted primarily of conducting research and development and raising capital to fund those efforts. The Company's fiscal year ends on December 31.

The Company has operated at a loss since its inception in 2003 and has no recurring revenues from operations. The Company anticipates that losses will continue for the foreseeable future. As of September 30, 2020, the Company had approximately $135.5 million of cash and cash equivalents and the Company's accumulated deficit was approximately $741.3 million. Given its current development plans, the Company anticipates cash resources will be sufficient to fund operations into mid-2022. The Company's ability to continue operations after its current cash resources are exhausted depends on its ability to obtain additional financing or to achieve profitable operations, as to which no assurances can be given. Cash requirements may vary materially from those now planned because of changes in the Company's focus and direction of its research and development programs, competitive and technical advances, patent developments, regulatory changes or other developments. If adequate additional funds are not available when required, or if the Company is unsuccessful in entering into partnership agreements for further development of its product candidates, management may need to curtail its development efforts and planned operations to conserve cash.

Basis of Presentation

The accompanying unaudited interim financial statements have been prepared in accordance with the instructions to Form 10-Q pursuant to the rules and regulations of the Securities and Exchange Commission, or the SEC. Certain information and note disclosures required by generally accepted accounting principles in the United States have been condensed or omitted pursuant to such rules and regulations.

It is management's opinion that the accompanying unaudited interim financial statements reflect all adjustments (which are normal and recurring) that are necessary for a fair statement of the results for the interim periods. The unaudited interim financial statements should be read in conjunction with the audited financial statements and the notes thereto for the year ended December 31, 2019, included in the Company's Annual Report on Form 10-K for the fiscal year ended December 31, 2019 filed with the SEC on March 2, 2020, or the Annual Report.

The year-end balance sheet data was derived from the audited financial statements but does not include all disclosures required by generally accepted accounting principles in the United States.

The results disclosed in the statements of operations for the three and nine months ended September 30, 2020 are not necessarily indicative of the results to be expected for the full fiscal year 2020.

ZIOPHARM Oncology, Inc.

NOTES TO FINANCIAL STATEMENTS (unaudited)

1. Business (Continued)

Use of Estimates

The preparation of financial statements in conformity with accounting principles generally accepted in the United States requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and the disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of revenues and expenses during the reporting period. Although the Company regularly assesses these estimates, actual results could differ from those estimates. Changes in estimates are recorded in the period in which they become known.

The Company's most significant estimates and judgments used in the preparation of its financial statements are:

- Clinical trial expenses;
- Collaboration agreements;
- Fair value measurements of stock-based compensation; and
- Income taxes

Impact of COVID-19 Pandemic

With the global spread of the ongoing COVID-19 pandemic, the Company has implemented business continuity plans designed to address and mitigate the impact of the COVID-19 pandemic on its business and operations. The extent to which the COVID-19 pandemic impacts the Company's business, clinical development and regulatory efforts and the value of its common stock, will depend on future developments that are highly uncertain and cannot be predicted with confidence at this time, such as the ultimate duration of the pandemic, travel restrictions, quarantines, social distancing and business closure requirements, and the effectiveness of actions taken globally to contain and treat the disease. The global economic slowdown, the overall disruption of global healthcare systems and the other risks and uncertainties associated with the COVID-19 pandemic could have a material adverse effect on the Company's business, financial condition, results of operations and growth prospects.

In addition, the Company is subject to other challenges and risks specific to its business and its ability to execute on its business plan and strategy, as well as risks and uncertainties common to companies in the biopharmaceutical industry with development and commercial operations, including, without limitation, risks and uncertainties associated with: obtaining regulatory approval of its product candidates; delays or problems in obtaining clinical supply, loss of single source suppliers or failure to comply with manufacturing regulations; identifying, acquiring or in-licensing additional products or product candidates; biopharmaceutical product development and the inherent uncertainty of clinical success; and the challenges of complying with applicable regulatory requirements. In addition, to the extent the ongoing COVID-19 pandemic adversely affects its business and results of operations, it may also have the effect of heightening many of the other risks and uncertainties discussed above.

Subsequent Events

The Company evaluated all events and transactions that occurred after the balance sheet date through the date of this filing. During this period, the Company did not have any material subsequent events that impacted its financial statements or disclosures.

ZIOPHARM Oncology, Inc.

NOTES TO FINANCIAL STATEMENTS (unaudited)

2. Financings

February 2020 Public Offering

On February 5, 2020, the Company entered into an underwriting agreement with Jefferies, as representative of the several underwriters named therein, relating to the issuance and sale of 27,826,086 shares of its common stock. The price to the public in the offering was $3.25 per share, and the underwriters agreed to purchase the shares from the Company pursuant to the underwriting agreement at a purchase price of $3.055 per share. Under the terms of the underwriting agreement, the Company also granted the underwriters an option, exercisable for 30 days, to purchase up to an additional 4,173,912 shares of common stock at a purchase price of $3.055 per share.

The offering was made pursuant to the Company's effective registration statement on Form S-3ASR (File No. 333-232283) previously filed with the SEC, and a prospectus supplement thereunder. The underwriters purchased the 27,826,086 shares on February 5, 2020. The net proceeds from the offering were approximately $84.8 million after deducting underwriting discounts and offering expenses paid by the Company.

On March 10, 2020, the underwriters exercised their option to purchase an additional 1,284,025 shares. The net proceeds were approximately $3.9 million after deducting underwriting discounts and offering expenses paid by the Company.

At-the-market Offering

In June 2019, the Company entered into an Open Market Sale Agreement, or sales agreement, with Jefferies LLC, as agent, or ("Jefferies"), pursuant to which the Company may offer and sell, from time to time through Jefferies, shares of its common stock having an aggregate offering price of up to $100.0 million. Shares will be sold pursuant to the Company's effective registration statement on Form S-3ASR (File No. 333-232283), as previously filed with the SEC.

During the nine months ended September 30, 2019, the Company sold an aggregate of 639,442 shares of its common stock. The offering was made pursuant to the Company's effective registration statement on Form S-3ASR (Registration Statement No. 333-232283) previously filed with the SEC, and a prospectus supplement thereunder. The net proceeds from the offering were approximately $3.0 million after deducting underwriting discounts and estimated offering expenses payable by the Company.

During the three months ended September 30, 2020, there were no at-the-market sales. During the nine months ended September 30, 2020, the Company issued and sold an aggregate of 2,814,673 shares of its common stock under the sales agreement for aggregate net proceeds of $13.0 million after deducting commissions and offering expenses.

ZIOPHARM Oncology, Inc.

NOTES TO FINANCIAL STATEMENTS (unaudited)

2. Financings (Continued)

November 2018 Private Placement and 2019 Inducement Warrants

On November 11, 2018, the Company entered into a securities purchase agreement with certain institutional and accredited investors pursuant to which it sold an aggregate of 18,939,394 immediately separable units at a price per unit of $2.64 to such investors, for net proceeds of approximately $47.1 million. Each unit was comprised of (i) one share of our common stock, par value $0.001 per share and (ii) a warrant to purchase one share of common stock. The securities issued by the Company pursuant to the securities purchase agreement and to be issued upon exercise of the warrants were not registered under the Securities Act and may not be offered or sold in the United States absent registration or an applicable exemption from registration requirements. When issuing the units, the Company relied on the private placement exemption from registration provided by Section 4(a)(2) of the Securities Act and by Rule 506 of Registration D, promulgated thereunder and on similar exemptions under applicable state laws and filed a Form D with the SEC on November 19, 2018. On February 7, 2019, the Company filed a registration statement on Form S-3 registering the resale of shares issued pursuant to the securities purchase agreement and the resale of shares that may be issued upon exercise of the warrants.

On July 26, 2019 and September 12, 2019, the Company entered into agreements for the exercise of the warrants issued in November 2018 to purchase common stock in a private placement. Pursuant to the terms of the agreements, investors exercised warrants for an aggregate of 17,803,031 shares of common stock, at an exercise price of $3.01 per share. The Company issued new warrants to purchase up to 17,803,031 additional shares of common stock as an inducement for warrant holders to exercise their 2018 warrants early. The new warrants will become exercisable six months following the date of issuance, will expire on the fifth anniversary of the initial exercise date, and have an exercise price of $7.00 (Note 9). Proceeds from the exercise of the warrants, before deducting placement agent fees and other related expenses of $1.1 million were approximately $52.5 million.

3. Summary of Significant Accounting Policies

The Company's significant accounting policies were identified in the Company's Annual Report. There have been no material changes in those policies since the filing of its Annual Report except as noted below.

New Accounting Pronouncements

In December 2019, the FASB issued ASU 2019-12, *Income Taxes (Topic 740): Simplifying the Accounting for Income Taxes*, which is intended to simplify various aspects related to accounting for income taxes. ASU 2019-12 removes certain exceptions to the general principles in ASC 740 and also clarifies and amends existing guidance to improve consistent application. This guidance is effective for public entities for fiscal years beginning after December 15, 2020, and for interim periods within those fiscal years. The Company is currently evaluating the impact of this new guidance on its consolidated financial statements.

In August 2018, the FASB issued ASU No. 2018-13, *Fair Value Measurement (Topic 820): Disclosure Framework—Changes to the Disclosure Requirements for Fair Value Measurement*, or ASU 2018-03. The guidance in this ASU modify the disclosure requirements on fair value measurements in Topic 820, Fair Value Measurement. Under the new guidance, transfers between asset classes and the valuation related to Level 3 assets is modified. The new standard is effective for annual reporting periods beginning after December 15, 2019, including interim reporting periods within each annual reporting period. The adoption did not have a material impact on the Company's financial statements.

ZIOPHARM Oncology, Inc.

NOTES TO FINANCIAL STATEMENTS (unaudited)

4. Fair Value Measurements

The Company accounts for its financial assets and liabilities using fair value measurements. The authoritative accounting guidance defines fair value, establishes a framework for measuring fair value under generally accepted accounting principles and enhances disclosures about fair value measurements. Fair value is defined as the exchange price that would be received for an asset or paid to transfer a liability (an exit price) in the principal or most advantageous market for the asset or liability in an orderly transaction between market participants on the measurement date. Valuation techniques used to measure fair value must maximize the use of observable inputs and minimize the use of unobservable inputs. The fair value hierarchy is based on three levels of inputs, of which the first two are considered observable and the last unobservable, that may be used to measure fair value as follows:

- Level 1 - Quoted prices in active markets for identical assets or liabilities.
- Level 2 - Inputs other than Level 1 that are observable, either directly or indirectly, such as quoted prices for similar assets or liabilities; quoted prices in markets that are not active; or other inputs that are observable or can be corroborated by observable market data for substantially the full term of the assets or liabilities.
- Level 3 - Unobservable inputs that are supported by little or no market activity and that are significant to the fair value of the assets or liabilities.

Assets and liabilities measured at fair value on a recurring basis as of September 30, 2020 and December 31, 2019 were as follows:

($ in thousands)		Fair Value Measurements at Reporting Date Using		
Description	Balance as of September 30, 2020	Quoted Prices in Active Markets for Identical Assets/Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Assets:				
Cash equivalents	$ 75,300	$ 75,300	$ —	$ —

($ in thousands)		Fair Value Measurements at Reporting Date Using		
Description	Balance as of December 31, 2019	Quoted Prices in Active Markets for Identical Assets/Liabilities (Level 1)	Significant Other Observable Inputs (Level 2)	Significant Unobservable Inputs (Level 3)
Assets:				
Cash equivalents	$ 68,031	$ 68,031	$ —	$ —

ZIOPHARM Oncology, Inc.

NOTES TO FINANCIAL STATEMENTS (unaudited)

4. Fair Value Measurements (Continued)

The cash equivalents represent deposits in short-term United States treasury money market mutual funds quoted in an active market and classified as a Level 1 asset.

5. Net Loss per Share

Basic net loss per share is computed by dividing net loss by the weighted average number of shares of common stock outstanding for the period. The Company's potentially dilutive shares, which include outstanding common stock options, unvested restricted stock and preferred stock, have not been included in the computation of diluted net loss per share for any of the periods presented as the result would be anti-dilutive. Such potentially dilutive shares of common stock at September 30, 2020 and 2019 consisted of the following:

	September 30,	
	2020	2019
Stock options	6,572,191	4,995,549
Inducement stock options	863,333	965,000
Unvested restricted stock	1,289,389	1,569,579
Warrants	22,272,727	18,939,394
	30,997,640	26,469,522

6. Commitments and Contingencies

License Agreements

Exclusive License Agreement with PGEN Therapeutics

On October 5, 2018, the Company entered into the License Agreement, with PGEN Therapeutics, Inc. or PGEN, a wholly owned subsidiary of Precigen Inc., or Precigen, which was formerly known as Intrexon Corporation. As between the Company and PGEN, the terms of the License Agreement replace and supersede the terms of: (a) that certain Exclusive Channel Partner Agreement by and between the Company and Precigen, dated January 6, 2011, as amended by the First Amendment to Exclusive Channel Partner Agreement effective September 13, 2011, the Second Amendment to the Exclusive Channel Partner Agreement effective March 27, 2015, and the Third Amendment to Exclusive Channel Partner Agreement effective June 29, 2016, which was subsequently assigned by Precigen to PGEN; (b) certain rights and obligations pursuant to that certain License and Collaboration Agreement effective March 27, 2015 between ZIOPHARM, Precigen and ARES TRADING S.A., or Ares Trading, a subsidiary of Merck KGaA, or Merck, as assigned by Precigen to PGEN, or the Ares Trading Agreement; (c) that certain License Agreement between the Company, Precigen, and MD Anderson, with an effective date of January 13, 2015, or the MD Anderson License, which was subsequently assigned by Precigen and assumed by PGEN effective as of January 1, 2018; and (d) that certain Research and Development Agreement between the Company, Precigen and MD Anderson with an effective date of August 17, 2015, or the Research and Development Agreement, and any amendments or statements of work thereto.

ZIOPHARM Oncology, Inc.

NOTES TO FINANCIAL STATEMENTS (unaudited)

6. Commitments and Contingencies (Continued)

Pursuant to the terms of the License Agreement, PGEN has granted the Company exclusive, worldwide rights to research, develop and commercialize (i) products utilizing PGEN's RheoSwitch® gene switch, or RTS®, for the treatment of cancer, referred to as IL-12 Products, (ii) CAR products directed to (A) CD19 for the treatment of cancer, referred to as CD19 Products, and (B) a second target for the treatment of cancer, subject to the rights of Ares Trading to pursue such target under the Ares Trading Agreement, and (iii) T-cell receptor, or TCR, products designed for neoantigens for the treatment of cancer. PGEN has also granted the Company an exclusive, worldwide, royalty-bearing, sub-licensable license for certain patents relating to the *Sleeping Beauty* technology to research, develop and commercialize TCR products for both neoantigens and shared antigens for the treatment of cancer, referred to as TCR Products.

The Company is solely responsible for all aspects of the research, development and commercialization of the exclusively licensed products for the treatment of cancer. The Company is required to use commercially reasonable efforts to develop and commercialize IL-12 Products, CD19 Products and TCR Products.

In consideration of the licenses and other rights granted by PGEN, the Company pays PGEN an annual license fee of $0.1 million. As of September 30, 2020, the Company had $0.1 million in prepaid expenses and other current assets. The Company did not have any annual license expenses for the three and nine months ended September 30, 2020 and 2019.

The Company will also make milestone payments totaling up to an additional $52.5 million for each exclusively licensed program upon the initiation of later stage clinical trials and upon the approval of exclusively licensed products in various jurisdictions. In addition, the Company will pay PGEN tiered royalties ranging from low-single digit to high-single digit on the net sales derived from the sales of any approved IL-12 Products and CAR Products. The Company will also pay PGEN royalties ranging from low-single digit to mid-single digit on the net sales derived from the sales of any approved TCR Products, up to a maximum royalty amount of $100.0 million in the aggregate. The Company will also pay PGEN 20% of any sublicensing income received by the Company relating to the licensed products.

The Company is responsible for all development costs associated with each of the licensed products, other than Gorilla IL-12 Products.

PGEN will pay the Company royalties ranging from low-single digits to mid-single digits on the net sales derived from the sale of PGEN's CAR products, up to $50.0 million.

During the three and nine months ended September 30, 2020, there were no expenses for services performed by PGEN. As of September 30, 2020 and December 31, 2019, the Company had $0 and $0.1 million, respectively, in accrued expenses for amounts due to PGEN. During the three and nine months ended September 30, 2019, the Company recorded an expense of $0.3 million and $1.7 million, respectively, for services performed by PGEN.

ZIOPHARM Oncology, Inc.

NOTES TO FINANCIAL STATEMENTS (unaudited)

6. Commitments and Contingencies (Continued)

License Agreement—The University of Texas MD Anderson Cancer Center

On January 13, 2015, the Company, together with Precigen, entered into the MD Anderson License with MD Anderson (which Precigen subsequently assigned to PGEN). Pursuant to the MD Anderson License, the Company, together with Precigen, holds an exclusive, worldwide license to certain technologies owned and licensed by MD Anderson including technologies relating to novel CAR T-cell therapies, non-viral gene transfer systems, genetic modification and/or propagation of immune cells and other cellular therapy approaches, Natural Killer, or NK Cells, and TCRs, arising from the laboratory of Laurence Cooper, M.D., Ph.D., who became the Company's Chief Executive Officer in May 2015 and was formerly a tenured professor of pediatrics at MD Anderson and is now currently a visiting scientist under that institution's policies.

The term of the MD Anderson License expires on the later of (a) the expiration of all patents licensed thereunder, or (b) the twentieth anniversary of the date of the MD Anderson License; provided, however, that following the expiration of the term of the MD Anderson License, the Company, together with PGEN, shall have a fully-paid up, royalty free, perpetual, irrevocable and sublicensable license to use the licensed intellectual property thereunder. After ten years from the date of the MD Anderson License and subject to a 90-day cure period, MD Anderson will have the right to convert the MD Anderson License into a non-exclusive license if ZIOPHARM and PGEN are not using commercially reasonable efforts to commercialize the licensed intellectual property on a case-by-case basis. After five years from the date of the MD Anderson License and subject to a 180-day cure period, MD Anderson will have the right to terminate the MD Anderson License with respect to specific technology(ies) funded by the government or subject to a third-party contract if the Company and PGEN are not meeting the diligence requirements in such funding agreement or contract, as applicable. MD Anderson may also terminate the agreement with written notice upon material breach by us and PGEN, if such breach has not been cured within 60 days of receiving such notice. In addition, the MD Anderson License will terminate upon the occurrence of certain insolvency events for both the Company and PGEN and may be terminated by the mutual written agreement of the Company, PGEN, and MD Anderson.

On August 17, 2015, the Company, Precigen and MD Anderson entered into the Research and Development, or the 2015 Agreement, to formalize the scope and process for the transfer by MD Anderson, pursuant to the terms of the MD Anderson License, of certain existing research programs and related technology rights, as well as the terms and conditions for future collaborative research and development of new and ongoing research programs.

Pursuant to the 2015 Agreement, the Company, Precigen and MD Anderson formed a joint steering committee to oversee and manage the new and ongoing research programs. Under the License Agreement with PGEN, the Company and PGEN agreed that PGEN would no longer participate on the joint steering committee after the date of the License Agreement. As provided under the MD Anderson License, the Company provided funding for research and development activities in support of the research programs under the Research and Development Agreement for a period of three years and in an amount of no less than $15.0 million and no greater than $20.0 million per year. On October 22, 2019, the Company entered into an amendment to the Research and Development Agreement extending its term until December 31, 2026.

During the nine months ended September 30, 2020 and 2019, the Company made no payments to MD Anderson. The net balance of cash resources on hand at MD Anderson available to offset expenses and future costs is $11.2 million, which is included in prepaid expenses and other current assets on the Company's balance sheet at September 30, 2020.

On October 22, 2019, the Company entered into the 2019 Research and Development Agreement, or the 2019 Agreement, with MD Anderson, pursuant to which the parties agreed to collaborate with respect to the Company's *Sleeping Beauty* immunotherapy program, which uses non-viral gene transfer to stably express and clinically evaluate neoantigen-specific TCRs in T cells. Under the 2019 Agreement, the parties will, among other things, collaborate on programs to expand the Company's TCR library and conduct clinical trials.

ZIOPHARM Oncology, Inc.

NOTES TO FINANCIAL STATEMENTS (unaudited)

6. Commitments and Contingencies (Continued)

The Company will own all intellectual property developed under the 2019 Agreement and will retain all rights to intellectual property for oncology products manufactured using non-viral gene transfer technologies under the Agreement, including the Company's *Sleeping Beauty* technology. The Company has granted MD Anderson an exclusive license for such intellectual property outside the field of oncology and to develop and commercialize autologous TCR products manufactured using viral gene transfer technologies, and a non-exclusive license for allogeneic TCR products manufactured using viral-based technologies.

The Company has agreed, beginning on January 1, 2021, to reimburse MD Anderson up to a total of $20.0 million for development costs incurred starting after January 1, 2021 under the 2019 Agreement. In addition, the Company will pay MD Anderson royalties on net sales of its TCR products at rates in the low single digits. The Company is required to make performance-based payments upon the successful completion of clinical and regulatory benchmarks relating to its TCR products. The aggregate potential benchmark payments are $36.5 million, of which only $3.0 million will be due prior to the first marketing approval of the Company's TCR products. The royalty rates and benchmark payments owed to MD Anderson may be reduced upon the occurrence of certain events. The Company also agreed that it will sell the Company's TCR products to MD Anderson at preferential prices and will sell its TCR products in Texas exclusively to MD Anderson for a limited period of time following the first commercial sale of the Company's TCR products.

In connection with the execution of the 2019 Agreement, the Company issued MD Anderson a warrant to purchase 3,333,333 shares of common stock. Refer to Note 9 – *Warrants* for further details.

License Agreement with the National Cancer Institute

On May 28, 2019, the Company entered into a patent license agreement, or the Patent License, with the National Cancer Institute, or the NCI. Pursuant to the Patent License, the Company holds an exclusive, worldwide license to certain intellectual property to develop and commercialize patient-derived (autologous), peripheral blood T-cell therapy products engineered by transposon-mediated gene transfer to express TCRs reactive to mutated KRAS, TP53 and EGFR. In addition, pursuant to the Patent License, the Company holds an exclusive, worldwide license to certain intellectual property for manufacturing technologies to develop and commercialize autologous, peripheral blood T-cell therapy products engineered by non-viral gene transfer to express TCRs, as well as a non-exclusive, worldwide license to certain additional manufacturing technologies.

Pursuant to the terms of the Patent License, the Company is required to pay the NCI a cash payment in the aggregate amount of $1.5 million payable in $0.5 million installments within sixty days, six-months, and the twelve-month anniversary of the effective date of the agreement of the Patent License. The $1.5 million was paid as of the nine months ended September 30, 2020.

On January 8, 2020, the Company entered into an amendment to the patent license agreement which expanded the TCR library to include additional TCR's reactive to mutated KRAS and TP53. Under the amendment, the Company paid $0.6 million during the nine months ending September 30, 2020. The Company recognized $0 and $0.6 million of expense for the three and nine months ended September 30, 2020, respectively.

The terms of the Patent License also require the Company to pay the NCI minimum annual royalties in the amount of $0.3 million, which amount will be reduced to $0.1 million once the aggregate minimum annual royalties paid by the Company equals $1.5 million. The first minimum annual royalty payment is payable on the date that is eighteen months following the date of the Patent License. As of September 30, 2020, the Company included a prepayment of $0.3 million related to the Patent License as prepaid expenses and other current assets on the Company's balance sheet.

ZIOPHARM Oncology, Inc.

NOTES TO FINANCIAL STATEMENTS (unaudited)

6. Commitments and Contingencies (Continued)

On September 28, 2020, the Company entered into a second amendment to the patent license agreement which expanded the TCR library to include additional TCR's receptors. Under the second amendment, the Company will pay the NCI an additional $0.4 million. The Company recorded $0.4 million as an accrued expense as of September 30, 2020.

The Company is also required to make performance-based payments upon successful completion of clinical and regulatory benchmarks relating to the licensed products. The aggregate potential benchmark payments are $4.3 million, of which aggregate payments of $3.0 million are due only after marketing approval in the United States or in Europe, Japan, Australia, China or India. The first benchmark payment of $0.1 million will be due upon the initiation of the Company's first sponsored Phase 1 clinical trial of a licensed product or licensed process in the field of use licensed under the Patent License.

In addition, the Company is required to pay the NCI one-time benchmark payments following aggregate net sales of licensed products at certain net sales up to $1.0 billion. The aggregate potential amount of these benchmark payments is $12.0 million. The Company must also pay the NCI royalties on net sales of products covered by the Patent License at rates in the low to mid-single digits depending upon the technology included in a licensed product. To the extent the Company enters into a sublicensing agreement relating to a licensed product, the Company is required to pay the NCI a percentage of all consideration received from a sublicensee, which percentage will decrease based on the stage of development of the licensed product at the time of the sublicense.

The Patent License will expire upon expiration of the last patent contained in the licensed patent rights, unless terminated earlier. The NCI may terminate or modify the Patent License in the event of a material breach, including if the Company does not meet certain milestones by certain dates, or upon certain insolvency events that remain uncured following the date that is 90 days following written notice of such breach or insolvency event. The Company may terminate the Patent License, or any portion thereof, in the Company's sole discretion at any time upon 60 days' written notice to the NCI. In addition, the NCI has the right to: (i) require the Company to sublicense the rights to the product candidates covered by the Patent License upon certain conditions, including if the Company is not reasonably satisfying required health and safety needs and (ii) terminate or modify the Patent License, including if the Company is not satisfying requirements for public use as specified by federal regulations.

Cooperative Research and Development Agreement (CRADA) with the National Cancer Institute

On January 10, 2017, the Company announced the signing of a CRADA, with the NCI for the development of adoptive cell transfer, or ACT,-based immunotherapies genetically modified using the *Sleeping Beauty* transposon/transposase system to express TCRs for the treatment of solid tumors. The principal goal of the CRADA is to develop and evaluate ACT for patients with advanced cancers using autologous peripheral blood lymphocytes, or PBL, genetically modified using the non-viral *Sleeping Beauty* system to express TCRs that recognize neoantigens expressed within a patient's cancer. Research conducted under the CRADA will be at the direction of Steven A. Rosenberg, M.D., Ph.D., Chief of the Surgery Branch at the NCI, in collaboration with the Company. In February 2019, the Company extended the CRADA with the NCI for two years, committing an additional $5.0 million to this program. The Company recorded $0.6 million and $1.3 million of expense for the three and nine months ended September 30, 2020 and 2019, respectively.

Ares Trading License and Collaboration Agreement

On March 27, 2015, the Company, together with PGEN, signed the Ares Trading Agreement, with Ares Trading S.A., a subsidiary of the biopharmaceutical business of Merck KGaA, Darmstadt, Germany, through which the parties established a collaboration for the research and development and commercialization of certain products for the prophylactic, therapeutic, palliative or diagnostic use for cancer in humans.

ZIOPHARM Oncology, Inc.

NOTES TO FINANCIAL STATEMENTS (unaudited)

6. Commitments and Contingencies (Continued)

PGEN was entitled to receive $5.0 million, from Ares Trading, payable in equal quarterly installments over two years for each identified product candidate, which will be used to fund discovery work. The Company was responsible for costs exceeding the quarterly installments and all other costs of the preclinical research and development. For the nine months ended September 30, 2020 and 2019, the Company incurred no expenses under the Ares Trading Agreement.

Ares Trading paid a non-refundable upfront fee of $115.0 million to PGEN as consideration for entry into the Ares Trading Agreement. Pursuant to the Third Amendment to Exclusive Channel Partner Agreement, or the 2016 ECP Amendment, the Company was entitled to receive 50% of the upfront fee, or $57.5 million, which was received from PGEN in July 2015.

Under the License Agreement, PGEN agreed to perform all future obligations of the Company under the Ares Trading Agreement other than certain payment obligations. Accordingly, the Company recognized the remaining deferred revenue as part of the settlement of a related party relationship in 2018.

Patent and Technology License Agreement—The University of Texas MD Anderson Cancer Center and the Texas A&M University System

On August 24, 2004, the Company entered into a patent and technology license agreement with MD Anderson and the Texas A&M University System, which the Company refers to, collectively, as the Licensors. Under this agreement, the Company was granted an exclusive, worldwide license to rights (including rights to U.S. and foreign patent and patent applications and related improvements and know-how) for the manufacture and commercialization of two classes of organic arsenicals (water- and lipid-based) for human and animal use. The class of water-based organic arsenicals includes darinaparsin.

Under the terms of the agreement, the Company may be required to make additional payments to the Licensors upon achievement of certain other milestones in varying amounts which, on a cumulative basis could total up to an additional $4.5 million. In addition, the Licensors are entitled to receive single digit percentage royalty payments on sales from a licensed product and will also be entitled to receive a portion of any fees that the Company may receive from a possible sublicense under certain circumstances.

Collaboration Agreement with Solasia Pharma K.K.

On March 7, 2011, the Company entered into a License and Collaboration Agreement with Solasia Pharma K.K., or Solasia, which was amended on July 31, 2014 to include an exclusive worldwide license. Pursuant to the License and Collaboration Agreement, the Company granted Solasia an exclusive license to develop and commercialize darinaparsin in both intravenous and oral forms and related organic arsenic molecules, in all indications for human use.

As consideration for the license, the Company is eligible to receive from Solasia development- and sales-based milestones, a royalty on net sales of darinaparsin, once commercialized, and a percentage of any sublicense revenues generated by Solasia. Solasia will be responsible for all costs related to the development, manufacturing and commercialization of darinaparsin. The Company's Licensors, as defined in the agreement, will receive a portion of all milestone and royalty payments made by Solasia to the Company in accordance with the terms of the license agreement with the Licensors.

ZIOPHARM Oncology, Inc.

NOTES TO FINANCIAL STATEMENTS (unaudited)

7. Leases

In June 2012, the Company entered into a master lease for the Company's corporate office headquarters in Boston, which was originally set to expire in August 2016, but renewed through August 31, 2021. As of September 30, 2020 and December 31, 2019, a total security deposit of $0.1 million is included in deposits on the Company's balance sheet. On January 30, 2018, the Company entered into a lease agreement for office space in Houston at MD Anderson. Under the terms of the Houston lease agreement, the Company leased approximately two hundred and ten square feet and were required to make rental payments at an average monthly rate of approximately $1 thousand. This lease was terminated effective March 31, 2020.

On March 12, 2019, the Company entered into a lease agreement for office space in Houston. Under the terms of the Houston lease agreement, the Company leases approximately one thousand and thirty-eight square feet and is required to make rental payments at an average monthly rate of approximately $2 thousand through April 2021. On October 15, 2019, the Company entered into a lease agreement for additional office space in Houston. Under the terms of the Second Houston Lease, the Company leases, from MD Anderson, approximately eight thousand four hundred and forty-three square feet and is initially required to make rental payments of approximately $17 thousand per month through February 2027, subject to an annual base rent increase of approximately 3.0% throughout the term. Effective April 13, 2020, the Company leased an additional five thousand five hundred eighty-four square feet from MD Anderson. The Company is initially required to make rental payments of approximately $12 thousand per month through February 2027, subject to an annual base rent increase of approximately 3.0% throughout the term. All future rent expense incurred in Houston, will be deducted from the Company's prepayments at MD Anderson. Effective June 1, 2020, the Company entered into a noncancelable lease for a period of less than a year with monthly payments of approximately $10 thousand. Effective September 1, 2020, the Company added additional space to the noncancelable lease for a period of less than a year with monthly payments now totaling approximately $15 thousand.

The components of lease expense were as follows:

(in thousands)	Three Months Ended September 30, 2020	Nine Months Ended September 30, 2020
Operating lease cost	$ 267	$ 768
Total lease cost	$ 267	$ 768
Weighted-average remaining lease term (years)	4.99	4.99
Weighted-average discount rate	8.00%	8.00%

ZIOPHARM Oncology, Inc.

NOTES TO FINANCIAL STATEMENTS (unaudited)

7. Leases (Continued)

(in thousands)	Three Months Ended September 30, 2019	Nine Months Ended September 30, 2019
Operating lease cost	$ 184	$ 585
Total lease cost	$ 184	$ 585
Weighted-average remaining lease term (years)	2.15	2.15
Weighted-average discount rate	8.00%	8.00%

As of September 30, 2020, the maturities of the Company's operating lease liabilities for the years ended December 31, were as follows (in thousands):

2020 (excluding the nine months ended September 30, 2020)	$ 318
2021	896
2022	353
2023	364
2024	375
Thereafter	851
Total lease payments	3,157
Less: Imputed interest and adjustments	(637)
Present value of lease payments	$2,520

8. Stock-Based Compensation

The Company recognized stock-based compensation expense on all employee and non-employee awards as follows:

(in thousands)	For the three months ended September 30, 2020	For the three months ended September 30, 2019	For the nine months ended September 30, 2020	For the nine months ended September 30, 2019
Research and development	$ 522	$ 339	$ 1,587	$ 1,068
General and administrative	1,270	1,147	3,806	3,673
Stock-based compensation expense	$ 1,792	$ 1,486	$ 5,393	$ 4,741

The Company granted an aggregate of 203,178 and 1,252,178 stock options during the three and nine months ended September 30, 2020 with a weighted-average grant date fair value of $1.96 and $2.45 per share, respectively. The Company granted an aggregate of 140,000 and 1,835,755 stock options during the three and nine months ended September 30, 2019 with a weighted average grant date fair value of $3.05 and $1.90 per share, respectively.

On January 6, 2019, the Company settled an accrued annual performance bonus by issuing 446,428 shares of common stock.

On May 26, 2020, the Company extended the contractual life of 448,130 fully vested stock options and 31,220 stock options that vested on June 30, 2020, held by an officer of the Company. Additionally, on May 26, 2020, the Company accelerated the vesting of 45,277 shares of restricted stock held by an officer. These extensions and acceleration of vesting resulted in additional stock compensation expense of $65 thousand and $154 thousand in the three and nine months ended September 30, 2020, respectively.

On September 18, 2020, the Company extended the contractual life of 113,350 fully vested stock options and 25,281 stock options that vested on September 18, 2020, held by a director of the Company. Additionally, on September 18, 2020, the Company accelerated the vesting of 25,281 stock options and 15,890 shares of restricted stock held by a director of the Company. These extensions and acceleration of vesting resulted in additional stock compensation expense of $(250) in the three and nine months ended September 30, 2020.

For the three months ended September 30, 2020 and 2019, the fair value of stock options was estimated on the date of grant using a Black-Scholes option valuation model with the following assumptions:

	For the three months ended September 30, 2020	For the three months ended September 30, 2019
Risk-free interest rate	0.36 - 0.39%	1.39 - 1.92%
Expected life in years	5.75 - 6.25	6.25
Expected volatility	73.59 - 74.18%	72.87 - 78.34%
Expected dividend yield	0%	0%

At September 30, 2020, there were 863,333 stock options that had been issued outside the 2012 Equity Incentive Plan, or the 2012 Plan. These options are excluded from the schedule below.

ZIOPHARM Oncology, Inc.

NOTES TO FINANCIAL STATEMENTS (unaudited)

8. Stock-Based Compensation (Continued)

Stock option activity under the Company's stock option plans for the nine months ended September 30, 2020 is as follows:

(in thousands, except share and per share data)	Number of Shares	Weighted-Average Exercise Price	Weighted-Average Contractual Term (Years)	Aggregate Intrinsic Value
Outstanding, December 31, 2019	5,842,879	$ 3.21		
Granted	1,252,178	3.89		
Exercised	(22,916)	1.87		
Cancelled	(499,950)	3.84		
Outstanding, September 30, 2020	6,572,191	$ 3.93	7.82	$ 978
Options exercisable, September 30, 2020	3,711,684	$ 4.10	6.96	$ 704
Options exercisable, December 31, 2019	2,765,357	$ 4.39	6.70	$ 3,603
Options available for future grant	6,986,610			

At September 30, 2020, total unrecognized compensation costs related to unvested stock options outstanding amounted to $8.0 million. The cost is expected to be recognized over a weighted-average period of 1.82 years.

A summary of the status of unvested restricted stock for the nine months ended September 30, 2020 is as follows:

	Number of Shares	Weighted-Average Grant Date Fair Value
Non-vested, December 31, 2019	939,636	$ 2.93
Granted	555,900	4.21
Vested	(64,917)	3.42
Cancelled	(141,230)	3.15
Non-vested, September 30, 2020	1,289,389	$ 3.43

At September 30, 2020, total unrecognized compensation costs related to unvested restricted stock outstanding amounted to $3.0 million. The cost is expected to be recognized over a weighted-average period of 1.57 years.

At the Company's annual meeting held on June 29, 2020, the shareholders approved the 2020 Equity Incentive Plan, or the 2020 Plan, which is a successor to and continuation of the Company's 2012 Equity Incentive Plan, or the 2012 plan. The 2020 Plan had 21 million shares authorized, plus the shares remaining for issuance under the 2012 Plan. Our ability to utilize the total shares authorized under the 2020 Plan will be limited by the total number of shares authorized in our certificate of incorporation. As of September 30, 2020, there are 6,986,610 Shares available to grant from the 2020 plan. No additional awards can be granted from the 2012 Plan or the Company's 2003 Stock Option Plan.

ZIOPHARM Oncology, Inc.

NOTES TO FINANCIAL STATEMENTS (unaudited)

9. Warrants

In connection with the Company's November 2018 private placement which provided net proceeds of approximately $47.1 million, the Company issued warrants to purchase an aggregate of 18,939,394 shares of common stock, or the 2018 warrants, which became exercisable six months after the closing of the private placement. The warrants have an exercise price of $3.01 per share and have a five-year term. The relative fair value of the warrants was estimated at $18.4 million using a Black-Scholes model with the following assumptions: expected volatility of 71%, risk free interest rate of 2.99%, expected life of five years and no dividends.

The Company assessed whether the warrants require accounting as derivatives. The Company determined that the warrants were (1) indexed to the Company's own stock and (2) classified in stockholders' equity in accordance with Financial Accounting Standards Board, or ("FASB") Accounting Standards Codification ("ASC") Topic 815, *Derivatives and Hedging*. As such, the Company has concluded the warrants meet the scope exception for determining whether the instruments require accounting as derivatives and should be classified in stockholders' equity.

On July 26, 2019 and September 12, 2019, the Company entered into agreements with existing investors for the exercise of previously issued warrants to purchase common stock in the private placement. Pursuant to the terms of the agreements, investors exercised their 2018 warrants for an aggregate of 17,803,031 shares of common stock, at an exercise price of $3.01 per share. Proceeds from the warrant exercise, after deducting placement agent fees and other related expenses of $1.1 million were approximately $52.5 million. The Company issued participating investors new warrants to purchase up to 17,803,031 additional shares of common stock, or the 2019 warrants, as an inducement for the warrant holders to exercise their 2018 warrants. The 2019 warrants will expire on the fifth anniversary of the initial exercise date and have an exercise price of $7.00. The 2019 warrants were valued using a Black-Scholes valuation model and resulted in a $60.8 million non-cash charge to the Company's statement of operations in 2019.

On October 22, 2019, the Company entered into the 2019 Agreement with MD Anderson. Refer to Note 6 – *Commitments and Contingencies* for further details In connection with the execution of the 2019 Agreement, the Company issued MD Anderson a warrant to purchase 3,333,333 shares of common stock. The warrant has an initial exercise price of $0.001 per share and grant date fair value of $14.5 million. The warrant expires on December 31, 2026 and vests upon the occurrence of certain clinical milestones. The Company will recognize expense on the warrant in the same manner as if the Company paid cash for services to be rendered. As of September 30, 2020, work related to the clinical milestones has not been started and therefore, the Company did not recognize any expense related to the warrant.

10. Joint Venture

On December 18, 2018, the Company entered into a Framework Agreement with TriArm Therapeutics, Ltd., or TriArm, pursuant to which the parties agreed to launch Eden BioCell, Ltd., or Eden BioCell, to lead clinical development and commercialization of certain *Sleeping Beauty*-generated CAR-T therapies as set forth in a separate license agreement.

On January 3, 2019, Eden BioCell was incorporated in Hong Kong as a private company. Eden BioCell, the Company and TriArm entered into a Share Subscription Agreement on January 23, 2019, where the Company and TriArm agreed to contribute certain intellectual property, services and cash (only with respect to TriArm) to Eden BioCell to subscribe for a certain number of newly issued ordinary shares in the share capital of Eden BioCell. On the closing date, upon the issuance and subscription of the shares, in respect of the aforementioned consideration, 10,000,000 ordinary shares were issued to the Company and 10,000,000 ordinary shares were issued to TriArm.

ZIOPHARM Oncology, Inc.

NOTES TO FINANCIAL STATEMENTS (unaudited)

10. Joint Venture (Continued)

The closing of the transaction occurred on July 5, 2019. The Framework Agreement and Share Subscription Agreements were each respectively amended to be effective as of this date. Upon consummation of the joint venture, Eden BioCell and the Company also entered into a license agreement, pursuant to which the Company licensed the rights to Eden BioCell for third-generation *Sleeping Beauty*-generated CAR-T therapies targeting the CD19 antigen for the territory of China (including Macau and Hong Kong), Taiwan and Korea. Eden BioCell will be responsible for certain milestone and royalty payments related to the Company's license agreements with MD Anderson and PGEN (see Note 6). TriArm entered into a Master Services Agreement with Eden BioCell and contributed $10.0 million of cash on the closing date. TriArm also committed to contribute an additional $25.0 million to Eden BioCell over time through the achievement of certain specified milestones. TriArm and the Company each received a 50% equity interest in the joint venture in exchange for their contributions to Eden BioCell.

As of July 5, 2019, as a result of the design and purpose of Eden BioCell, the Company determined that Eden BioCell was considered a variable interest entity, or VIE, and concluded that it is not the primary beneficiary of the VIE as it did not have the power to direct the activities of the VIE that most significantly impact its performance. Rather, the Company accounts for the equity interest in Eden BioCell under the equity method of accounting as it has the ability to exercise significant influence over the operations of Eden BioCell.

The Company determined that Eden BioCell was not a customer and therefore, accounted for the transaction as the transfer of nonfinancial assets to be recognized at their fair value on the contribution date. The fair value of the intellectual property contributed to Eden BioCell had a de minimis value due to the early stage of the technology and the likelihood of clinical success. Due to the de minimis fair value of the intellectual property contributed, the Company did not record a gain or loss on this transaction and recognized no value for its equity-method investment.

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

The following information should be read in conjunction with our unaudited condensed consolidated financial statements and the notes thereto included in this Quarterly Report on Form 10-Q and the audited financial information and the notes thereto included in our Annual Report on Form 10-K, which was filed with the Securities and Exchange Commission, or the SEC, on March 2, 2020, or the Annual Report.

Except for the historical information contained herein, the matters discussed in this Quarterly Report on Form 10-Q may be deemed to be forward-looking statements that involve risks and uncertainties. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. In this Quarterly Report on Form 10-Q, words such as "may," "expect," "anticipate," "estimate," "intend," "plan," and similar expressions (as well as other words or expressions referencing future events, conditions or circumstances) are intended to identify forward-looking statements.

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

The following information and any forward-looking statements should be considered in light of factors discussed elsewhere in this Quarterly Report on Form 10-Q, including those risks identified under Part II, Item 1A. Risk Factors.

We caution readers not to place undue reliance on any forward-looking statements made by us, which speak only as of the date they are made. We disclaim any obligation, except as specifically required by law and the rules of the SEC, to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.

Overview

We are a clinical-stage biopharmaceutical company focused on discovering, acquiring, developing and commercializing next generation immuno-oncology platforms that leverage cell- and gene-based therapies to treat patients with cancer. We are developing two immuno-oncology platform technologies that utilize the immune system by employing innovative cell engineering and novel, controlled gene expression technologies designed to deliver safe, effective, and scalable non-viral cell- and viral-based gene therapies for the treatment of multiple cancer types. Our first platform is referred to as *Sleeping Beauty* and is based on the genetic engineering of immune cells using a non-viral transposon/transposase system that is intended to stably reprogram T cells outside of the body for subsequent infusion. Our second platform is referred to as Controlled IL-12 and is designed to stimulate expression of interleukin 12, or IL-12, a master regulator of the immune system, in a controlled and safe manner to focus the patient's immune system to more effectively attack cancer cells. We intend to use both of our platforms to become a leading immuno-oncology company focused on developing innovative, cost-effective therapies primarily aimed at the large unmet needs in solid tumors.

Using our *Sleeping Beauty* platform, we are developing T cell receptor, or TCR, T cell therapies to target solid tumors. Our program designs and manufactures T cells that are intended to target tumor-specific antigens, thereby delivering personalized therapy that can attack patients' malignancies. These genetic changes are referred to as neoantigens as they are only expressed by the tumor, reducing the potential for toxicity upon targeting normal cells. Under our Cooperative Research and Development Agreement, the National Cancer Institute, or NCI, is conducting a Phase 2 clinical trial to evaluate autologous peripheral blood lymphocytes genetically modified with the *Sleeping Beauty* system to express autologous (personalized) TCRs. In addition, we are currently planning a clinical program to study our TCR approach with The University of Texas MD Anderson Cancer Center, or MD Anderson. Under this program, we expect to clinically evaluate both our Personalized TCR Approach and our Library TCR Approach.

Our Controlled IL-12 platform uses virotherapy based on an engineered replication-incompetent adenovirus, referred to as Ad-RTS-hIL-12, plus veledimex as a gene delivery system to conditionally produce IL-12, a potent, naturally occurring anti-cancer protein, to treat patients with solid tumors where a specific target is unknown. Our Controlled IL-12 platform allows us to deliver IL-12 in a tunable dose as the cytokine is under transcriptional control of the RheoSwitch Therapeutic System® (RTS®). We are currently studying our Controlled IL-12 Platform as a monotherapy for the treatment of recurrent glioblastoma multiforme, or rGBM, and for the treatment of glioma in the pontine region of the brain, known as diffuse intrinsic pontine glioma, or DIPG. We are currently evaluating Ad-RTS-hIL-12 plus veledimex in a Phase 1/2 clinical trial for the treatment of DIPG and expect to dose up to 12 patients in the initial portion of the clinical trial. We are also developing our Controlled IL-12 platform in combination with immune checkpoint inhibitors. We have completed dosing in a Phase 1 dose-escalation clinical trial of Ad-RTS-hIL-12 plus veledimex in combination with PD-1 antibody OPDIVO® (nivolumab) in patients with rGBM. We have also completed dosing in a Phase 2 clinical trial evaluating Ad-RTS-hIL-12 plus veledimex in combination with PD-1 antibody Libtayo® (cemiplimab) for the treatment of recurrent or progressive glioblastoma multiforme in adults.

We are developing chimeric antigen receptor, or CAR, T cell, or CAR^+ T, therapies targeting CD19 on malignant B cells using our *Sleeping Beauty* platform in collaboration with MD Anderson. In a Phase 1 trial, we plan to infuse donor-derived T cells after allogeneic bone marrow transplantation, or BMT, for recipients who have relapsed with $CD19^+$ leukemias and lymphomas with our CD19-specific CAR^+ T therapies manufactured using our rapid personalized manufacturing, or RPM, technology. RPM enables T cells to be infused as soon as the day after gene transfer which is made possible by the genetic modification of resting T cells to express CAR and membrane bound IL-15, or mbIL15. We are also advancing our RPM technology in Greater China with Eden BioCell, Ltd., or Eden BioCell, our joint venture with TriArm Therapeutics, Ltd. Eden BioCell will lead the clinical development and commercialization of *Sleeping Beauty*-generated CD19-specific RPM CAR^+ T therapies using patient-derived (autologous) T cells in order to treat patients with relapsed or refractory $CD19^+$ leukemias and lymphomas.

We have not generated significant revenue and have incurred significant net losses in each year since our inception. For the nine months ended September 30, 2020, we had a net loss of $57.2 million, and, as of September 30, 2020, we have incurred approximately $741.3 million of accumulated deficit since our inception in 2003. We expect to continue to incur significant operating expenditures and net losses. Further development of our product candidates will likely require substantial increases in our expenses as we:

- continue to undertake clinical trials for product candidates;
- seek regulatory approvals for product candidates;
- work with regulatory authorities to identify and address program-related inquiries;
- implement additional internal systems and infrastructure;
- hire additional personnel; and
- scale-up the formulation and manufacturing of our product candidates.

We continue to seek additional financial resources to fund the further development of our product candidates. If we are unable to obtain sufficient additional capital, one or more of these programs could be delayed, and we may be unable to continue our operations at planned levels and be forced to reduce our operations. Because of the numerous risks and uncertainties associated with product development, we are unable to predict the timing or amount of increased expenses or when or if we will be able to achieve or maintain profitability.

Recent Developments

The ongoing COVID-19 global pandemic has presented a significant health and economic challenge around the world and is affecting our employees, partners and business operations. The full extent to which the COVID-19 pandemic will directly or indirectly impact our business, results of operations and financial condition will depend on future developments that are highly uncertain and cannot be accurately predicted. We have implemented work-from-home policies for most of our employees in response to the COVID-19 pandemic. The effects of our work-from-home policies may negatively impact productivity, disrupt our business and delay our clinical programs and timelines, the magnitude of which will depend, in part, on the length and severity of the restrictions and other limitations on our ability to conduct our business in the ordinary course. These and similar, and perhaps more severe, disruptions in our operations could negatively impact our business, operating results and financial condition. We continue to work with our partners, including the NCI and MD Anderson, to mitigate the impact the COVID-19 pandemic is having on our business and clinical programs.

Clinical and Regulatory Developments

We expect to initiate our clinical trial with MD Anderson for our Library TCR-T approach in mid-2021. This study will evaluate treatments using TCR+ T cells expressing third party (allogeneic) TCRs in autologous (patient-derived) T cells to treat several cancers, including gynecologic, colorectal, pancreatic, non-small cell lung cancer and cholangiocarcinoma. We expect to submit an Investigational New Drug, or IND, application for this clinical trial to the FDA in the first quarter of 2021.

Under our Cooperative Research and Development Agreement, the NCI is undertaking a Phase 2 Personalized TCR-T clinical trial under an IND cleared by the FDA. In this study, the NCI will test autologous peripheral blood lymphocytes genetically modified with the *Sleeping Beauty* system to express TCRs that recognize neoantigens expressed by patients with a broad range of solid tumors. The NCI's manufacturing facility is validating our non-viral manufacturing approach, which has been authenticated by the NCI at laboratory scale. Patient enrollment will be determined by the NCI and is expected to occur after the process validation, the completion of regulatory requirements and the NCI has identified and screened patients for neoantigens and TCRs to render them eligible for the trial.

Eden BioCell has continued to make significant progress preparing for a clinical trial of *Sleeping Beauty*-generated CD19-specific RPM CAR+ T therapies using patient-derived (autologous) T cells to treat patients with relapsed or refractory CD19+ leukemias and lymphomas. Eden BioCell has commenced the filing of its IND for a clinical trial in Taiwan. In addition, Eden BioCell and our partners in this joint venture have informed us that preliminary observations from several patients dosed under compassionate use requests in Greater China confirm the presence of infused autologous T cells measured weeks after infusion, which appears to support the benefit of genetically modifying T cells with our RPM technology.

In July 2020, we announced the initiation of our U.S. Phase 1 trial clinical trial infusing donor-derived T cells after allogeneic BMT for recipients who have relapsed with $CD19^+$ leukemias and lymphomas with our CD19-specific CAR^+ T therapies manufactured using our RPM technology. This clinical trial is being performed in collaboration with MD Anderson.

Each of the clinical trials of our Controlled IL-12 program continues to progress. We previously completed enrollment in our Phase 1 clinical trial of adult patients with rGBM evaluating Ad-RTS-hIL-12 plus daily veledimex in combination with OPDIVO®. In June 2020, we completed patient enrollment in our Phase 2 clinical trial evaluating Controlled IL-12 in combination with PD-1 antibody Libtayo® for the treatment of rGBM. In July 2020, we announced the first patient had been dosed in a Phase 1/2 clinical trial evaluating Ad-RTS-hIL-12 plus veledimex for the treatment of DIPG. Data will be presented from all three trials at the 2020 Society for Neuro-Oncology Annual Meeting (SNO).

Appointment of Personnel

In September 2020, our Board of Directors appointed J. Kevin Buchi to our Board to fill the vacancy created by the resignation of Douglas Pagán. Mr. Buchi was Chief Executive Officer of Cephalon, Inc., which was acquired by Teva Pharmaceutical Industries Limited in October 2011. Following the acquisition of Cephalon by Teva in 2011, Mr. Buchi served as corporate vice president of global branded products at Teva. Following Teva, he was Chief Executive Officer of TetraLogic Pharmaceuticals and Biospecifics Technologies.

In June 2020, we announced the appointment of Carl June, M.D., as Chairman of our newly formed Scientific Advisory Board, or SAB. Dr. June is recognized in the oncology field for his groundbreaking work in the development and commercialization of gene therapy and T-cell therapies. In September 2020, we announced Adi Barzel, Ph.D., Gavin Dunn, M.D., Ph.D., Matthew Porteus, M.D., Ph.D., and Kole Roybal, Ph.D had joined the SAB. The SAB will provide strategic counsel to guide the efficient development of our innovative technologies and pipeline of immunotherapies.

Financial Overview

Overview of Results of Operations

Three and Nine Months Ended September 30, 2020 Compared to Three and Nine Months Ended September 30, 2019

Research and development expenses. Research and development expenses during the three and nine months ended September 30, 2020 and 2019 were as follows:

	Three months ended September 30,				Nine months ended September 30,			
($ in thousands)	2020	2019	Change		2020	2019	Change	
Research and development	$13,968	$ 8,641	$5,327	62%	$38,725	$28,115	$10,610	38%

Research and development expenses for the three months ended September 30, 2020 increased by $5.3 million when compared to the three months ended September 30, 2019. The increase in research and development expenses for the three months ended September 30, 2020, is primarily due to $3.9 million in increased gene therapy manufacturing costs and $2.0 million related to increased headcount and facilities costs, offset by $0.6 million of decreased manufacturing technology initiatives and preclinical costs.

Research and development expenses for the nine months ended September 30, 2020 increased by $10.6 million when compared to the nine months ended September 30, 2019. The increase in research and development expenses for the nine months ended September 30, 2020, is primarily due to $7.1 million in increased gene therapy manufacturing costs and $5.1 million related to increased headcount and facilities costs, offset by $1.3 million of decreased cell therapy costs and $0.3 million in travel and other.

Our research and development expense consists primarily of salaries and related expenses for personnel, costs of contract manufacturing services, costs of facilities and equipment, fees paid to professional service providers in conjunction with our clinical trials, fees paid to contract research organizations in conjunction with preclinical animal studies, costs of materials used in research and development, consulting, license and milestone payments and sponsored research fees paid to third parties.

We have not accumulated and tracked our internal historical research and development costs or our personnel and personnel-related costs on a program-by-program basis. Our employee and infrastructure resources are allocated across several projects, and many of our costs are directed to broadly applicable research endeavors. As a result, we cannot state the costs incurred for each of our programs on a program-by-program basis.

For the nine months ended September 30, 2020, our clinical stage projects included a Phase 1 clinical trial with Ad-RTS-IL-12 plus veledimex in progressive glioblastoma; a Phase 1 clinical trial infusing our 2nd generation CD19-specific CAR^+ T cells in patients with advanced lymphoid malignancies; and a Phase 1/2 clinical trial of Ad-RTS-hIL-12 with veledimex for the treatment of pediatric brain tumors. The expenses incurred by us to third parties for our Phase 1 clinical trial with Ad-RTS-IL-12 plus veledimex in progressive glioblastoma were $2.5 million for the nine months ended September 30, 2020 and $13.8 million from the project's inception in September 2015 through September 30, 2020. The expenses incurred by us to third parties for our Phase 1 clinical trial infusing our 2nd generation CD19-specific CAR^+ T cells in patients with advanced lymphoid malignancies were $0.1 million for the nine months ended September 30, 2020 and $6.2 million from the project's inception in December 2015 through September 30, 2020. The expenses incurred by us to third parties for our Phase 1/2 clinical trial of Ad-RTS-hIL-12 with veledimex for the treatment of pediatric brain tumors were $0.1 million for the nine months ended September 30, 2020 and $2.1 million from the project's inception in October 2017 through September 30, 2020. The expense incurred by us to third parties for our investigator-led Phase 2 clinical trial of Ad-RTS-hIL-12 with veledimex in combination with cemiplimab-rwlc in progressive glioblastoma were $3.8 million for the nine months ended September 30, 2020 and $5.4 million from the projects inception in June 2019 through September 30, 2020.

Our future research and development expenses in support of our current and future programs will be subject to numerous uncertainties in timing and cost to completion. We test potential products in numerous preclinical studies for safety, toxicology and efficacy. We may conduct multiple clinical trials for each product. As we obtain results from trials, we may elect to discontinue or delay clinical trials for certain products to focus our resources on more promising products or indications. Completion of clinical trials may take several years or more, and the length of time generally varies substantially according to the type, complexity, novelty and intended use of a product. It is not unusual for preclinical and clinical development of each of these types of products to require the expenditure of substantial resources.

We estimate that clinical trials of the type generally needed to secure new drug approval are typically completed over the following timelines:

Clinical Phase	Estimated Completion Period
Phase 1	1 - 2 years
Phase 2	2 - 3 years
Phase 3	2 - 4 years

The duration and the cost of clinical trials may vary significantly over the life of a project as a result of differences arising during clinical development, including, among others, the following:

- The number of clinical sites included in the trials;
- The length of time required to enroll suitable patents;
- The number of patients that ultimately participate in the trials;
- The cost to manufacture the clinical products for patients;
- The duration of patient follow-up to ensure the absence of long-term product-related adverse events; and
- The efficacy and safety profile of the product.

As a result of the uncertainties discussed above, we are unable to determine the duration and completion costs of our programs or when and to what extent we will receive cash inflows from the commercialization and sale of a product. Our inability to complete our programs in a timely manner or our failure to enter into appropriate collaborative agreements could significantly increase our capital requirements and could adversely impact our liquidity. These uncertainties could force us to reduce or eliminate our activities in one or more of our programs or seek additional, external sources of financing from time-to-time in order to continue with our product development strategy. Our inability to raise additional capital, or to do so on terms reasonably acceptable to us, would jeopardize the future success of our business.

General and administrative expenses. General and administrative expenses during the three and nine months ended September 30, 2020 and 2019 were as follows:

	Three months ended September 30,				Nine months ended September 30,			
	2020	2019	Change		2020	2019	Change	
($ in thousands)								
General and administrative	$ 6,353	$ 4,807	$1,546	32%	$18,862	$13,707	$5,155	38%

General and administrative expenses for the three months ended September 30, 2020 increased by $1.5 million as compared to three months ended September 30, 2019. The increase during the three months ended September 30, 2020 was primarily due to an increase of $0.8 million of legal expenses incurred as a result of our expanded patent portfolio and an increase of $0.7 million of salary and employee related expenses, including stock compensation expense.

General and administrative expenses for the nine months ended September 30, 2020 increased by $5.2 million as compared to nine months ended September 30, 2019. The increase during the nine months ended September 30, 2020 was primarily due to an increase of $2.5 million of salary and employee related expenses, including stock compensation expense, along with an increase of $2.0 million of legal expenses incurred as a result of our expanded patent portfolio, and an increase of $1.4 million in facilities expenses, offset by a decrease of $0.7 million of business development expenses.

Other income, (net). Other income, net for the three and nine months ended September 30, 2020 and 2019 was as follows:

	Three months ended September 30,				Nine months ended September 30,			
	2020	2019	Change		2020	2019	Change	
($ in thousands)								
Other income, net	$ 6	$ 203	$ (197)	(97%)	$383	$ 523	$ (140)	(27%)
Noncash inducement warrants	—	(60,751)	$60,751	(100%)	—	(60,751)	$60,751	(100%)
Total	$ 6	$ (60,548)			$383	$(60,228)		

Other income for the three months ended September 30, 2020 decreased by $0.2 million as compared to the three months ended September 30, 2019 because interest rates decreased due to market fluctuations. Other income for the nine months ended September 30, 2020 decreased by $0.1 million as compared to the nine months ended September 30, 2019 because interest rates decreased due to market fluctuations.

On July 26, 2019 and September 12, 2019, we entered into agreements with existing investors for the exercise of previously issued 2018 warrants. Pursuant to the terms of the agreements, we issued the investors new warrants to purchase an aggregate of 17,803,031 shares of common stock, as an inducement to exercise their 2018 warrants early. The 2019 warrants became exercisable six months following the date of issuance, will expire on the fifth anniversary of the initial exercise date, and have an exercise price of $7.00. The Black-Scholes valuation of the 2019 warrants resulted in a non-cash charge to the statement of operations of $60.8 million for the three and nine months ended September 30, 2019.

Liquidity and Capital Resources

Source of liquidity

We have not generated any revenue from product sales. Since inception, we have incurred net losses and negative cash flows from our operations.

As of September 30, 2020, we have approximately $135.5 million of cash and cash equivalents. During the nine months ended September 30, 2020, we completed an underwritten public offering of 29,110,111 shares of common stock, which includes a partial exercise by the underwriters of its over-allotment option of 1,284,025 shares from us at a price to the public of $3.25, less underwriting discounts. Our net proceeds from the sale of the shares, after deducting the underwriting discounts and offering expenses of $5.9 million, were $88.7 million.

At-the-market offering program

In June 2019, we entered into an Open Market Sale Agreement, or sales agreement, with Jefferies LLC, or Jefferies, as a sale agent pursuant to which we may offer and sell, from time to time through Jefferies, shares of our common stock having an aggregate offering value of up to $100.0 million. Shares will be sold pursuant to the Company's effective registration statement on Form S-3ASR (File No. 333-232283), as previously filed with the Securities and Exchange Commission. Subject to the terms of the sales agreement, we are able to determine, at our sole discretion, the timing and number of shares to be sold under this ATM facility. The compensation to Jefferies for sales of our common stock pursuant to the sales agreement will be an amount equal to 3% of the gross proceeds of any shares of common stock sold under the sales agreement. During the nine months ended September 30, 2020, we issued and sold 2,814,673 shares of common stock under the sales agreement for aggregate net proceeds of $13.0 million, after deducting commission and offering expenses of $0.4 million. During the nine months ended September 30, 2019, we issued and sold 639,442 shares of common stock under the sales agreement for aggregate net proceeds of $3.0 million, after deducting commission and offering expenses of $0.1 million.

Funding requirements

Given our current development plans, we expect that our existing cash and cash equivalents will be sufficient to fund our current operations into mid-2022. We currently do not have any committed sources of additional capital at this time. The forecast of cash resources is forward-looking information that involves risks and uncertainties, and the actual amount of our expenses could vary materially and adversely as a result of a number of factors. We have based our estimates on assumptions that may prove to be wrong, and our expenses could prove to be significantly higher than we currently anticipate. Management does not know whether additional financing will be on terms favorable or acceptable to us when needed, if at all. In addition, we have issued or reserved for future issuance shares nearing the maximum number of shares of common stock authorized by our certificate of incorporation. If we are unable to increase the total number of authorized shares, we may be unable to effectively utilize our common stock to raise capital. If adequate additional funds are not available when required, or if we are unsuccessful in entering into partnership agreements for further development of our products, management may need to curtail development efforts. The COVID-19 pandemic continues to rapidly evolve and has already resulted in a significant disruption of global financial markets. If the disruption persists and deepens, we could experience an inability to access additional capital when and if needed. If we are unable to obtain funding, we could be forced to delay, reduce or eliminate some or all of our research and development programs, product portfolio expansion or commercialization efforts, which would adversely affect our business prospects, or we may be unable to continue operations.

In addition to these factors, our actual cash requirements may vary materially from our current expectations due to a number of other factors that may include, but are not limited to, changes in the focus and direction of our development programs, competitive and technical advances, costs associated with the development of our product candidates, our ability to secure partnering arrangements, and the costs of filing, prosecuting, defending and enforcing our intellectual property rights. If we exhaust our capital reserves more quickly than anticipated, regardless of the reason, and we are unable to obtain additional financing on terms acceptable to us or at all, we will be unable to proceed with development of some or all of our product candidates on expected timelines and will be forced to prioritize among them.

Cash flows

The following table summarizes our net decrease in cash, cash equivalents, and restricted cash for the nine months ended September 30, 2020:

	Nine months ended September 30,	
($ in thousands)	**2020**	**2019**
Net cash provided by (used in):		
Operating activities	$ (39,977)	$ (29,246)
Investing activities	(6,012)	(184)
Financing activities	101,719	56,120
Net increase in cash, cash equivalents, and restricted cash	$ 55,730	$ 26,690

Cash flows from operating activities represent the cash receipts and disbursements related to all of our activities other than investing and financing activities. Operating cash flow is derived by adjusting our net loss for:

- Non-cash operating items such as depreciation and stock-based compensation; and
- Changes in operating assets and liabilities, which reflect timing differences between the receipt and payment of cash associated with transactions and when they are recognized in results of operations.

Net cash used in operating activities for the nine months ended September 30, 2020 was $40.0 million, as compared to net cash used in operating activities of $29.2 million for the nine months ended September 30, 2019. The net cash used in operating activities for the nine months ended September 30, 2020 was primarily due to our net loss of $57.2 million, the change in receivables of $2.1 million, other noncurrent assets of $0.6 million, offset by the change in prepaid and other current assets of $8.2 million primarily related to the use of our funds at MD Anderson, the change in accrued expenses of $3.8 million, the change in accounts payable of $1.7 million and non-cash stock-based compensation on $5.4 million.

Net cash used in investing activities was $6.0 million for the nine months ended September 30, 2020 compared to $0.2 million for the nine months ended September 30, 2019.

Net cash provided by financing activities the nine months ended September 30, 2020 was $101.7 million. The net cash was provided by $88.7 million from the issuance of common stock in our follow-on public offering, net and $13.0 million from the issuance of common stock pursuant to our ATM facility. Net cash provided by financing activities for the nine months ended September 30, 2019 was $56.1 million. During the quarter, we received $52.5 million in proceeds from the exercise of warrants (Note 2), $3.0 million in proceeds through ATM offerings (Note 2) and $1.0 million in proceeds from the exercise of stock options.

Operating capital and capital expenditure requirements

We anticipate that losses will continue for the foreseeable future. At September 30, 2020, our accumulated deficit was approximately $741.3 million. Our actual cash requirements will depend on and could increase significantly as a result of a number of factors, including:

- the scope, number, initiation, progress, timing, costs, design, duration, any potential delays, and results of clinical trials and nonclinical studies for our current or future product candidates;
- changes in the focus, direction and pace of our development programs;
- the effect of competitive and technical advances and market developments;
- costs associated with the development of our product candidates;
- our ability to establish and maintain partnering, collaborations or similar arrangements on favorable terms and whether and to what extent we retain development or commercialization responsibilities under any new licensing, collaboration or similar arrangement;
- diversion of healthcare resources away from the conduct of clinical trials as a result of the ongoing COVID-19 pandemic, including the diversion of hospitals serving as our clinical trial sites and hospital staff supporting the conduct of our clinical trials;
- the interruption of key clinical trial activities, such as clinical trial site monitoring, due to limitations on travel, quarantines or social distancing protocols imposed or recommended by federal or state governments, employers and others in connection with the ongoing COVID-19 pandemic;
- our need and ability to hire additional management and scientific and medical personnel;
- the costs of acquiring, licensing or investing in businesses, product candidates and technologies;
- costs of filing, prosecuting, defending and enforcing any patent claims and any other intellectual property rights, or other developments; and
- other matters identified under Part II, Item 1A. “Risk Factors.”

Working capital as of September 30, 2020 was $135.8 million, consisting of $155.1 million in current assets and $19.3 million in current liabilities. Working capital as of December 31, 2019 was $93.0 million, consisting of $105.5 million in current assets and $12.5 million in current liabilities.

Contractual Obligations

The following table summarizes our outstanding obligations as of September 30, 2020 and the effect those obligations are expected to have on our liquidity and cash flows in future periods:

($ in thousands)	Total	Less than 1 year	2 - 3 years	4 - 5 years	More than 5 years
Operating leases	$3,157	$ 1,128	$ 712	$ 755	$ 562
CRADA	3,125	2,500	625	—	—
Royalty and license fees	3,461	434	700	700	1,627
Total	$9,743	$ 4,062	$ 2,037	$ 1,455	$ 2,189

Our commitments for operating leases relate to the lease for our corporate headquarters in Boston, Massachusetts, and office and laboratory space in Houston, Texas. On December 21, 2015 and April 15, 2016, we renewed the sublease for our corporate headquarters in Boston, Massachusetts through August 31, 2021. On March 12, 2019, we entered into a lease agreement for additional office space in Houston through April 2021. On October 15, 2019, we entered into another lease agreement for additional office and laboratory space in Houston through February 2027. On April 13, 2020, we entered into another lease agreement for additional office and laboratory space in Houston through February 2027. On June 1, 2020, we entered into a short-term lease in Houston for office and laboratory space. On September 1, 2020, we entered an additional short-term lease in Houston for additional office and laboratory space.

On January 10, 2017, we announced the signing of the CRADA with the NCI for the development of ACT-based immunotherapies genetically modified using the *Sleeping Beauty* transposon/transposase system for the treatment of solid tumors. In February 2019, we extended the CRADA with the NCI until January 9, 2022.

On October 5, 2018, we entered into the License Agreement with PGEN. Under the License Agreement, we are obligated to pay PGEN an annual licensing fee of $0.1 million expected to be paid through the term of the agreement.

On May 28, 2019, we entered into a patent license agreement, or the Patent License, with the NCI. The terms of the Patent License require us to pay the NCI minimum annual royalties in the amount of $0.3 million, which amount will be reduced to $0.1 million once the aggregate minimum annual royalties paid by us equals $1.5 million. The first minimum annual royalty payment is payable on the date that is eighteen months following the date of the Patent License.

On January 8, 2020, we entered into an amendment of Patent License agreement. Under this agreement, we paid $0.6 million. On September 28, 2020, we entered into a second amendment of the Patent License with the NCI. The terms of the second Amendment require us to pay the NCI a one-time payment of $0.4 million, which is included in the “less than 1-year” category in the chart above.

Off-balance Sheet Arrangements

We did not have during the periods presented, and we do not currently have, any off-balance sheet arrangements, as defined in the rules and regulations of the SEC.

Critical Accounting Policies and Estimates

In our Annual Report, our most critical accounting policies and estimates upon which our financial status depends were identified as those relating to clinical trial expenses; collaboration agreements; fair value measurements for stock-based compensation; and income taxes. We reviewed our policies and determined that those policies remain our most critical accounting policies for the nine months ended September 30, 2020.

Item 3. Quantitative and Qualitative Disclosures about Market Risk

Our exposure to market risk is limited to our cash. The goals of our investment policy are preservation of capital, fulfillment of liquidity needs and fiduciary control of cash. We also seek to maximize income from our investments without assuming significant risk. To achieve our goals, we maintain our cash in interest-bearing bank accounts in global banks, United States treasuries and other government-backed investments, which are subject to minimal interest rate risk.

Effect of Currency Exchange Rates and Exchange Rate Risk Management

We currently have no clinical studies or clinical trials taking place outside of the United States. Therefore, any currency fluctuations will not have a material impact on our financial position, results of operations or cash flows.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Under the supervision and with the participation of our management, including our Chief Executive Officer and Chief Financial Officer, we have evaluated the effectiveness of our disclosure controls and procedures, as such term is defined under Rule 13a-15(e) or 15d-15(e) promulgated under the Exchange Act, as of September 30, 2020. Based on that evaluation, our Chief Executive Officer and Chief Financial Officer have concluded that as of September 30, 2020, our disclosure controls and procedures were not effective due to a material weakness identified in our internal control over financial reporting as described below under "Management's Report on Internal Control over Financial Reporting".

Management's Report on Internal Control over Financial Reporting

Our management is responsible for establishing and maintaining adequate internal control over financial reporting for us. Internal control over financial reporting (as defined in Rule 13a-15(f) of the Exchange Act) is a process to provide reasonable assurance regarding the reliability of our financial reporting for external purposes in accordance with accounting principles generally accepted in the United States. Internal control over financial reporting includes maintaining records that in reasonable detail accurately and fairly reflect our transactions; providing reasonable assurance that transactions are recorded as necessary for preparation of our financial statements; providing reasonable assurance that receipts and expenditures of company assets are made in accordance with management authorization; and providing reasonable assurance that unauthorized acquisition, use or disposition of company assets that could have a material effect on our financial statements would be prevented or detected on a timely basis. Because of its inherent limitations, internal control over financial reporting is not intended to provide absolute assurance that a misstatement of our financial statements would be prevented or detected.

As of December 31, 2019, Management conducted an evaluation of the effectiveness of our internal control over financial reporting based on the framework in *Internal Control—Integrated Framework* issued by the Committee of Sponsoring Organizations of the Treadway Commission in 2013.

As previously disclosed under "Item 9A. Controls and Procedures" in our Annual Report on Form 10-K for our fiscal year ended December 31, 2019, we identified the following deficiency that existed as of December 31, 2019 which continued to exist at September 30, 2020. This deficiency represented a material weakness in our internal control over financial reporting. A material weakness is a control deficiency or a combination of control deficiencies that results in more than a remote likelihood that a material misstatement of the annual or interim financial statements will not be prevented or detected.

As of December 31, 2019 and September 30, 2020, management identified a material weakness in the design and effectiveness of our internal control over financial reporting. We did not design and maintain effective controls relating to the monitoring and oversight of expensing third party clinical trial costs. Specifically, our internal controls were not designed effectively to provide reasonable assurance regarding the accurate and timely evaluation of the amount of third-party costs to record.

Based on this evaluation, management concluded that our internal control over financial reporting was not effective at September 30, 2020 because of the material weakness described above.

Despite the existence of the material weakness described above, our financial statements as of September 30, 2020, are presented fairly, in all material respects, in conformity with accounting principles generally accepted in the United States of America.

Remediation

We have implemented measures to remediate the control deficiency that constituted the above material weakness by implementing changes to our internal control over financial reporting. We have designed, implemented and tested measures to work towards remediating the underlying causes of the control deficiency that gave rise to the material weakness. In addition, we have provided in-house accounting personnel training to ensure that they have the relevant expertise related to the monitoring and oversight of expensing third party clinical trial costs. We will continue to monitor the effectiveness of these controls and will make any further changes management determines appropriate.

Changes in Internal Controls over Financial Reporting

Except for the material weakness and related remediation efforts discussed above, there were no other changes in our internal control over financial reporting (as defined in Rule 13a-15(f) of the Exchange Act) that occurred during the fiscal quarter ended September 30, 2020 that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

Part II - Other Information

Item 1. Legal Proceedings

In the ordinary course of business, we may periodically become subject to legal proceedings and claims arising in connection with ongoing business activities from time to time. The results of litigation and claims cannot be predicted with certainty, and unfavorable resolutions are possible and could materially affect our results of operations, cash flows or financial position. In addition, regardless of the outcome, litigation could have an adverse impact on us because of defense costs, diversion of management attention and resources and other factors.

As of September 30, 2020, based on information readily available, there are no material matters that, in the opinion of management, are likely to result in a material adverse effect on our financial position, results of operations or cash flows.

Item 1A. Risk Factors

The following important factors could cause our actual business and financial results to differ materially from those contained in forward-looking statements made in this Quarterly Report on Form 10-Q or elsewhere by management from time to time. The risk factors in this Quarterly Report have been revised to incorporate changes to our risk factors from those included in our Annual Report on Form 10-K for the fiscal year ended December 31, 2019. The risk factors set forth below with an asterisk (*) next to the title are new risk factors or risk factors containing changes, which may be material, from the risk factors previously disclosed in Item 1A of our Annual Report on Form 10-K for the fiscal year ended December 31, 2019, as filed with the Securities and Exchange Commission. The market price of our common stock could decline if one or more of these risks or uncertainties actually occur, causing you to lose all or part of your investment. The impact of COVID-19 may also exacerbate other risks discussed in this filing, any of which could have a material effect on us. This situation is changing rapidly and additional impacts may arise. Additional risks that we currently do not know about, or that we currently believe to be immaterial, may also impair our business. Certain statements below are forward-looking statements. See “Cautionary Note Regarding Forward-Looking Statements” in this Quarterly Report.

RISKS RELATED TO OUR BUSINESS

* ***Our business, operations and clinical development plans and timelines could be adversely affected by the effects of health epidemics, including the COVID-19 pandemic, on the manufacturing, clinical trial and other business activities performed by us or by third parties with whom we conduct business, including our contract manufacturers, clinical research organizations, or CROs, shippers and others.***

Our business could be adversely affected by health epidemics wherever we have clinical trial sites or other business operations. In addition, health epidemics could cause significant disruption in the operations of third-party manufacturers, CROs and other third parties upon whom we rely. For instance, NCI has taken precautionary measures in response to the COVID-19 pandemic that has delayed the commencement of the clinical trial under the CRADA and MDACC may take precautionary measures in the future that may delay enrollment of patients in our ongoing and planned clinical trials.

We have implemented work-from-home policies for most of our employees. The effects of our work-from-home policies and travel restrictions may negatively impact productivity, disrupt our business and delay our clinical programs and timelines, the magnitude of which will depend, in part, on the length and severity of the restrictions and other limitations on our ability to conduct our business in the ordinary course. These and similar, and perhaps more severe, disruptions in our operations could negatively impact our business, operating results and financial condition.

We depend on a worldwide supply chain to manufacture products used in our preclinical studies and clinical trials. Quarantines, shelter-in-place and similar government orders, or the expectation that such orders, shutdowns or other restrictions could occur, whether related to COVID-19 or other infectious diseases, could impact personnel at third-party manufacturing facilities in the United States and other countries, or the availability or cost of materials, which could disrupt our supply chain.

If our relationships with our suppliers or other vendors are terminated or scaled back as a result of the COVID-19 pandemic or other health epidemics, we may not be able to enter into arrangements with alternative suppliers or vendors or do so on commercially reasonable terms or in a timely manner. Switching or adding additional suppliers or vendors involves substantial cost and requires management time and focus. In addition, there is a natural transition period when a new supplier or vendor commences work. As a result, delays may occur, which could adversely impact our ability to meet our desired clinical development and any future commercialization timelines. Although we carefully manage our relationships with our suppliers and vendors, there can be no assurance that we will not encounter challenges or delays in the future or that these delays or challenges will not harm our business.

In addition, our preclinical studies and clinical trials have been and may continue to be affected by the COVID-19 pandemic. Clinical site initiation, patient enrollment and activities that require visits to clinical sites, including data monitoring, have been and may continue to be delayed due to prioritization of hospital resources toward the COVID-19 pandemic or concerns among patients about participating in clinical trials during a pandemic. Some patients may have difficulty following certain aspects of clinical trial protocols if quarantines impede patient movement or interrupt healthcare services. Similarly, if we are unable to successfully recruit and retain patients and principal investigators and site staff who, as healthcare providers, may have heightened exposure to COVID-19 or experience additional restrictions by their institutions, city, or state our clinical trial operations could be adversely impacted.

The spread of COVID-19, which has caused a broad impact globally, may materially affect us economically. While the potential economic impact brought by, and the duration of, COVID-19 may be difficult to assess or predict, a widespread pandemic could result in significant disruption of global financial markets, reducing our ability to access capital, which could in the future negatively affect our liquidity. In addition, a recession or market correction resulting from the spread of COVID-19 could materially affect our business and the value of our common stock.

The global COVID-19 pandemic continues to evolve rapidly. The ultimate impact of the COVID-19 pandemic or a similar epidemic is highly uncertain and subject to change. We may experience a material impact on our operations, and we continue to monitor the COVID-19 situation closely.

* ***We will require substantial additional financial resources to continue ongoing development of our product candidates and pursue our business objectives; if we are unable to obtain these additional resources when needed, we may be forced to delay or discontinue our planned operations, including clinical testing of our product candidates.***

We have not generated significant revenue and have incurred significant net losses in each year since our inception. For the nine months ended September 30, 2020, we had a net loss of $57.2 million, and, as of September 30, 2020 we have incurred approximately $741.3 million of accumulated deficit since our inception in 2003. We expect to continue to incur significant operating expenditures and net losses. Further development of our product candidates will likely require substantial increases in our expenses as we:

- continue to undertake clinical trials for product candidates;
- scale-up the formulation and manufacturing of our product candidates;
- seek regulatory approvals for product candidates;
- work with regulatory authorities to identify and address program-related inquiries;
- implement additional internal systems and infrastructure; and
- hire additional personnel.

As of September 30, 2020, we have approximately $135.5 million of cash and cash equivalents.

Given our current development plans, we anticipate our cash resources will be sufficient to fund our operations into mid-2022 and we have no committed sources of additional capital at this time. The forecast of cash resources is forward-looking information that involves risks and uncertainties, and the actual amount of our expenses could vary materially and adversely as a result of a number of factors. We have based our estimates on assumptions that may prove to be wrong, and our expenses could prove to be significantly higher than we currently anticipate. Management does not know whether additional financing will be on terms favorable or acceptable to us when needed, if at all.

Our actual cash requirements may vary materially from our current expectations for a number of other factors that may include, but are not limited to, changes in the focus and direction of our development programs, slower than expected progress of our research and development efforts, changes in governmental regulation, competitive and technical advances, costs associated with the development of our product candidates, our ability to secure partnering arrangements, and costs of filing, prosecuting, defending and enforcing our intellectual property rights. The COVID-19 pandemic continues to rapidly evolve and has already resulted in a significant disruption of global financial markets. If the disruption persists and deepens, we could experience an inability to access additional capital, which could in the future negatively affect our operations. If we exhaust our capital reserves more quickly than anticipated, regardless of the reason, and we are unable to obtain additional financing on terms acceptable to us or at all, we will be unable to proceed with development of some or all of our product candidates on expected timelines and will be forced to prioritize among them. Further, we may elect to prioritize one or more of our programs and reduce or eliminate our activities on our other programs to preserve our capital resources. Any decision to reduce or eliminate activities for a program may negatively impact the potential for the program, which could have a material adverse effect on our business.

* ***We need to raise additional capital to fund our operations. The manner in which we raise any additional funds may affect the value of your investment in our common stock.***

Until such time, if ever, as we can generate substantial revenue, we expect to finance our cash needs through a combination of equity offerings, debt financings and license and collaboration agreements. We do not have any committed external source of funds. The unpredictability of the capital markets may severely hinder our ability to raise capital within the time periods needed or on terms we consider acceptable, if at all. In addition, the recent outbreak of the novel coronavirus known as COVID-19 has significantly disrupted world financial markets, negatively impacted U.S. market conditions and may reduce opportunities for us to seek out additional funding. In particular, a decline in the market price of our common stock could make it more difficult for us to sell equity or equity-related securities in the future at a time and price that we deem appropriate. Moreover, if we fail to advance one or more of our current product candidates into early or later-stage clinical trials, successfully commercialize one or more of our product candidates, or acquire new product candidates for development, we may have difficulty attracting investors that might otherwise be a source of additional financing.

To the extent that we raise additional capital by issuing equity securities, our existing stockholders' ownership will be diluted, and the terms of these securities may include liquidation or other preferences that adversely affect the rights of our common stockholders. Debt financing and preferred equity financing, if available, may involve agreements that include covenants limiting or restricting our ability to take specific actions, such as incurring additional debt, making capital expenditures or declaring dividends. Any debt financing that we enter into may involve covenants that restrict our operations. These restrictive covenants may include limitations on additional borrowing and specific restrictions on the use of our assets as well as prohibitions on our ability to create liens, pay dividends, redeem our stock or make investments. Furthermore, the impact of COVID-19 on global financial markets could make the terms of any available financing less attractive to use and more dilutive to our existing shareholders. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, limit, reduce or terminate our product development or future commercialization efforts or grant rights to develop and market product candidates that we would otherwise prefer to develop and market ourselves. If we raise additional funds through collaborations, strategic alliances or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us.

* ***We have issued or reserved for future issuance shares nearing the maximum number of shares of common stock authorized by our certificate of incorporation. If we are unable to increase the total number of authorized shares, we may be unable to effectively utilize our common stock to establish strategic relationships with other companies, expand our business through acquisitions, raise capital, or offer equity incentives to employees.***

Our amended and restated certificate of incorporation authorizes us to issue 250,000,000 shares of common stock. As of September 30, 2020, there were 214,165,690 shares of common stock outstanding and an additional 29,708,251 shares of common stock reserved for issuance pursuant to outstanding stock options and warrants. Though we have no immediate plans to issue additional shares of common stock, other than in connection with our 2020 Equity Incentive Plan, we may need additional shares for business and financial purposes in the future. For example, we will need additional shares of authorized common stock to raise capital to, among other things, fund our operations, conduct and/or complete clinical trials, continue our research and development activities, seek regulatory approval for our product candidates and commercialize our product candidates. In addition, we may wish to issue additional shares in connection with entering into future strategic relationships, or acquiring other businesses, therapeutics or product candidates. Furthermore, our success depends, in part, on our continued ability to attract, retain and motivate highly qualified management and clinical and scientific personnel, and the lack of available unissued shares may also have an adverse impact on our to provide appropriate equity incentives to employees, officers, directors, consultants and/or advisors. If we are unable to increase the total number of authorized shares available to us, our business development and financing opportunities may be limited, and stockholder value may be harmed.

Our plans to develop and commercialize non-viral and viral adoptive cellular therapies based on engineered cytokines and CAR T-cell as well as TCR therapies can be considered as new approaches to cancer treatment, the successful development of which is subject to significant challenges.

We intend to employ technologies such as the technology licensed from MD Anderson pursuant to the MD Anderson License described above, and from PGEN, pursuant to the License Agreement, to pursue the development and commercialization of non-viral and viral adoptive cellular therapies based on cytokines, T-cells, CARs and TCRs, possibly under control of the RTS® and other switch technologies targeting both hematologic and solid tumor malignancies. Because this is a new approach to cancer immunotherapy and cancer treatment generally, developing and commercializing product candidates subjects us to a number of challenges, including:

- obtaining regulatory approval from the FDA and other regulatory authorities that have very limited experience with the commercial development of genetically modified and/or unmodified T-cell therapies for cancer;
- identifying and manufacturing appropriate TCRs from patient and from third parties that can be administered to a patient;
- developing and deploying consistent and reliable processes for engineering a patient's and/or donor's T-cells *ex vivo* and infusing the T-cells back into the patient;
- possibly conditioning patients with chemotherapy in conjunction with delivering each of the potential products, which may increase the risk of adverse side effects of the potential products;
- educating medical personnel regarding the potential side effect profile of each of the potential products, such as the potential adverse side effects related to cytokine release;
- addressing any competing technological and market developments;
- developing processes for the safe administration of these potential products, including long-term follow-up for all patients who receive the potential products;
- sourcing additional clinical and, if approved, commercial supplies for the materials used to manufacture and process the potential products;

- developing a manufacturing process and distribution network with a cost of goods that allows for an attractive return on investment;
- establishing sales and marketing capabilities after obtaining any regulatory approval to gain market acceptance;
- developing therapies for types of cancers beyond those addressed by the current potential products;
- maintaining and defending the intellectual property rights relating to any products we develop;
- and not infringing the intellectual property rights, in particular, the patent rights, of third parties, including competitors, such as those developing T-cell therapies.

We cannot assure you that we will be able to successfully address these challenges, which could prevent us from achieving our research, development and commercialization goals.

Our current product candidates are based on novel technologies and are supported by limited clinical data and we cannot assure you that our current and planned clinical trials will produce data that supports regulatory approval of one or more of these product candidates.

The immuno-oncology effector platform in which we have acquired rights pursuant to our License Agreement with PGEN represents early-stage technology in the field of human oncology biotherapeutics, with Ad-RTS-IL-12 plus veledimex having completed trials, in melanoma, breast cancer and rGBM. Similarly, our genetically modified and/ or non-modified T-cell candidates are supported by limited clinical data, all of which has been generated through trials conducted by MD Anderson and the NCI, not by us. We plan to assume control of the overall clinical and regulatory development of our T-cell product candidates, and any failure to obtain, or delays in obtaining, sponsorship of new INDs, or in filing INDs sponsored by us for these or any other product candidates we determine to advance could negatively affect the timing of our potential future clinical trials. Such an impact on timing could increase research and development costs and could delay or prevent obtaining regulatory approval for our product candidates, either of which could have a material adverse effect on our business.

Further, we did not control the design or conduct of the previous trials. It is possible that the FDA will not accept these previous trials as providing adequate support for future clinical trials, whether controlled by us or third parties, for any of one or more reasons, including the safety, purity, and potency of the product candidate, the degree of product characterization, elements of the design or execution of the previous trials or safety concerns, or other trial results. We may also be subject to liabilities arising from any treatment-related injuries or adverse effects in patients enrolled in these previous trials. As a result, we may be subject to unforeseen third-party claims and delays in our potential future clinical trials. We may also be required to repeat in whole or in part clinical trials previously conducted by MD Anderson or other entities, which will be expensive and delay the submission and licensure or other regulatory approvals with respect to any of our product candidates.

In addition, the results of the limited clinical trials conducted to date may not be replicated in future clinical trials. Our Ad-RTS-IL-12 plus veledimex and genetically modified and non-modified T-cell product candidates, as well as other product candidates, may fail to show the desired safety and efficacy in clinical development, and we cannot assure you that the results of any future trials will demonstrate the value and efficacy of our product candidates. Moreover, there are a number of regulatory requirements that we must satisfy before we can continue clinical trials of CAR+ T, TCRs or other cellular

therapy product candidates in the United States. Satisfaction of these requirements will entail substantial time, effort and financial resources. Any time, effort and financial resources we expend on our Ad-RTS-IL-12 plus veledimex and genetically modified and non-modified T-cell product candidates and other early-stage product candidate development programs may adversely affect our ability to continue development and commercialization of our immuno-oncology product candidates.

We report interim data on certain of our clinical trials and we cannot assure you that interim data will be predictive of either future interim results or final study results.

As part of our business, we provide updates related to the development of our product candidates, which may include updates related to interim clinical trial data. To date, our clinical trials have involved small patient populations and because of the small sample size, the interim results of these clinical trials may be subject to substantial variability and may not be indicative of either future interim results or final results.

* ***We face substantial competition from other biopharmaceutical companies, which may result in others discovering, developing or commercializing products before, or more successfully than, we do.***

The development and commercialization for new products to treat cancer, including the indications we are pursuing, is highly competitive and considerable competition exists from major pharmaceutical, biotechnology and specialty cancer companies. Many of these companies have more experience in preclinical and clinical development, manufacturing, regulatory, and global commercialization. We are also competing with academic institutions, governmental agencies, and private organizations that are conducting research in the field of cancer.

Our genetically engineering T-cell programs face significant competition in the CAR and TCR technology space from multiple companies and their collaborators. Two such companies, Novartis International AG (Kymriah®) and Kite Pharma Inc./Gilead Sciences, Inc. (Yescarta®), have now commercialized autologous CAR+ T cells against CD19. Additional companies developing autologous CAR+ T targets include Bristol-Myers Squibb Company, Precigen, Inc., bluebird bio, Inc., in collaboration with Celgene Corporation, Nanjing Legend Biotech and Janssen Biotech, Inc., a subsidiary of Johnson & Johnson, Bellicum Pharmaceuticals, Inc., Autolus Therapeutics plc, Exuma Biotech Corp., CARsgen Therapeutics Co., Ltd., Mustang Bio, Inc., Crispr Therapeutics AG, Precision Biosciences Inc., Protheragen Inc. and Marker Therapeutics, Inc. Several companies are pursuing the development of allogeneic CAR+ T therapies, including Allogene Therapeutics, Inc. (in collaboration with Pfizer Inc.), Atara Biotherapeutics, Inc. and Cellectis SA (in collaboration with Servier) which may compete with our product candidates.

Our TCR program faces competition from several companies, including from Adaptimmune Therapeutics plc in collaboration with GlaxoSmithKline plc, ArsenalBio, Lyell, bluebird bio, Kite Pharma Inc./Gilead Sciences, Inc., Achilles Therapeutics Limited, Iovance Biotherapeutics, Inc., Immatics Biotechnologies GmbH, Tmunity Therapeutics Inc, Medigene AG, Tactiva Therapeutics, LLC, Takara Bio, Inc., TC Biopharm Ltd., TCR2 Therapeutics Inc., Zelluna Immunotherapy AS, PACT Pharma, Inc. and others. Several companies, including Advaxis Inc./Amgen Inc., BioNTech AG and Gritstone Oncology, Inc., are pursuing vaccine platforms to target neoantigens for solid tumors. Other companies are developing non-viral gene therapies, including Poseida Therapeutics, Inc. and several companies developing CRISPR technology. We also face competition from companies developing therapies using cells other than T cells such as Takeda Pharmaceutical Company, Incysus Therapeutics, Inc., and TC BioPharm Limited. We also face competition from companies developing T cells with cytokines such as Repertoire Immune Medicines and Obsidian Therapeutics, Inc. We also face competition from non-cell- based treatments offered by other companies such as Amgen Inc., AstraZeneca plc, Bristol-Myers Squibb Company, Incyte Corporation, Merck & Co., Inc., and Roche Holding AG.

We are initially developing our Controlled IL-12 platform for the treatment of rGBM. Companies that sell marketed drugs for rGBM are Genentech Inc. and Roche Holding AG with Avastin (bevacizumab), a vascular endothelial growth factor directed antibody indicated for the treatment of adults with rGBM. Arbor Pharmaceuticals Inc. markets GLIADEL Wafer, which is indicated in patients with newly diagnosed high-grade malignant glioma as an adjunct to surgery and radiation and is also indicated in patients with recurrent glioblastoma multiforme as an adjunct to surgery. Additionally, Novocure has developed Optune (tumor treating fields) for newly diagnosed and recurrent glioblastoma. Several companies have product candidates in Phase 3 development for the treatment of glioblastoma, including, but not limited to, Vascular Biogenics Ltd. and Kintra Therapeutics. Several companies and institutions have product candidates currently in Phase 2 clinical trials, including, but not limited to, Abbvie Inc., DNAtrix Therapeutics, Istari Oncology, Karyopharm and MedImmune LLC/AstraZeneca plc, and other companies are actively developing additional products to treat brain cancer including Mustang Bio Inc. and Northwest Biotherapeutics, Inc. Other competitors with product candidates currently in Phase 2 clinical trials include AbbVie Inc.'s Depatus-M (ABT-414) and DNA-2401, a conditionally replicative adenovirus being evaluated in combination with pembrolizumab Phase 2 study of oncolytic polio/rhinovirus recombinant (PVSRIPO) alone or in combination with lomustine in recurrent WHO Grade IV malignant glioma patients. Also, MedImmune, LLC/AstraZeneca plc's durvalumab was evaluated in a Phase 2 trial in patients with rGBM.

Even if we obtain regulatory approval of potential products, we may not be the first to market and that may affect the price or demand for our potential products. Existing or future competing products may provide greater therapeutic convenience or clinical or other benefits for a specific indication than our products or may offer comparable performance at a lower cost. Additionally, the availability and price of our competitors' products could limit the demand and the price we are able to charge for our potential products. We may not be able to implement our business plan if the acceptance of our potential products is inhibited by price competition or the reluctance of physicians to switch from existing methods of treatment to our potential products, or if physicians switch to other new drug or biologic products or choose to reserve our potential products. Additionally, a competitor could obtain orphan product exclusivity from the FDA with respect to such competitor's product. If such competitor product is determined to be the same product as one of our potential products, that may prevent us from obtaining approval from the FDA for such potential products for the same indication for seven years, except in limited circumstances. If our products fail to capture and maintain market share, we may not achieve sufficient product revenues and our business will suffer.

We compete against fully integrated pharmaceutical companies and smaller companies that are collaborating with larger pharmaceutical companies, academic institutions, government agencies and other public and private research organizations. Many of these competitors have products already approved or in development. In addition, many of these competitors, either alone or together with their collaborative partners, operate larger research and development programs or have substantially greater financial resources than we do, as well as significantly greater experience in:

- developing drugs and biopharmaceuticals;
- undertaking preclinical testing and human clinical trials;
- obtaining FDA and other regulatory approvals of drugs and biopharmaceuticals;
- formulating and manufacturing drugs and biopharmaceuticals; and
- launching, marketing, and selling drugs and biopharmaceuticals.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of generic products.

Any termination of our licenses with PGEN, MD Anderson or the National Cancer Institute or our research and development agreements with MD Anderson could result in the loss of significant rights and could harm our ability to develop and commercialize our product candidates.

We are dependent on patents, know-how, and proprietary technology that are licensed from others, particularly MD Anderson, Precigen and the National Cancer Institute, or the NCI, as well as the contributions by MD Anderson under our research and development agreements. Any termination of these licenses or research and development agreements could result in the loss of significant rights and could harm our ability to commercialize our product candidates. Disputes may also arise between us and these licensors regarding intellectual property subject to a license agreement, including those relating to:

- the scope of rights granted under the applicable license agreement and other interpretation-related issues;
- whether and the extent to which our technology and processes, and the technology and processes of PGEN, MD Anderson, the NCI and our other licensors, infringe intellectual property of the licensor that is not subject to the applicable license agreement;
- our right to sublicense patent and other rights to third parties pursuant to our relationships with our licensors and partners;
- whether we are complying with our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our potential products under the MD Anderson License, the License Agreement with PGEN and our patent license agreement with the NCI; and
- whether or not our partners are complying with all of their obligations to support our programs under licenses and research and development agreements; and
- the allocation of ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and by us.

In addition, under our License Agreement, PGEN is obligated to provide certain transition services and transfer certain know-how to us. For example, PGEN was previously responsible for manufacturing the products used in our clinical programs and is now responsible for transferring the related know-how so that we can begin manufacturing products used in our clinical trials. There is no guarantee that PGEN will perform these activities to our satisfaction, if at all. If PGEN fails to perform these activities our ability to pursue our clinical programs may be adversely affected.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements, particularly with MD Anderson, PGEN and the NCI, on acceptable terms, we may be unable to successfully develop and commercialize the affected potential products. We are generally also subject to all of the same risks with respect to protection of intellectual property that we license as we are for intellectual property that we own. If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize potential products under our applicable licenses could suffer. There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including interference, derivation, and reexamination proceedings before the United States Patent and Trademark Office, or USPTO, or oppositions and other comparable proceedings in foreign jurisdictions. Recently, due to changes in U.S. law referred to as patent reform, new procedures including inter partes review and post-grant review have been implemented, which adds uncertainty to the possibility of challenge to our or our licensors' patents in the future.

Furthermore, certain of the above risks and uncertainties may be amplified as a result of the impact of COVID-19. The extent to which COVID-19 may impact our agreements with PGEN, MD Anderson or the National Cancer Institute, or any other third-party partner, will depend on future developments, which are highly uncertain and cannot be predicted, including new information which may emerge concerning the severity of COVID-19 and the actions to contain COVID-19 or treat its impact, among others.

* ***We are partly reliant on the National Cancer Institute for research and development and early clinical testing of certain of our product candidates.***

A portion of our research and development is being conducted by the NCI under the CRADA entered into in January 2017 and amended in February 2019. Under the CRADA, the NCI, with Dr. Steven A. Rosenberg as the principal investigator, is responsible for conducting a clinical trial using the *Sleeping Beauty* system to express TCRs for the treatment of solid tumors. We have limited control over the nature or timing of the NCI's clinical trial and limited visibility into their day-to-day activities, including with respect to how they are providing and administering T cell therapy. For example, the research we are funding constitutes only a small portion of the NCI's overall research. Additionally, other research being conducted by Dr. Rosenberg may at times receive higher priority than research on our program. Further, in response to the COVID-19 pandemic, the NCI has taken precautionary measures that have delayed the enrollment of the TCR-T clinical trial using the *Sleeping Beauty* system to express TCRs for the treatment of solid tumors.

The CRADA terminates on January 9, 2022 unless it is extended in writing by the parties, and either party may terminate the CRADA by providing at least 60 days' prior written notice to the other party. If the NCI unilaterally terminates the CRADA or the CRADA lapses without any extension, part or all of the research and development of the *Sleeping Beauty* system conducted at the NCI would be suspended, and the research and development of our TCR program would be impacted.

Clinical trials are very expensive, time-consuming, difficult to design, initiate and implement.

Human clinical trials are very expensive and difficult to design, initiate and implement, in part because they are subject to rigorous regulatory requirements. The clinical trial start-up and process itself is also time-consuming and results are inherently uncertain. We estimate that clinical trials of our product candidates will take at least several years to complete. Furthermore, failure can occur at any stage of the trials, and we could encounter problems that cause us to delay the start of, abandon or repeat clinical trials. The commencement and completion of clinical trials may be delayed by several factors, including:

- Additional nonclinical data requests by regulatory agencies;
- Unforeseen safety issues;
- Determination of dosing issues;
- Lack of effectiveness during clinical trials;
- Slower than expected rates of patient recruitment and enrollment;
- Inability to monitor patients adequately during or after treatment;
- Inability or unwillingness of medical investigators or patients to follow our clinical protocols, including as a result of the recent COVID-19 pandemic; and
- Regulatory determinations to temporarily or permanently cease enrollment for other reasons not related to patient safety.

Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful. In addition, we or the FDA may suspend our clinical trials at any time if it appears that we are exposing participants to unacceptable health risks or if the FDA finds deficiencies in our IND submission or in the conduct of these trials. In June 2018, we announced that the FDA placed our Phase 1 trial on clinical hold to evaluate CD19-specific CAR-T therapies manufactured using our rapid personalized manufacturing technology with patient-derived (autologous) T cells in order to treat patients with relapsed or refractory CD19+ leukemias and lymphomas. The FDA requested additional information in support of the IND submission for the trial. Our business may be materially harmed if we or our partners are unable to adequately address the FDA's requests for this trial.

See also "Risks Related to the Clinical Testing, Regulatory Approval and Manufacturing of our Product Candidates—*Our product candidates are in various stages of clinical trials, which are very expensive and time- consuming. We cannot be certain when we will be able to submit a BLA, to the FDA and any failure or delay in completing clinical trials for our product candidates could harm our business."*

We may not be able to commercialize any products, generate significant revenues, or attain profitability.

To date, none of our product candidates have been approved for commercial sale in any country. The process to develop, obtain regulatory approval for, and commercialize potential product candidates is long, complex, and costly. Unless and until we receive approval from the FDA and/or other foreign regulatory authorities for our product candidates, we cannot sell our products and will not have product revenues. Even if we obtain regulatory approval for one or more of our product candidates, if we are unable to successfully commercialize our products, we may not be able to generate sufficient revenues to achieve or maintain profitability, or to continue our business without raising significant additional capital, which may not be available. Our failure to achieve or maintain profitability could negatively impact the trading price of our common stock.

Ethical, legal and social concerns about synthetic biologically engineered products could limit or prevent the use of our product candidates.

Our product candidates use an immuno-oncology platform. Public perception about the safety and environmental hazards of, and ethical concerns over, genetically engineered products could influence public acceptance of our product candidates. If we and our collaborators are not able to overcome the ethical, legal and social concerns relating to biological engineering, our product candidates may not be accepted. These concerns could result in increased expenses, regulatory scrutiny, delays or other impediments to the public acceptance and commercialization of our product candidates. Our ability to develop and commercialize products could be limited by public attitudes and governmental regulation.

The subject of genetically modified organisms has received negative publicity, which has aroused public debate. This adverse publicity could lead to greater regulation and trade restrictions on the development and commercialization of genetically altered products. Further, there is a risk that our product candidates could cause adverse health effects or other AEs, which could also lead to negative publicity.

The biological platform that we use may have significantly enhanced characteristics compared to those found in naturally occurring organisms, enzymes or microbes. While we believe we produce biological technologies only for use in a controlled laboratory and industrial environment, the release of such biological technologies into uncontrolled environments could have unintended consequences. Any adverse effect resulting from such a release could have a material adverse effect on our business and financial condition, and we may have exposure to liability for any resulting harm.

* ***We may not be able to retain the rights licensed to us and PGEN by MD Anderson to technologies relating to CAR, T-cell therapies and other related technologies.***

Under the MD Anderson License, we, together with PGEN, received an exclusive, worldwide license to certain technologies owned and licensed by MD Anderson including technologies relating to novel CAR+ T cell and TCR cell therapies arising from the laboratory of Laurence Cooper, M.D., Ph.D., who was then at MD Anderson, as well as either co-exclusive or non-exclusive licenses under certain related technologies. When combined with PGEN's technology suite and Ziopharm's clinically tested RTS® interleukin 12 modules, the resulting proprietary methods and technologies may help realize the promise of genetically modified CAR+ T cells and TCR therapies by controlling cell expansion and activation in the body, minimizing off-target and unwanted on-target effects and toxicity while maximizing therapeutic efficacy. The term of the MD Anderson License expires on the last to occur of (a) the expiration of all patents licensed thereunder, or (b) the twentieth anniversary of the date of the MD Anderson License; provided, however, that following the expiration of the term, we and PGEN shall then have a fully-paid up, royalty free, perpetual, irrevocable and sublicensable license to use the licensed intellectual property thereunder.

After 10 years from the date of the MD Anderson License and subject to a 90-day cure period, MD Anderson will have the right to convert the MD Anderson License into a non-exclusive license if we and PGEN are not using commercially reasonable efforts to commercialize the licensed intellectual property on a case-by-case basis. After five years from the date of the MD Anderson License and subject to a 180-day cure period, MD Anderson will have the right to terminate the MD Anderson License with respect to specific technology(ies) funded by the government or subject to a third-party contract if we and PGEN are not meeting the diligence requirements in such funding agreement or contract, as applicable. MD Anderson may also terminate the agreement with written notice upon material breach by us or PGEN, if such breach has not been cured within 60 days of receiving such notice. In addition, the MD Anderson License will terminate upon the occurrence of certain insolvency events for both us or PGEN and may be terminated by the mutual written agreement of us, PGEN and MD Anderson.

There can be no assurance that we will be able to successfully perform under the MD Anderson License and if the MD Anderson License is terminated it may prevent us from achieving our business objectives.

We have a limited operating history upon which to base an investment decision.

We have not demonstrated an ability to perform the functions necessary for the successful commercialization of any product candidates. The successful commercialization of any product candidates will require us to perform a variety of functions, including:

- Continuing to undertake preclinical development and clinical trials;
- Participating in regulatory approval processes;
- Formulating and manufacturing products; and
- Conducting sales and marketing activities.

Our operations have been limited to organizing and staffing our company, acquiring, developing and securing our proprietary product candidates, and undertaking preclinical and clinical trials of our product candidates. These operations provide a limited basis for you to assess our ability to commercialize our product candidates and the advisability of investing in our securities.

We may not be successful in establishing development and commercialization collaborations, which failure could adversely affect, and potentially prohibit, our ability to develop our product candidates.

Developing biopharmaceutical products and complementary technologies, conducting clinical trials, obtaining marketing approval, establishing manufacturing capabilities and marketing approved products is expensive and, therefore, we anticipate exploring collaborations with third parties that have alternative technologies, more resources and more experience than we do. In situations where we enter into a development and commercial collaboration arrangement for a product candidate or complementary technology, we may also seek to establish additional collaborations for development and commercialization in territories outside of those addressed by the first collaboration arrangement for such product candidate or technology. There are a limited number of potential partners, and we expect to face competition in seeking appropriate partners. If we are unable to enter into any development and commercial collaborations and/or sales and marketing arrangements on reasonable and acceptable terms, if at all, we may be unable to successfully develop and seek regulatory approval for our product candidates and/or effectively market and sell future approved products, if any, in some or all of the territories outside of the United States where it may otherwise be valuable to do so.

* ***Because we currently have limited internal research capabilities, we are dependent upon pharmaceutical and biotechnology companies and academic and other researchers to sell or license us their product candidates and technology.***

Proposing, negotiating, and implementing an economically viable product acquisition or license is a lengthy and complex process. We compete for partnering arrangements and license agreements with pharmaceutical, biopharmaceutical, and biotechnology companies, many of which have significantly more experience than we do and have significantly more financial resources. Our competitors may have stronger relationships with certain third parties including academic research institutions, with whom we are interested in collaborating and may have, therefore, a competitive advantage in entering into partnering arrangements with those third parties. We may not be able to acquire rights to additional product candidates or complementary technology on terms that we find acceptable, or at all.

We expect that any product candidate to which we acquire rights will require significant additional development and other efforts prior to commercial sale, including extensive clinical testing and approval by the FDA and applicable foreign regulatory authorities. All product candidates are subject to the risks of failure inherent in biopharmaceutical product development, including the possibility that the product candidate will not be shown to be sufficiently safe or effective for approval by regulatory authorities. Even if our product candidates are approved, they may not be economically manufactured or produced, or be successfully commercialized.

We actively evaluate complementary technologies to acquire or license. Such complementary technologies could significantly increase our capital requirements and place further strain on the time of our existing personnel, which may delay or otherwise adversely affect the development of our existing product candidates. We must manage our development efforts and clinical trials effectively, and hire, train and integrate additional management, administrative, and research and development personnel. We may not be able to accomplish these tasks, and our failure to accomplish any of them could prevent us from successfully growing.

Our business will subject us to the risk of liability claims associated with the use of hazardous materials and chemicals.

Our contract research and development activities may involve the controlled use of hazardous materials and chemicals. Although we believe that our safety procedures for using, storing, handling and disposing of these materials comply with federal, state and local laws and regulations, we cannot completely eliminate the risk of accidental injury or contamination from these materials. In the event of such an accident, we could be held liable for any resulting damages and any liability could have a materially adverse effect on our business, financial condition, and results of operations. In addition, the federal, state and local laws and regulations governing the use, manufacture, storage, handling and disposal of hazardous or radioactive materials and waste products may require our contractors to incur substantial compliance costs that could materially adversely affect our business, financial condition, and results of operations.

We may not be able to successfully manage our growth.

In the future, if we are able to advance our product candidates to the point of, and thereafter through, clinical trials, we will need to expand our development, regulatory, manufacturing, marketing and sales capabilities or contract with third parties to provide for these capabilities. Further, to advance our TCR program, we anticipate significantly expanding our internal research capabilities, including hiring additional employees focusing on pre-clinical research. This growth will place a significant strain on our management and on our administrative, operational, and financial resources. Therefore, 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 manage this growth, we must expand our facilities, augment our operational, financial and management systems, and hire and train additional qualified personnel. If we are unable to manage our growth effectively, our business may be harmed.

We rely on key executive officers and scientific and medical advisors, and their knowledge of our business and technical expertise would be difficult to replace.

We may not be able to attract or retain qualified management and commercial, scientific and clinical personnel due to the intense competition for qualified personnel among biotechnology, pharmaceutical and other businesses. If we are not able to attract and retain necessary personnel to accomplish our business objectives, we may experience constraints that will significantly impede the achievement of our development objectives, our ability to raise additional capital and our ability to implement our business strategy.

We are highly dependent on Dr. Laurence J.N. Cooper, our Chief Executive Officer and our principal scientific, regulatory, and medical advisors. Dr. Cooper may terminate his employment with us at any time, subject, however, to certain non-compete and non-solicitation covenants. The loss of the technical knowledge and management and industry expertise of Dr. Cooper or any of our other key personnel, could result in delays in product development, loss of key personnel or partnerships, and diversion of management resources, which could adversely affect our operating results. We do not carry “key person” life insurance policies on any of our officers or key employees.

If we are unable to hire additional qualified personnel, our ability to grow our business may be harmed.

We will need to hire additional qualified personnel with expertise in preclinical and clinical research and testing, government regulation, formulation and manufacturing, and eventually, sales and marketing. In particular, we expect to significantly expand our internal cell therapy capabilities in our Houston, Texas facilities by hiring additional research and development personnel. We compete for qualified individuals with numerous biopharmaceutical companies, universities, and other research institutions. Competition for such individuals is intense and we cannot be certain that our search for such personnel will be successful. Attracting and retaining qualified personnel will be critical to our success. If we are unable to hire additional qualified personnel, our ability to grow our business may be harmed.

We may incur substantial liabilities and may be required to limit commercialization of our products in response to product liability lawsuits.

The testing and marketing of medical products entail an inherent risk of product liability. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our products, if approved. Even a successful defense would require significant financial and management resources. Regardless of the merit or eventual outcome, liability claims may result in:

- Decreased demand for our product candidates;
- Injury to our reputation;
- Withdrawal of clinical trial participants;
- Withdrawal of prior governmental approvals;
- Costs of related litigation;
- Substantial monetary awards to patients;
- Product recalls;
- Loss of revenue; and
- The inability to commercialize our product candidates.

We currently carry clinical trial insurance and product liability insurance. However, an inability to renew our policies or to obtain sufficient insurance at an acceptable cost could prevent or inhibit the commercialization of pharmaceutical products that we develop, alone or with collaborators.

Our business and operations would suffer in the event of system failures.

Despite the implementation of security measures, our internal computer systems and those of our current and future contractors and consultants are vulnerable to damage from computer viruses, unauthorized access, natural disasters, terrorism, war and telecommunication and electrical failures. While we are not aware of any such material system failure, accident or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our 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. Likewise, we rely on third parties to manufacture our product candidates and conduct clinical trials, and similar events relating to their computer systems could also have a material adverse effect on our business. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our product candidates could be delayed.

RISKS RELATED TO THE CLINICAL TESTING, REGULATORY APPROVAL AND MANUFACTURING OF OUR PRODUCT CANDIDATES

If we are unable to obtain the necessary U.S. or worldwide regulatory approvals to commercialize any product candidate, our business will suffer.

We may not be able to obtain the approvals necessary to commercialize our product candidates, or any product candidate that we may acquire or develop in the future for commercial sale. We will need FDA approval to commercialize our product candidates in the United States and approvals from regulatory authorities in foreign jurisdictions equivalent to the FDA to commercialize our product candidates in those jurisdictions. In order to obtain FDA approval of any product candidate, we must submit to the FDA a Biologics License Application, or BLA, demonstrating that the product candidate is safe for humans and effective for its intended use. This demonstration requires significant research and animal tests, which are referred to as preclinical studies, as well as human tests, which are referred to as clinical trials. Satisfaction of the FDA's regulatory requirements typically takes many years, depending upon the type, complexity, and novelty of the product candidate, and will require substantial resources for research, development, and testing. We cannot predict whether our research, development, and clinical approaches will result in products that the FDA will consider safe for humans and effective for their intended uses. The FDA has substantial discretion in the approval process and may require us to conduct additional preclinical and clinical testing or to perform post-marketing studies. The approval process may also be delayed by changes in government regulation, future legislation, or administrative action or changes in FDA policy that occur prior to or during our regulatory review. Delays in obtaining regulatory approvals may:

- Delay commercialization of, and our ability to derive product revenues from, our product candidates;
- Impose costly procedures on us; and
- Diminish any competitive advantages that we may otherwise enjoy.

Even if we comply with all FDA requests, the FDA may ultimately reject one or more of our BLAs. We cannot be sure that we will ever obtain regulatory approval for any of our product candidates. Failure to obtain FDA approval for our product candidates will severely undermine our business by leaving us without a saleable product, and therefore without any potential revenue source, until another product candidate can be developed. There is no guarantee that we will ever be able to develop or acquire another product candidate or that we will obtain FDA approval if we are able to do so.

In foreign jurisdictions, we similarly must receive approval from applicable regulatory authorities before we can commercialize any of our product candidates. Foreign regulatory approval processes generally include all of the risks associated with the FDA approval procedures described above.

Our product candidates are in various stages of clinical trials, which are very expensive and time-consuming. We cannot be certain when we will be able to submit a BLA to the FDA and any failure or delay in completing clinical trials for our product candidates could harm our business.

Our product candidates are in various stages of development and require extensive clinical testing. Notwithstanding our current clinical trial plans for each of our existing product candidates, we may not be able to commence additional trials or see results from these trials within our anticipated timelines. As they enter later stages of development, our product candidates generally will become subject to more stringent regulatory requirements, including the FDA's requirements for chemistry, manufacturing and controls for product candidates entering Phase 3 clinical trials. There is no guarantee the FDA will allow us to commence Phase 3 clinical trials for product candidates studied in early clinical trials. For example, we are currently evaluating Ad-RTS-hIL-12 plus veledimex in combination with PD-1 antibody Libtayo® (cemiplimab-rwlc) in a Phase 2 clinical trial for the treatment of recurrent or progressive glioblastoma multiforme in adults. If we elect to advance Ad-RTS-hIL-12 plus veledimex into a Phase 3 clinical trial, we will need to meet all of the FDA's requirements for this clinical trial. We previously announced that we were pausing a pivotal randomized control trial for Ad-RTS-hIL-12 plus veledimex for the treatment of rGBM and that were resolving previously disclosed technical requirements related to chemistry, manufacturing and controls prior to commencing a Phase 3 clinical trial. These efforts remain ongoing and there is no guarantee we will be able to meet the FDA's requirements for a Phase 3 clinical trial or that the development of Ad-RTS-hIL-12 plus veledimex will not be delayed in order to address these requirements.

If the FDA does not allow our product candidates to enter later stage clinical trials, or requires changes to the formulation or manufacture of our product candidates before commencing Phase 3 clinical trials, our ability to further develop, or seek approval for, such product candidates may be materially impacted. As such, we cannot predict with any certainty if or when we might submit a BLA for regulatory approval of our product candidates or whether such a BLA will be accepted. Because we do not anticipate generating revenues unless and until we submit one or more BLAs and thereafter obtain requisite FDA approvals, the timing of our BLA submissions and FDA determinations regarding approval thereof, will directly affect if and when we are able to generate revenues.

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

As with many pharmaceutical and biological products, treatment with our product candidates may produce undesirable side effects or adverse reactions or events, including potential adverse side effects related to cytokine release. If our product candidates or similar products or product candidates under development by third parties demonstrate unacceptable AEs, we may be required to halt or delay further clinical development of our product candidates. The FDA or other foreign regulatory authorities could order us to cease further development of or deny approval of our product candidates for any or all targeted indications. For instance, Ad-RTS-hIL-12 plus veledimex may result in local reactions during the time of injection, including severe swelling and bleeding. If a serious adverse event was to occur in any of our clinical trials, including in our trial of Ad-RTS-hIL-12 plus veledimex for the treatment of DIPG, the FDA may place a hold on the clinical trial for this indication and, potentially, our clinical trials of Ad-RTS-hIL-12 plus veledimex in other indications.

The product-related side effects could affect patient recruitment or the ability of enrolled patients to complete the trial or result in potential product liability claims. In addition, these side effects may not be appropriately or timely recognized or managed by the treating medical staff, particularly outside of the institutions that collaborate with us, as toxicities resulting from our novel technologies may not be normally encountered in the general patient population and by medical personnel. We expect to have to train medical personnel using our product candidates to understand their side effect profiles, both for our planned clinical trials and upon any commercialization of any product candidates. Inadequate training in recognizing or managing the potential side effects of our product candidates could result in adverse effects to patients, including death.

Additionally, if one or more of our product candidates receives marketing approval, and we or others later identify undesirable side effects caused by such products, including during any long-term follow-up observation period recommended or required for patients who receive treatment using our products, a number of potentially significant negative consequences could result, including:

- regulatory authorities may withdraw approvals of such product;
- regulatory authorities may require additional warnings on the label;
- we may be required to create a risk evaluation and mitigation strategy plan, which could include a medication guide outlining the risks of such side effects for distribution to patients, a communication plan for healthcare providers, and/or other elements to assure safe use;
- we could be sued and held liable for harm caused to patients; and
- our reputation may suffer.

Any of the foregoing could prevent us from achieving or maintaining market acceptance of the particular product candidate, if approved. Furthermore, any of these occurrences may harm our business, financial condition and prospects significantly.

Our cell-based and gene therapy immuno-oncology products rely on the availability of reagents, specialized equipment, and other specialty materials and infrastructure, which may not be available to us on acceptable terms or at all. For some of these reagents, equipment, and materials, we rely or may rely on sole source vendors or a limited number of vendors, which could impair our ability to manufacture and supply our products.

Manufacturing our product candidates will require many reagents, which are substances used in our manufacturing processes to bring about chemical or biological reactions, and other specialty materials and equipment, some of which are manufactured or supplied by small companies with limited resources and experience to support commercial biologics production. We currently depend on a limited number of vendors for certain materials and equipment used in the manufacture of our product candidates. Some of these suppliers may not have the capacity to support commercial products manufactured under current good manufacturing practices by biopharmaceutical firms or may otherwise be ill-equipped to support our needs. We also do not have supply contracts with many of these suppliers and may not be able to obtain supply contracts with them on acceptable terms or at all. Accordingly, we may experience delays in receiving key materials and equipment to support clinical or commercial manufacturing.

For some of these reagents, equipment, infrastructure, and materials, we rely and may in the future rely on sole source vendors or a limited number of vendors. An inability to continue to source product from any of these suppliers, which could be due to regulatory actions or requirements affecting the supplier, adverse financial or other strategic developments experienced by a supplier, labor disputes or shortages, unexpected demands, or quality issues, could adversely affect our ability to satisfy demand for our product candidates, which could adversely and materially affect our product sales and operating results or our ability to conduct clinical trials, either of which could significantly harm our business.

In addition, some of the reagents and products used by us, including in our clinical trials, may be stored at a single vendor. The loss of materials located at a single vendor, or the failure of such a vendor to manufacture clinical product in accordance with our specifications, would impact our ability to conduct ongoing or planned clinical trials and continue the development of our products. Further, manufacturing replacement material may be expensive and require a significant amount of time, which may further impact our clinical programs.

As we continue to develop and scale our manufacturing process, we expect that we will need to obtain rights to and supplies of certain materials and equipment to be used as part of that process. We may not be able to obtain rights to such materials on commercially reasonable terms, or at all, and if we are unable to alter our process in a commercially viable manner to avoid the use of such materials or find a suitable substitute, it would have a material adverse effect on our business. Even if we are able to alter our process so as to use other materials or equipment, such a change may lead to a delay in our clinical development and/or commercialization plans. If such a change occurs for product candidate that is already in clinical testing, the change may require us to perform both ex vivo comparability studies and to collect additional data from patients prior to undertaking more advanced clinical trials.

The results of our clinical trials may not support our product candidate claims.

Even if our clinical trials are completed as planned, we cannot be certain that their results will support approval of our product candidates. The FDA normally expects two randomized, well-controlled Phase 3 pivotal trials in support of approval of a BLA. Success in preclinical testing and early clinical trials does not ensure that later clinical trials will be successful, and we cannot be certain that the results of later clinical trials will replicate the results of prior clinical trials and preclinical testing. The clinical trial process may fail to demonstrate that our product candidates are safe for humans and effective for the indicated uses. This failure would cause us to abandon a product candidate and may delay development of other product candidates. Any delay in, or termination of, our clinical trials will delay the submission of our BLAs with the FDA and, ultimately, our ability to commercialize our product candidates and generate product revenues. In addition, our clinical trials involve small patient populations. Because of the small sample size, the results of these clinical trials may not be indicative of future results.

Our immuno-oncology product candidates are based on a novel technology, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval. Currently, few gene therapy and cell therapy products have been approved in the United States and Europe.

We are currently focused on developing products in immuno-oncology that employ novel gene expression, control and cell technologies to deliver safe, effective and scalable cell- and viral-based therapies for the treatment of cancer. Due to the novelty of this medical technology, there can be no assurance that any development problems we experience in the future related to our immuno-oncology platforms will not cause significant delays or unanticipated costs, or that such development problems can be solved. We may also experience unanticipated problems or delays in expanding our manufacturing capacity or transferring our manufacturing process to commercial partners, which may prevent us from completing our clinical trials or commercializing our immuno-oncology product candidates on a timely or profitable basis, if at all.

In addition, the clinical study requirements of the FDA, the EMA and other regulatory agencies and the criteria these regulators use to determine the safety and efficacy of a product candidate vary substantially according to the type, complexity, novelty and intended use and market of the potential products. The regulatory approval process for novel product candidates such as ours can be more expensive and take longer than for other, better known or extensively studied pharmaceutical or other product candidates. These factors make it difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in either the United States or Europe. Approvals by the EMA may not be indicative of what the FDA may require for approval.

Regulatory requirements governing gene and cell therapy products have changed frequently and may continue to change in the future. For example, the FDA has established the Office of Tissue and Advanced Therapies within its Center for Biologics Evaluation and Research, or CBER, to consolidate the review of gene therapy and related products, and the Cellular, Tissue and Gene Therapies Advisory Committee to advise CBER on its review. Also, before a clinical trial can begin at an institution, that institution's institutional review board, or IRB, and its Institutional Biosafety Committee will have to review the proposed clinical trial to assess the safety of the trial. In addition, adverse developments in clinical trials of gene therapy products conducted by others may cause the FDA or other regulatory bodies to change the requirements for approval of any of our product candidates.

These regulatory review committees and advisory groups and the new guidelines they promulgate may lengthen the regulatory review process, require us to perform additional studies, increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of these treatment candidates or lead to significant post-approval limitations or restrictions. As we advance our immuno-oncology 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 for oncology product candidates. 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 to maintain our business.

* ***Because we are dependent upon clinical research institutions and other contractors for clinical testing and for research and development activities, the results of our clinical trials and such research activities are, to a certain extent, beyond our control.***

We materially rely upon independent investigators and collaborators, such as universities and medical institutions, to conduct our preclinical and clinical trials under agreements with us. These collaborators are not our employees and we cannot control the amount or timing of resources that they devote to our programs. These investigators may not assign as great a priority to our programs or pursue them as diligently as we would if we were undertaking such programs ourselves. If outside collaborators fail to devote sufficient time and resources to our product development programs, or if their performance is substandard, the approval of our FDA applications, if any, and our introduction of new products, if any, will be delayed. These institutions may also have, or implement in the future, policies and procedures that limit their ability to advance our programs. For instance, our partners may take measures in response to the COVID-19 pandemic, that may impact enrollment in our clinical trials. These collaborators may also have relationships with other commercial entities, some of whom may compete with us. If our collaborators assist our competitors to our detriment, our competitive position would be harmed.

* ***Our reliance on third parties to formulate and manufacture our product candidates exposes us to a number of risks that may delay the development, regulatory approval and commercialization of our products or result in higher product costs.***

We have limited experience in biopharmaceutical manufacturing. We currently lack the internal resources and expertise to formulate or manufacture our own product candidates and, therefore, contract the manufacture of our product candidates with third parties. We intend to contract with one or more manufacturers to manufacture, supply, store, and distribute supplies for our clinical trials. If a product candidate we develop or acquire in the future receives FDA approval, we may rely on one or more third-party contractors to manufacture our products. Our

anticipated future reliance on a limited number of third-party manufacturers exposes us to the following risks:

- We may be unable to identify manufacturers on acceptable terms or at all because the number of potential manufacturers is limited and the FDA must approve any replacement contractor. This approval would require new testing and compliance inspections. In addition, a new manufacturer would have to be educated in, or develop substantially equivalent processes for, production of our products after receipt of FDA approval, if any.
- Our third-party manufacturers might be unable to formulate and manufacture our products in the volume and of the quality required to meet our clinical needs and commercial needs, if any.
- Our future contract manufacturers may not perform as agreed or may not remain in the contract manufacturing business for the time required to supply our clinical trials or to successfully produce, store, and distribute our products.
- Biopharmaceutical manufacturers are subject to ongoing periodic unannounced inspection by the FDA, the Drug Enforcement Administration and corresponding state and foreign agencies to ensure strict compliance with current good manufacturing practices, or cGMP, and other government regulations and corresponding foreign standards. We do not have control over third-party manufacturers' compliance with these regulations and standards.
- If any third-party manufacturer makes improvements in the manufacturing process for our products, we may not own, or may have to share, the intellectual property rights to the innovation.
- Further third party manufacturers may encounter difficulties in achieving volume production, quality control, and quality assurance and also may experience shortages in qualified personnel and obtaining materials for our product candidates, including delays or shortages due to limited supply or capacity of production facilities as a result of the recent COVID-19 pandemic.
- Our third-party manufacturers may not be able to comply with cGMP regulations or similar regulatory requirements outside the United States. Our failure, or the failure of our third-party manufacturers, to comply with applicable regulations could result in sanctions being imposed on us, including clinical holds, fines, injunctions, civil penalties, delays, suspension or withdrawal of approvals, license revocation, seizures or recalls of products, operating restrictions and criminal prosecutions, any of which could significantly and adversely affect supplies of our products.

Each of these risks could delay our clinical trials, the approval, if any, of our product candidates by the FDA or the commercialization of our product candidates or result in higher costs or deprive us of potential product revenues.

Any product candidate for which we obtain marketing approval could be subject to post-marketing restrictions or withdrawal from the market and we may be subject to penalties if we fail to comply with regulatory requirements or if we experience unanticipated problems with our products, when and if any of them are approved.

Any product candidate for which we obtain marketing approval, along with the manufacturing processes, post- approval clinical data, labeling, advertising and promotional activities for such product, will be subject to continual requirements of and review by the FDA and other regulatory authorities. These requirements include, among other things, submissions of safety and other post-marketing information and reports, registration and listing requirements, cGMP requirements relating to manufacturing, quality control, quality assurance and corresponding maintenance of records and documents, requirements regarding the distribution of samples to physicians and recordkeeping. Even if marketing approval of a product candidate is granted, the approval may be subject to limitations on the indicated uses for which the product may be marketed or to the conditions of approval, including the requirement to implement a risk evaluation and mitigation strategy, or REMS, which could include requirements for a restricted distribution system. If any of our product candidates receives marketing approval, the accompanying label may limit the approved uses, which could limit sales of the product.

The FDA may also impose requirements for costly post-marketing studies or clinical trials and surveillance to monitor the safety or efficacy of our approved products. The FDA closely regulates the post-approval marketing and promotion of products to ensure that they are marketed only for the approved indications and in accordance with the provisions of the approved labeling. However, companies may share truthful and not misleading information that is otherwise consistent with the labeling. The FDA imposes stringent restrictions on manufacturers' communications regarding off-label use and if we market our products outside of their approved indications, we may be subject to enforcement action for off-label marketing. Violations of the Federal Food, Drug and Cosmetic Act relating to the promotion of prescription drugs may lead to investigations alleging violations of federal and state health care fraud and abuse laws, as well as state consumer protection laws.

In addition, later discovery of previously unknown AEs or other problems with our products, manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may yield various results, including:

- Litigation involving patients taking our product;
- Restrictions on such products, manufacturers or manufacturing processes;
- Restrictions on the labeling or marketing of a product;
- Restrictions on product distribution or use;
- Requirements to conduct post-marketing studies or clinical trials;
- Warning letters;
- Withdrawal of the products from the market;
- Refusal to approve pending applications or supplements to approved applications that we submit;
- Recall of products;
- Fines, restitution or disgorgement of profits or revenues;
- Suspension or withdrawal of marketing approvals;
- Damage to relationships with existing and potential collaborators;
- Unfavorable press coverage and damage to our reputation;
- Refusal to permit the import or export of our products;
- Product seizure; or
- Injunctions or the imposition of civil or criminal penalties.

Noncompliance with requirements regarding safety monitoring or pharmacovigilance can also result in significant financial penalties. Similarly, failure to comply with U.S. and foreign regulatory requirements regarding the development of products for pediatric populations and the protection of personal health information can also lead to significant penalties and sanctions.

RISKS RELATED TO OUR ABILITY TO COMMERCIALIZE OUR PRODUCT CANDIDATES

If we are unable either to create sales, marketing and distribution capabilities or enter into agreements with third parties to perform these functions, we will be unable to commercialize our product candidates successfully.

We currently have no marketing, sales, or distribution capabilities. If and when we become reasonably certain that we will be able to commercialize our current or future product candidates, we anticipate allocating resources to the marketing, sales and distribution of our proposed products in North America and in certain other countries; however, we cannot assure that we will be able to market, sell, and distribute our products successfully. Our future success also may depend, in part, on our ability to enter into and maintain collaborative relationships for such capabilities and to encourage the collaborator's strategic interest in the products under development, and such collaborator's ability to successfully market and sell any such products. Although we intend to pursue certain collaborative arrangements regarding the sale and marketing of certain of our product candidates, there are no assurances that we will be able to establish or maintain collaborative arrangements or, if we are able to do so, whether we would be able to conduct our own sales efforts. There can also be no assurance that we will be able to establish or maintain relationships with third-party collaborators or develop in-house sales and distribution capabilities. To the extent that we depend on third parties for marketing and distribution, any revenues we receive will depend upon the efforts of such third parties, and there can be no assurance that such efforts will be successful. In addition, there can also be no assurance that we will be able to market and sell our product candidates in the United States or overseas.

If we are not able to partner with a third party and are not successful in recruiting sales and marketing personnel or in building a sales and marketing infrastructure, we will have difficulty commercializing our product candidates, which would harm our business. If we rely on pharmaceutical or biotechnology companies with established distribution systems to market our products, we will need to establish and maintain partnership arrangements, and we may not be able to enter into these arrangements on acceptable terms or at all. To the extent that we enter into co-promotion or other arrangements, any revenues we receive will depend upon the efforts of third parties that may not be successful and that will be only partially in our control.

If we cannot compete successfully for market share against other biopharmaceutical companies, we may not achieve sufficient product revenues and our business will suffer.

The market for our product candidates is characterized by intense competition and rapid technological advances. If a product candidate receives FDA approval, it will compete with a number of existing and future products and therapies developed, manufactured and marketed by others. Existing or future competing products may provide greater therapeutic convenience or clinical or other benefits for a specific indication than our products or may offer comparable performance at a lower cost. If our products fail to capture and maintain market share, we may not achieve sufficient product revenues and our business will suffer.

We will compete against fully integrated pharmaceutical companies and smaller companies that are collaborating with larger pharmaceutical companies, academic institutions, government agencies and other public and private research organizations. Many of these competitors have products already approved or in development. In addition, many of these competitors, either alone or together with their collaborative partners, operate larger research and development programs or have substantially greater financial resources than we do, as well as significantly greater experience in:

- Developing drugs and biopharmaceuticals;
- Undertaking preclinical testing and human clinical trials;
- Obtaining FDA and other regulatory approvals of drugs and biopharmaceuticals;
- Formulating and manufacturing drugs and biopharmaceuticals; and
- Launching, marketing, and selling drugs and biopharmaceuticals.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. In addition, our ability to compete may be affected in many cases by insurers or other third-party payors seeking to encourage the use of generic products.

If physicians and patients do not accept and use our product candidates, our ability to generate revenue from sales of our products will be materially impaired.

Even if the FDA and/or foreign equivalents thereof approve our product candidates, physicians and patients may not accept and use them. Acceptance and use of our products will depend upon a number of factors including:

- Perceptions by members of the healthcare community, including physicians, about the safety and effectiveness of our products;
- Pharmacological benefit and cost-effectiveness of our products relative to competing products;
- Availability of coverage and adequate reimbursement for our products from government or other third- party payors;
- Effectiveness of marketing and distribution efforts by us and our licensees and distributors, if any; and
- The price at which we sell our products.

Because we expect sales of our current product candidates, if approved, to generate substantially all of our product revenues for the foreseeable future, the failure of a product to find market acceptance would harm our business and could require us to seek additional financing in order to fund the development of future product candidates.

Our ability to generate product revenues will be diminished if our products do not obtain coverage and adequate reimbursement from payors.

Our ability to commercialize our product candidates, if approved, alone or with collaborators, will depend in part on the extent to which coverage and reimbursement will be available from third-party payors, including government and health administration authorities, private health maintenance organizations and health insurers and other payors.

Patients who are prescribed medicine for the treatment of their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their prescription drugs. Sufficient coverage and adequate reimbursement from third-party payors are critical to new product acceptance. Coverage decisions may depend upon clinical and economic standards that disfavor new drug products when more established or lower cost therapeutic alternatives are already available or subsequently become available. It is difficult to predict the coverage and reimbursement decisions that will be made by third-party payors for novel gene and cell therapy products such as ours. Even if we obtain coverage for our product candidates, the resulting reimbursement payment rates might not be adequate or may require co-payments that patients find unacceptably high. Patients are unlikely to use our product candidates unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our product candidates.

In addition, the market for our product candidates for which we may receive regulatory approval will depend significantly on access to third-party payors' drug formularies or lists of medications for which third-party payors provide coverage and reimbursement, which might not include all of the FDA-approved drugs for a particular indication. The industry competition to be included in such formularies often leads to downward pricing pressures on pharmaceutical companies. Also, third-party payors may refuse to include a particular branded drug in their formularies or otherwise restrict patient access to a branded drug when a less costly generic equivalent or other alternative is available.

Third-party payors, whether foreign or domestic, or governmental or commercial, are developing increasingly sophisticated methods of controlling healthcare costs. In addition, in the United States, no uniform policy of coverage and reimbursement for drug products exists among third-party payors. Therefore, coverage and reimbursement for drug products can differ significantly from payor to payor. As a result, the coverage determination process is often a time-consuming and costly process that requires us to provide scientific and clinical support for the use of our products to each payor separately, with no assurance that approval will be obtained. If we are unable to obtain coverage of and adequate payment levels for our product candidates from third-party payors, physicians may limit how much or under what circumstances they will prescribe or administer our products and patients may decline to purchase them. This in turn could affect our ability to successfully commercialize our products and impact our profitability, results of operations, financial condition, and future success.

In addition, in many foreign countries, particularly the countries of the EU, the pricing of prescription drugs is subject to government control. In some non-U.S. jurisdictions, the proposed pricing for a drug must be approved before it may be lawfully marketed. The requirements governing drug pricing vary widely from country to country. For example, the EU provides options for its member states to restrict the range of medicinal products for which their national health insurance systems provide reimbursement and to control the prices of medicinal products for human use. A member state may approve a specific price for the medicinal product or it may instead adopt a system of direct or indirect controls on the profitability of the company placing the medicinal product on the market. We may face competition for our product candidates from lower-priced products in foreign countries that have placed price controls on pharmaceutical products. In addition, there may be importation of foreign products that compete with our own products, which could negatively impact our profitability.

The market opportunities for our product candidates may be limited to those patients who are ineligible for or have failed prior treatments and may be small.

Cancer therapies are sometimes characterized as first line, second line, or third line, and the FDA often approves new therapies initially only for third line use. When cancer is detected early enough, first line therapy is sometimes adequate to cure the cancer or prolong life without a cure. Whenever first line therapy, usually chemotherapy, hormone therapy, surgery, or a combination of these, proves unsuccessful, second line therapy may be administered. Second line therapies often consist of more chemotherapy, radiation, antibody drugs, tumor targeted small molecules, or a combination of these. Third line therapies can include bone marrow transplantation, antibody and small molecule targeted therapies, more invasive forms of surgery, and new technologies. We expect to initially seek approval of our product candidates as a third line therapy for patients who have failed other approved treatments.

Subsequently, for those products that prove to be sufficiently beneficial, if any, we would expect to seek approval as a second line therapy and potentially as a first line therapy, but there is no guarantee that our product candidates, even if approved, would be approved for second line or first line therapy. In addition, we may have to conduct additional clinical trials prior to gaining approval for second line or first line therapy.

Our projections of both the number of people who have the cancers we are targeting, as well as the subset of people with these cancers in a position to receive therapy and who have the potential to benefit from treatment with our product candidates, are based on our beliefs and estimates. These estimates have been derived from a variety of sources, including scientific literature, surveys of clinics, patient foundations, or market research and may prove to be incorrect. Further, new studies may change the estimated incidence or prevalence of these cancers. The number of patients may turn out to be lower than expected. Additionally, the potentially addressable patient population for our product candidates may be limited or may not be amenable to treatment with our product candidates.

Our market opportunities may also be limited by competitor treatments that may enter the market. See also “Risks Related to Our Ability to Commercialize Our Product Candidates—*If we cannot compete successfully for market share against other biopharmaceutical companies, we may not achieve sufficient product revenues and our business will suffer*.”

**** Healthcare legislative reform measures may have a material adverse effect on our business and results of operations.***

In both the United States and certain foreign jurisdictions, there have been a number of legislative and regulatory enactments in recent years that change the healthcare system in ways that could impact our future ability to sell our product candidates profitably.

Furthermore, there have been and continue to be a number of initiatives at the federal and state level that seek to reduce healthcare costs. Most significantly, in March 2010, President Obama signed into law the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or collectively the ACA, which included measures that have significantly changed the way healthcare is financed by both governmental and private insurers. Among the provisions of the ACA of importance to the pharmaceutical industry are the following:

- Created an annual, nondeductible fee on any entity that manufactures or imports certain branded prescription drugs and biologic agents, apportioned among these entities according to their market share in certain government healthcare programs;

- Increased the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program to 23.1% and 13% of the average manufacturer price for most branded and generic drugs, respectively;
- Created a new Medicare Part D coverage gap discount program, in which manufacturers must now agree to offer 70% 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;
- Extended manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;
- Created new methodologies by which rebates owed by manufacturers under the Medicaid Drug Rebate Program are calculated for drugs that are inhaled, infused, instilled, implanted or injected, and for drugs that are line extensions;
- Expanded eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals with income at or below 133% of the Federal Poverty Level, thereby potentially increasing both the volume of sales and manufacturers' Medicaid rebate liability;
- Expanded the entities eligible for discounts under the Public Health Service pharmaceutical pricing program;
- Created a new requirement to annually report drug samples that certain manufacturers and authorized distributors provide to physicians;
- Expanded healthcare fraud and abuse laws, including the False Claims Act and the federal Anti-Kickback Statute, new government investigative powers, and enhanced penalties for noncompliance;
- Created a licensure framework for follow-on biologic products;
- Created new requirements under the federal Physician Payments Sunshine Act for certain drug manufacturers to annually report information related to payments and other transfers of value made to physicians, as defined by such law, and teaching hospitals as well as ownership or investment interests held by physicians and their immediate family members;
- Created a Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research; and
- Established a Center for Medicare and Medicaid Innovation at the Centers for Medicare & Medicaid Services, or CMS, to test innovative payment and service delivery models to lower Medicare and Medicaid spending, potentially including prescription drug spending.

There remain executive, legal and political challenges to certain aspects of the ACA. Since January 2017, President Trump has signed several executive orders and other directives designed to delay, circumvent, or loosen certain requirements mandated by the ACA. Concurrently, Congress has considered legislation that would repeal or repeal and replace all or part of the ACA. While Congress has not passed comprehensive repeal legislation, several bills affecting the implementation of certain taxes under the ACA have been signed into law. In December 2017, Congress repealed the tax penalty, effective January 1, 2019, for an individual's failure to maintain ACA-mandated health insurance as part of the Tax Cuts and Jobs Act of 2017, or Tax Act. Further, the 2020 federal spending package permanently eliminated effective January 1, 2020, the ACA-mandated "Cadillac" tax on high-cost employer-sponsored health coverage and medical device tax and effective January 1,

2021 also eliminates the health insurance tax. The Bipartisan Budget Act of 2018, or the BBA, among other things, amended the ACA, effective January 1, 2019, to increase from 50 percent to 70 percent the point-of-sale discount that is owed by pharmaceutical manufacturers who participate in Medicare Part D and close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole". In December 2018, CMS published a new final rule permitting further collections and payments to and from certain ACA qualified health plans and health insurance issuers under the ACA risk adjustment program. On April 27, 2020, the U.S. Supreme Court reversed a Federal Circuit decision that previously upheld Congress' denial of $12 billion in "risk corridor" funding. On December 14, 2018, a Texas U.S. District Court Judge ruled that ACA is unconstitutional in its entirety because the "individual mandate" was repealed by Congress as part of the Tax Act. Additionally, on December 18, 2019, the U.S. Court of Appeals for the 5th Circuit upheld the District Court ruling that the individual mandate was unconstitutional and remanded the case back to the District Court to determine whether the remaining provisions of the ACA are invalid as well. On March 2, 2020, the United States Supreme Court granted the petitions for writs of certiorari to review this case. It is unclear how such litigation and other efforts to repeal and replace ACA will impact ACA and our business. The ultimate content, timing or effect of any healthcare reform measures on the U.S. healthcare industry is unclear.

In addition, other legislative changes have been proposed and adopted since the ACA was enacted. For example, in August 2011, President Obama signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend proposals in spending reductions to Congress. The Joint Select Committee on Deficit Reduction did not achieve its targeted deficit reduction of at least $1.2 trillion for the years 2013 through 2021, triggering the legislation's automatic reductions to several government programs. These reductions include aggregate reductions to Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013, and, due to subsequent legislative amendments, including the BBA, will stay in effect through 2030 unless additional Congressional action is taken. The Coronavirus Aid, Relief and Economic Security Act, or CARES Act, which was signed into law in March 2020 and is designed to provide financial support and resources to individuals and businesses affected by the COVID-19 pandemic, suspended the 2% Medicare sequester from May 1, 2020 through December 31, 2020, and extended the sequester by one year, through 2030. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, further reduced Medicare payments to several providers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

Further, there has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. As a result, there have been several U.S. Congressional inquiries and proposed and enacted federal and state legislation designed to, among other things, bring more transparency to drug pricing, review the relationship between pricing and manufacturer patient programs, and reform government program reimbursement methodologies for drugs. At the federal level, the Trump administration's budget proposal for fiscal year 2021 includes a $135 billion allowance to support legislative proposals seeking to reduce drug prices, increase competition, lower out-of-pocket drug costs for patients, and increase patient access to lower cost generic and biosimilar drugs. Further, the Trump administration previously released a "Blueprint", or plan, to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase drug 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 Department of Health and Human Services, or HHS, has solicited feedback on some of these measures and, has implemented others under its existing authority. For example in May 2019, CMS issued a final rule to allow Medicare Advantage Plans the option of using step therapy for Part B drugs beginning January 1, 2020. This final rule codified a CMS policy change that was effective January 1, 2019. On July 24, 2020, the Trump administration announced four executive orders related to prescription drug pricing that attempt to implement several of the administration's proposals, including a policy that would tie certain Medicare Part B drug prices to international drug prices, or the "most favored nation price," the details of which were released on September 13, 2020 and also expanded to cover certain Part D drugs; one that directs HHS to finalize the Canadian drug importation

proposed rule previously issued by HHS and makes other changes allowing for personal importation of drugs from Canada; one that directs HHS to finalize the rulemaking process on modifying the anti-kickback law safe harbors for discounts for plans, pharmacies, and pharmaceutical benefit managers; and one that reduces costs of insulin and epipens to patients of federally qualified health centers. The FDA also recently released a final rule, effective November 30, 2020, implementing a portion of the importation executive order providing guidance for states to build and submit importation plans for drugs from Canada. While some measures may require additional authorization to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative, administrative and/or executive measures to control drug costs. Individual states in the United States have also increasingly passed legislation and implemented regulations designed to control pharmaceutical 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, designed to encourage importation from other countries and bulk purchasing.

Additionally, on May 30, 2018, the Trickett Wendler, Frank Mongiello, Jordan McLinn, and Matthew Bellina Right to Try Act of 2017, or the Right to Try Act, was signed into law. The law, among other things, provides a federal framework for certain patients to access certain investigational new drug products that have completed a Phase 1 clinical trial and that are undergoing investigation for FDA approval. Under certain circumstances, eligible patients can seek treatment without enrolling in clinical trials and without obtaining FDA permission under the FDA expanded access program. There is no obligation for a manufacturer to make its products available to eligible patients as a result of the Right to Try Act.

It is possible that additional governmental action is taken in response to the COVID-19 pandemic. For example, on August 6, 2020, the Trump administration issued an executive order that instructs the federal government to develop a list of "essential" medicines and then buy them and other medical supplies from U.S. manufacturers instead of from companies around the world, including China. The order is meant to reduce regulatory barriers to domestic pharmaceutical manufacturing and catalyze manufacturing technologies needed to keep drug prices low and the production of drug products in the United States.

We expect that the ACA, as well as other healthcare reform measures that may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we may receive for any approved product. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or if we receive regulatory approval, commercialize our products.

* ***If we fail to comply with federal and state healthcare laws, including fraud and abuse and health information privacy and security laws, we could face substantial penalties and our business, results of operations, financial condition and prospects could be adversely affected.***

As a pharmaceutical company, even though we do not and will not control referrals of healthcare services or bill directly to Medicare, Medicaid or other third-party payors, certain federal and state healthcare laws and regulations pertaining to fraud and abuse and patients' rights are and will be applicable to our business. For example, we could be subject to healthcare fraud and abuse and patient privacy regulation by both the federal government and the states in which we conduct our business. The laws that may affect our ability to operate include, among others:

- The federal Anti-Kickback Statute, which regulates our business activities, including our marketing practices, educational programs, pricing policies, and relationships with healthcare providers or other entities, by prohibiting, among other things, soliciting, receiving, offering or paying remuneration, directly or indirectly, to induce, or in return for, either the referral of an individual or the purchase or recommendation of an item or service reimbursable under a federal healthcare program, such as the Medicare and Medicaid programs;
- Federal civil and criminal false claims laws, including the False Claims Act which permits a private individual acting as a "whistleblower" to bring actions on behalf of the federal government alleging violations of the False Claims Act, and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment from Medicare, Medicaid, or other third-party payors that are false or fraudulent;

- The federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal civil and criminal statutes that prohibit, among other things, executing a scheme to defraud any healthcare benefit program or making false statements relating to healthcare matters;
- HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their implementing regulations, which imposes certain requirements relating to the privacy, security and transmission of individually identifiable health information on entities and individuals subject to the law including certain healthcare providers, health plans, and healthcare clearinghouses, known as covered entities, as well as individuals and entities that perform services for them which involve the use, or disclosure of, individually identifiable health information, known as business associates; and their subcontractors that use, disclose or otherwise process individually idendifiable health information;
- Requirements under the Physician Payments Sunshine Act to report annually to CMS certain financial arrangements with physicians, as defined by such law, and teaching hospitals, as defined in the ACA and its implementing regulations, including reporting any "transfer of value" made or distributed to teaching hospitals, and physicians, as defined by such law and reporting any ownership and investment interests held by physicians and their immediate family members and applicable group purchasing organizations during the preceding calendar year, which will be expanded beginning in 2022, to require applicable manufacturers to report such information regarding its relationships with physician assistants, nurse practitioners, clinical nurse specialists, certified registered nurse anesthetists and certified nurse midwives during the previous year; and
- State and foreign law equivalents of each of the above federal laws, such as anti-kickback and false claims laws which may apply to items or services reimbursed by any third-party payor, including commercial insurers; state laws that require pharmaceutical companies to comply with the industry's voluntary compliance guidelines and the applicable compliance guidance promulgated by the federal government that otherwise restricts certain payments that may be made to healthcare providers and entities; state laws that require drug manufacturers to report information related to payments and other transfer of value to physicians and other healthcare providers and entities; state laws that require the reporting of information related to drug pricing; state and local laws that require the registration of pharmaceutical sales representatives; and state and foreign laws governing the privacy and security of health information in certain circumstances, many of which differ from each other in significant ways and often are not preempted by HIPAA, thus complicating compliance efforts.

Because of the breadth of these laws and the narrowness of the statutory exceptions and safe harbors available, it is possible that some of our business activities, including our consulting agreements with physicians, some of whom receive stock or stock options as compensation for their services, could be subject to challenge under one or more of such laws. In addition, recent health care reform legislation has further strengthened these laws. For example, the ACA, among other things, amended the intent requirement of the federal Anti-Kickback Statute and certain criminal healthcare fraud statutes. A person or entity no longer needs to have actual knowledge of this statute or specific intent to violate it. Moreover, the ACA provides that the government may assert that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the False Claims Act.

To the extent that any of our product candidates is ultimately sold in a foreign country, we may be subject to similar foreign laws and regulations.

Efforts to ensure that our business arrangements comply with applicable healthcare laws involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices do not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, we may be subject to significant penalties, including administrative, civil and criminal penalties, damages, fines, exclusion from participation in United States federal or state health care programs, such as Medicare and Medicaid, disgorgement, imprisonment, integrity oversight and reporting obligations, and the curtailment or restructuring of our operations any of which could materially adversely affect our

ability to operate our business and our financial results. Although compliance programs can mitigate the risk of investigation and prosecution for violations of these laws, the risks cannot be entirely eliminated. Any action against us for violation of these laws, even if we successfully defend against it, could cause us to incur significant legal expenses and divert our management's attention from the operation of our business. Moreover, achieving and sustaining compliance with applicable federal and state privacy, security and fraud laws may prove costly.

Our immuno-oncology product candidates may face competition in the future from biosimilars.

The Biologics Price Competition and Innovation Act of 2009, or BPCIA, provides an abbreviated pathway for the approval of follow-on biological products. Under the BPCIA, an application for a biosimilar product cannot be approved by the FDA until 12 years after the original branded product was approved under a BLA. However, there is a risk that the U.S. Congress could amend the BPCIA to significantly shorten this exclusivity period, potentially creating the opportunity for generic competition sooner than anticipated. Further, this data exclusivity does not prevent another company from developing a product that is highly similar to the original branded product, generating its own data and seeking approval. Data exclusivity only assures that another company cannot rely upon the data within the innovator's application to support the biosimilar product's approval.

RISKS RELATED TO OUR INTELLECTUAL PROPERTY

If we or our licensors fail to adequately protect or enforce our intellectual property rights or secure rights to patents of others, the value of our intellectual property rights would diminish and our ability to successfully commercialize our products may be impaired.

Our success, competitive position, and future revenues will depend in part on our ability and the abilities of our licensors to obtain and maintain patent protection for our products, methods, processes and other technologies, to preserve confidential information, including trade secrets, to prevent third parties from infringing our proprietary rights, and to operate without infringing the proprietary rights of third parties.

To date, we have exclusive rights in the field of cancer treatment to certain U.S. and foreign intellectual property with respect to the PGEN technology, including Ad-RTS-IL-12 plus veledimex, and with respect to CAR+ T, NK and TCR cell therapies arising from the laboratory of Laurence Cooper, M.D., Ph.D., who was then at MD Anderson. Under our License Agreement with PGEN, PGEN has the right, but not the obligation, to prepare, file, prosecute, and maintain the patents and patent applications licensed to us and shall bear any related costs incurred by it in regard to those actions. PGEN is required to consult with us and keep us reasonably informed of the status of the patents and patent applications licensed to us, and to confer with us and incorporate our comments prior to submitting any related filings and correspondence. Although under the agreement PGEN has agreed to consider in good faith and consult with us regarding any comments we may have regarding these patents and patent applications, we cannot guarantee that our comments will be solicited or followed. Under the MD Anderson License, future filings and applications require the agreement of each of MD Anderson, PGEN and us, and MD Anderson has the right to control the preparation and filing of additional patent applications unless the parties agree that we or PGEN may prosecute the application directly. Although under the agreement MD Anderson has agreed to review and incorporate any reasonable comments that we or PGEN may have regarding these patents and patent applications, we cannot guarantee that our comments will be solicited or followed. Under the patent license agreement with the NCI, the NCI is responsible for the preparation, filing, prosecution, and maintenance of patent applications or patents licensed to us. Although under the agreement, the NCI is required to consult with us in the preparation, filing, prosecution, and maintenance of all patent applications or patents licensed to us, we cannot guarantee that our comments will be solicited or followed. Without direct control of the in-licensed patents and patent applications, we are dependent on PGEN, MD Anderson or the NCI, as applicable, to keep us advised of prosecution, particularly in foreign jurisdictions where prosecution information may not be publicly available. We anticipate that we, PGEN, MD Anderson and the NCI will file additional patent applications both in the United States and in other countries. However, we cannot predict or guarantee:

- The degree and range of protection any patents will afford us against competitors, including whether third parties will find ways to invalidate or otherwise circumvent our patents;

- If and when patents will be issued;
- Whether or not others will obtain patents claiming subject matter related to or relevant to our product candidates; or
- Whether we will need to initiate litigation or administrative proceedings that may be costly whether we win or lose.

The patent position of biotechnology and pharmaceutical companies generally is highly uncertain, involves complex legal and factual questions and has in recent years been the subject of much litigation. In addition, the laws of foreign countries may not protect our rights to the same extent as the laws of the United States and we may fail to seek or obtain patent protection in all major markets. For example, European patent law restricts the patentability of methods of treatment of the human body more than United States law does. Publications of discoveries in the scientific literature often lag behind the actual discoveries, and patent applications in the United States and other jurisdictions are typically not published until 18 months after filing, or in some cases not at all.

Changes in patent laws or in interpretations of patent laws in the United States and other countries may diminish the value of our intellectual property or narrow the scope of our patent protection. In September 2011, the Leahy- Smith America Invents Act, or the Leahy-Smith Act, was signed into law, resulting in a number of significant changes to United States patent law. These changes include provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. In addition, the United States Supreme Court has ruled on several patent cases in recent years, either narrowing the scope of patent protection available in certain circumstances or weakening the rights of patent owners in certain situations. This combination of events has created uncertainty with respect to the value of patents, once obtained, and with regard to our ability to obtain patents in the future. As the USPTO continues to implement the Leahy-Smith Act, and as the federal courts have the opportunity to interpret the Leahy-Smith Act, the laws and regulations governing patents, and the rules regarding patent procurement 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.

Certain technologies utilized in our research and development programs are already in the public domain. Moreover, a number of our competitors have developed technologies, filed patent applications or obtained patents on technologies, compositions and methods of use that are related to our business and may cover or conflict with our owned or licensed patent applications, technologies or product candidates. Such conflicts could limit the scope of the patents that we may be able to obtain or may result in the rejection of claims in our patent applications. Because patent applications in the United States and many foreign jurisdictions are typically not published until 18 months after filing, or in some cases not at all, and because publications of discoveries in the scientific literature often lag behind actual discoveries, neither we nor our licensors can be certain that others have not filed or maintained patent applications for technology used by us or covered by our pending patent applications without our being aware of these applications. Therefore, we cannot know with certainty whether we were the first to make the inventions claimed in our owned patents or pending patent applications, or that we were the first to file for patent protection of such inventions, nor can we know whether those from whom we license patents were the first to make the inventions claimed or were the first to file. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Our pending and future patent applications may not result in patents being issued which protect our technology or products, in whole or in part, or which effectively prevent others from commercializing competitive technologies and products. Changes in either the patent laws or interpretation of the patent laws in the United States and other countries may diminish the value of our patents or narrow the scope of our patent protection. In addition, our own earlier filed patents and applications or those of PGEN MD Anderson, or NCI may limit the scope of later patents we obtain or may result in the rejection of claims in our later filed patent applications. If third parties filed patent applications or obtained patents on technologies, compositions and methods of use that are related to our business and that cover or conflict with our owned or licensed patent applications, technologies or product candidates, we may be required to challenge such protection, terminate or modify our programs impacted by such protection or obtain licenses from such third parties, which might not be available on acceptable terms, or at all.

Even if our owned and licensed patent applications issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors from competing with us or otherwise provide us with any competitive advantage. Our competitors may be able to circumvent our owned or licensed patents by developing similar or alternative technologies or products in a non-infringing manner.

The issuance of a patent is not conclusive as to its inventorship, scope, validity or enforceability, and our owned and licensed patents may be challenged in the courts or patent offices in the United States and abroad. Such challenges may result in loss of exclusivity or freedom to operate or in patent claims being narrowed, invalidated or held unenforceable, in whole or in part, which could limit our ability to stop others from using or commercializing similar or identical technology and products, or limit the duration of the patent protection of our technology and products. 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 confidential information, our business and competitive position would be harmed.

Our success also depends upon the skills, knowledge, and experience of our scientific and technical personnel, our consultants and advisors, as well as our licensors and contractors. To help protect our proprietary know-how and our inventions for which patents may be unobtainable or difficult to obtain, and to maintain our competitive position, we rely on trade secret protection and confidentiality agreements. To this end, it is our general policy to require our employees, consultants, advisors, and contractors to enter into agreements that prohibit the disclosure of confidential information and, where applicable, require disclosure and assignment to us of the ideas, developments, discoveries, and inventions important to our business. These agreements may not provide adequate protection for our trade secrets, know-how, confidential information or other proprietary information in the event of any unauthorized use or disclosure or the lawful development by others of such information. Moreover, we may not be able to obtain adequate remedies for any breaches of these agreements. Our trade secrets or other confidential information may also be obtained by third parties by other means, such as breaches of our physical or computer security systems. Enforcing a claim that a party illegally disclosed or misappropriated a trade secret or other confidential information is difficult, expensive and time-consuming, and the outcome is unpredictable. In addition, some courts inside and outside the United States are less willing or unwilling to protect trade secrets. If any of our trade secrets or other confidential information were to be lawfully obtained or independently developed by a competitor, we would have no right to prevent them, or those to whom they communicate it, from using that technology or information to compete with us. If any of our trade secrets, know-how or other proprietary information is disclosed, the value of our trade secrets, know-how and other proprietary rights would be significantly impaired and our business and competitive position would suffer.

Third-party claims of intellectual property infringement would require us to spend significant time and money and could prevent us from developing or commercializing our products.

In order to protect or enforce patent rights, we may initiate patent infringement litigation against third parties. Similarly, we may be sued by others for patent infringement. We also may become subject to proceedings conducted in the United States Patent and Trademark Office, including interference proceedings to determine the priority or derivation of inventions, or post-grant review, inter partes review, or reexamination proceedings reviewing the patentability of our patented claims. In addition, any foreign patents that are granted may become subject to opposition, nullity, or revocation proceedings in foreign jurisdictions having such proceedings. The defense and prosecution, if necessary, of intellectual property actions are costly and divert technical and management personnel away from their normal responsibilities.

Our commercial success depends upon our ability, and the ability of our collaborators, to develop, manufacture, market and sell our product candidates without infringing the proprietary rights of third parties. There is considerable intellectual property litigation in the biotechnology and pharmaceutical industries. While no such litigation has been brought against us and we have not been held by any court to have infringed a third party's intellectual property rights, we cannot guarantee that our products or use of our products do not infringe third- party patents. It is also possible that we have failed to identify relevant third-party patents or applications. For example, applications filed before November 29, 2000 and certain applications filed after that date that will not be filed outside the United States remain confidential until patents issue. Patent applications in the United States and elsewhere are published approximately 18 months after the earliest filing, which is referred to as the priority date. Therefore, patent applications covering our products or technology could have been filed by others without our knowledge. Additionally, pending patent applications which have been published can, subject to certain limitations, be later amended in a manner that could cover our products or the use of our products.

Our research, development and commercialization activities, as well as any product candidates or products resulting from these activities, may infringe or be claimed to infringe patents or patent applications under which we do not hold licenses or other rights. Patents do not protect its owner from a claim of infringement of another owner's patent. Therefore, our patent position cannot and does not provide any assurance that we are not infringing the patent rights of another.

The patent landscape in the field of immuno-oncology is particularly complex. We are aware of numerous United States and foreign patents and pending patent applications of third parties that cover compositions, methods of use and methods of manufacture of immuno-oncology products. In addition, there may be patents and patent applications in the field of which we are not aware. The technology we license from PGEN, MD Anderson and NCI is early-stage technology and we are in the process of designing and developing products using this technology. Although we will seek to avoid pursuing the development of products that may infringe any patent claims that we believe to be valid and enforceable, we may fail to do so. Moreover, given the breadth and number of claims in patents and pending patent applications in the field of immuno-oncology and the complexities and uncertainties associated with them, third parties may allege that we are infringing patent claims even if we do not believe such claims to be valid and enforceable.

If a claim for patent infringement is asserted, there can be no assurance that the resolution of the claim would permit us to continue marketing the relevant product on commercially reasonable terms, if at all. We may not have sufficient resources to bring these actions to a successful conclusion. If we do not successfully defend any infringement actions to which we become a party or are unable to have infringed patents declared invalid or unenforceable, we may have to pay substantial monetary damages, which can be tripled if the infringement is deemed willful or be required to discontinue or significantly delay commercialization and development of the affected products.

Any legal action against us or our collaborators claiming damages and seeking to enjoin developmental or marketing activities relating to affected products could, in addition to subjecting us to potential liability for damages, require us or our collaborators to obtain licenses to continue to develop, manufacture, or market the affected products. Such a license may not be available to us on commercially reasonable terms, if at all.

An adverse determination in a proceeding involving our owned or licensed intellectual property may allow entry of substitutes, including biosimilar or generic substitutes, for our products.

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 noncompliance with these requirements.

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance 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. Noncompliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. In such an event, our competitors might be able to enter the market, which would have a material adverse effect on our business.

We license rights to products and technology that are important to our business, and we expect to enter into additional licenses in the future. For instance, we have exclusively licensed patents and patent applications under our License Agreement with PGEN as well as under the MD Anderson License and our patent license agreement with the NCI. Under these agreements, we are subject to a range of commercialization and development, sublicensing, royalty, patent prosecution and maintenance, insurance and other obligations.

Any failure by us to comply with any of these obligations or any other breach by us of our license agreements could give the licensor the right to terminate the license in whole, terminate the exclusive nature of the license or bring a claim against us for damages. Any such termination or claim could have a material adverse effect on our financial condition, results of operations, liquidity or business. Even if we contest any such termination or claim and are ultimately successful, such dispute could lead to delays in the development or commercialization of potential products and result in time-consuming and expensive litigation or arbitration. On termination we may be required to license to the licensor any related intellectual property that we developed.

In addition, in certain cases, the rights licensed to us are rights of a third party licensed to our licensor. In such instances, if our licensors do not comply with their obligations under such licenses, our rights under our license agreements with our licensor may be adversely affected.

We may be subject to claims by third parties asserting that our employees or we have misappropriated their intellectual property or claiming ownership of what we regard as our own intellectual property.

Many of our employees 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 do not use the proprietary information or know-how of others in their work for us, we may be subject to claims that these employees or we have used or disclosed intellectual property, including trade secrets or other proprietary information, of any such employee's former employer. Litigation may be necessary to defend against these claims.

In addition, while it is our policy to require our employees and contractors who may be involved in the development of intellectual property to execute agreements assigning such intellectual property to us, we may be unsuccessful in executing such an agreement with each party who in fact develops intellectual property that we regard as our own. Our and their assignment agreements may not be self-executing or may be breached, and we may be forced to bring claims against third parties, or defend claims they may bring against us, to determine the ownership of what we regard as our intellectual property.

If we fail in prosecuting or defending any such claims, in addition to paying monetary damages, we may lose valuable intellectual property rights or personnel. Even if we are successful in prosecuting or defending against such claims, litigation could result in substantial costs and be a distraction to management.

OTHER RISKS RELATED TO OUR COMPANY

**** Our stock price has been, and may continue to be, volatile.***

The market price for our common stock is volatile and may fluctuate significantly in response to a number of factors, most of which we cannot control, including:

- Price and volume fluctuations in the overall stock market;
- Changes in operating results and performance and stock market valuations of other biopharmaceutical companies generally, or those that develop and commercialize cancer drugs in particular;
- Market conditions or trends in our industry or the economy as a whole;
- Preclinical or clinical trial results;
- Public concern as to the safety of drugs developed by us or others;
- The financial or operational projections we may provide to the public, any changes in these projections or our failure to meet these projections;
- Comments by securities analysts or changes in financial estimates or ratings by any securities analysts who follow our common stock, our failure to meet these estimates or failure of those analysts to initiate or maintain coverage of our common stock;
- The public's response to press releases or other public announcements by us or third parties, including our filings with the SEC, as well as announcements of the status of development of our products, announcements of technological innovations or new therapeutic products by us or our competitors, announcements regarding collaborative agreements and other announcements relating to product development, litigation and intellectual property impacting us or our business;
- Government regulation;
- FDA determinations on the approval of a product candidate BLA submission;
- The sustainability of an active trading market for our common stock;
- Future sales of our common stock by us, our executive officers, directors and significant stockholders;
- Announcements of mergers or acquisition transactions;
- Our inclusion or deletion from certain stock indices;
- Developments in patent or other proprietary rights;
- Changes in reimbursement policies;
- Announcements of medical innovations or new products by our competitors;
- Announcements of changes in our senior management or directors;
- General economic, industry, political and market conditions, including, but not limited to, the ongoing impact of the COVID-19 pandemic;
- Other events or factors, including those resulting from war, incidents of terrorism, natural disasters or responses to these events; and
- Changes in accounting principles.

In addition, the stock market in general and our stock in particular from time to time experiences significant price and volume fluctuations unrelated to the operating performance of particular companies, including in connection with the ongoing COVID-19 pandemic, which has resulted in decreased stock prices for many companies notwithstanding the lack of a fundamental change in their underlying business models or prospects. Public debt and equity markets, and in particular the Nasdaq Capital Market, have experienced extreme price and volume fluctuations that have affected and continue to affect the market prices of equity securities of many biopharmaceutical companies.

Stock prices of many biopharmaceutical companies have fluctuated in a manner unrelated or disproportionate to the operating performance of those companies. In the past, stockholders have instituted securities class action litigation following periods of market volatility. If we were involved in securities litigation, we could incur substantial costs and our resources, and the attention of management could be diverted from our business.

Anti-takeover provisions in our charter documents and under Delaware law may make an acquisition of us, which may be beneficial to our stockholders, more difficult.

Provisions of our amended and restated certificate of incorporation and bylaws, as well as provisions of Delaware law, could make it more difficult for a third party to acquire us, even if doing so would benefit our stockholders. These provisions authorize the issuance of "blank check" preferred stock that could be issued by our board of directors to increase the number of outstanding shares and hinder a takeover attempt, and limit who may call a special meeting of stockholders. In addition, Section 203 of the Delaware General Corporation Law generally prohibits a publicly-held Delaware corporation from engaging in a business combination with a party that owns at least 15% of its common stock unless the business combination is approved by the company's board of directors before the person acquires the 15% ownership stake or later by its board of directors and two-thirds of its stockholders. Section 203 could have the effect of delaying, deferring or preventing a change in control that our stockholders might consider to be in their best interests.

Because we do not expect to pay dividends, you will not realize any income from an investment in our common stock unless and until you sell your shares at profit.

We have never paid dividends on our common stock and we do not anticipate that we will pay any dividends for the foreseeable future. Accordingly, any return on an investment in us will be realized, if at all, only when you sell shares of our common stock.

Our ability to use net operating loss carryforwards and research tax credits to reduce future tax payments may be limited or restricted.

We have generated significant net operating loss carryforwards, or NOLs, and research and development tax credits, or R&D credits, as a result of our incurrence of losses and our conduct of research activities since inception. We generally are able to carry NOLs and R&D credits forward to reduce our tax liability in future years. However, our ability to utilize the NOLs and R&D credits is subject to the rules of Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, or the Code, respectively. Those sections generally restrict the use of NOLs and R&D credits after an "ownership change." An ownership change occurs if, among other things, the stockholders (or specified groups of stockholders) who own or have owned, directly or indirectly, 5% or more of a corporation's common stock or are otherwise treated as 5% stockholders under Section 382 of the code and the United States Treasury Department regulations promulgated thereunder increase their aggregate percentage ownership of that corporation's stock by more than 50 percentage points over the lowest percentage of the stock owned by these stockholders over the applicable testing period. In the event of an ownership change, Section 382 imposes an annual limitation on the amount of taxable income a corporation may offset with NOL carry forwards and Section 383 imposes an annual limitation on the amount of tax a corporation may offset with business credit (including the R&D credit) carry forwards.

We may have experienced an "ownership change" within the meaning of Section 382 in the past and there can be no assurance that we will not experience additional ownership changes in the future. As a result, our NOLs and business credits (including the R&D credit) may be subject to limitations and we may be required to pay taxes earlier and in larger amounts than would be the case if our NOLs or R&D credits were freely usable.

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

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

* ***Our principal stockholders, executive officers and directors have substantial control over the company, which may prevent you and other stockholders from influencing significant corporate decisions and may harm the market price of our common stock.***

As of September 30, 2020, our executive officers, directors and holders of five percent or more of our outstanding common stock, beneficially owned, in the aggregate, 42.0% of our outstanding common stock. These stockholders may have interests that conflict with our other stockholders and, if acting together, have the ability to influence the outcome of matters submitted to our stockholders for approval, including the election and removal of directors and any merger, consolidation or sale of all or substantially all of our assets. Accordingly, this concentration of ownership may harm the market price of our common stock by:

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

* ***We have identified a material weakness in our internal control over financial reporting for the year ended December 31, 2019 and may identify additional material weaknesses in the future or otherwise fail to maintain an effective system of internal controls, which may result in material misstatements of our financial statements or could have a material adverse effect on our business and trading price of our securities.***

We are subject to the reporting requirements of the Securities Exchange Act of 1934, as amended, the Sarbanes- Oxley Act of 2002 and the rules and regulations of The Nasdaq Global Market. Pursuant to Section 404 of the Sarbanes-Oxley Act of 2002, we are required to perform system and process evaluation and testing of our internal control over financial reporting to allow our management to report on the effectiveness of our internal control over financial reporting. We are also required to have our independent registered public accounting firm issue an opinion on the effectiveness of our internal control over financial reporting on an annual basis.

In connection with the audit of our consolidated financial statements as of and for the year ended December 31, 2019, we identified a material weakness in our internal control over financial reporting, which continued to exist at September 30, 2020. A material weakness is a deficiency, or combination of deficiencies, in internal control over financial reporting such that there is a reasonable possibility that a material misstatement of our consolidated financial statements will not be prevented or detected on a timely basis. The material weakness is related to the design and maintenance of effective controls relating to the monitoring and oversight of expensing third party clinical trial costs. Specifically, our internal controls were not designed effectively to provide reasonable assurance regarding the accurate and timely evaluation of the amount of third-party costs to record.

We are in the process of designing and implementing measures to remediate the underlying causes of the control deficiencies that gave rise to the material weakness. In addition, we are providing in-house accounting personnel training to ensure that they have the relevant expertise related to the monitoring and oversight of expensing third party clinical trial costs. We will continue to monitor the effectiveness of these controls and will make any further changes management determines appropriate.

We cannot assure you that the measures we have taken to date, together with any measures we may take in the future, will be sufficient to remediate the control deficiencies that led to the material weakness in our internal control over financial reporting or to avoid potential future material weaknesses. If we are unable to successfully remediate our existing or any future material weakness in our internal control over financial reporting, or if we identify any additional material weaknesses, the accuracy and timing of our financial reporting may be adversely affected. If we are unable to maintain effective internal controls, we may not have adequate, accurate or timely financial information, and we may be unable to meet our reporting obligations as a public company, including the requirements of the Sarbanes-Oxley Act, we may be unable to accurately report our financial results in future periods, or report them within the timeframes required by the requirements of the SEC, Nasdaq or the Sarbanes- Oxley Act. Failure to comply with the Sarbanes-Oxley Act, when and as applicable, could also potentially subject us to sanctions or investigations by the SEC or other regulatory authorities. Any failure to maintain or implement required new or improved controls, or any difficulties we encounter in their implementation, could result in identification of additional material weaknesses or significant deficiencies, cause us to fail to meet our reporting obligations or result in material misstatements in our financial statements. Furthermore, if we cannot provide reliable financial reports or prevent fraud, our business and results of operations could be harmed and investors could lose confidence in our reported financial information.

The Tax Cuts and Jobs Act, signed into law in 2017 could adversely affect our business and financial condition.

On December 22, 2017, President Trump signed into law legislation, known as the Tax Cuts and Jobs Act of 2017, or Tax Act, that significantly revises the Code. The federal income tax law is referred to as the Tax Act, and contains significant changes to corporate taxation, including reduction of the corporate tax rate from a top marginal rate of 35% to a flat rate of 21%, limitation of the tax deduction for interest expense to 30% of adjusted earnings (except for certain small businesses), limitation of the deduction for NOLs to 80% of current year taxable income and elimination of NOL carrybacks, one time taxation of offshore earnings at reduced rates regardless of whether they are repatriated, elimination of U.S. tax on foreign earnings (subject to certain important exceptions), immediate deductions for certain new investments instead of deductions for depreciation expense over time, and modifying or repealing many business deductions and credits. Notwithstanding the reduction in the corporate income tax rate, the overall impact of the Tax Act is uncertain and our business and financial condition could be adversely affected. In addition, it is uncertain if and to what extent various states will conform to the Tax Act. The impact of the Tax Act on holders of our common stock is also uncertain and could be adverse. We urge our stockholders to consult with their legal and tax advisors with respect to this legislation and the potential tax consequences of investing in or holding our common stock.

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

None.

Item 3. Defaults upon Senior Securities

Not applicable.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

None.

Item 6. Exhibits

Exhibit Number	Description
3.1	Amended and Restated Certificate of Incorporation of the Registrant, as filed with the Delaware Secretary of State on April 26, 2006 (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, SEC File No. 000-32353, filed April 26, 2006).
3.2	Amended and Restated Bylaws of the Registrant (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K, SEC File No. 001-33038, filed September 22, 2020).
3.3	Amended and Restated Certificate of Designation, Preferences and Rights of Series 1 preferred stock, as filed with the Delaware Secretary of State on July 1, 2016 (incorporated by reference to Exhibit 3.1 to the Registrant's Current Report on Form 8-K/A, SEC File No. 001-33038, filed July 1, 2016).
10.1+†	Second Amendment to Patent License Agreement, dated as of September 28, 2020, by and between the Registrant and the National Cancer Institute.
10.2+†	Amendment No. 1 to the Exclusive License Agreement by and between the Registrant and PGEN Therapeutics, Inc. (formerly known as Precigen, Inc.), dated October 15, 2020.
31.1+	Certification of Principal Executive Officer pursuant to Exchange Act rules 13a-14 or 15d-14 under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002.
31.2+	Certification of Principal Financial Officer pursuant to Exchange Act rules 13a-14 or 15d-14 under the Securities Exchange Act of 1934, as Adopted Pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1++	Certifications of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as Adopted Pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS+	Inline 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+	Inline XBRL Taxonomy Extension Schema Document
101.CAL+	Inline XBRL Taxonomy Extension Calculation Linkbase Document
101.DEF+	Inline XBRL Taxonomy Definition Linkbase Document
101.LAB+	Inline XBRL Taxonomy Extension Label Linkbase Document
101.PRE+	Inline XBRL Taxonomy Extension Presentation Linkbase Document
104+	Cover Page Interactive Data File—the cover page interactive data is embedded within the Inline XBRL document or included within the Exhibit 101 attachments

\+ Filed herewith.

++ This certification is deemed not filed for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liability of that section, nor shall it be deemed incorporated by reference into any filing under the Securities Act of 1933, as amended, or the Securities Exchange Act of 1934, as amended, whether made before or after the date hereof, regardless of any general incorporation language in such filing.

† Certain portions of this exhibit (indicated by asterisks) have been omitted because they are not material and would likely cause competitive harm to Ziopharm Oncology, Inc. if publicly disclosed.

SIGNATURES

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

ZIOPHARM ONCOLOGY, INC.

By:

/s/ Laurence J.N. Cooper

Laurence J.N. Cooper, M.D., Ph.D.
Chief Executive Officer
(On behalf of the Registrant and as Principal Executive Officer)
Dated: November 5, 2020

By:

/s/ Satyavrat Shukla

Satyavrat Shukla
Executive Vice President and Chief Financial Officer
(Principal Financial Officer)
Dated: November 5, 2020

CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY [***], HAS BEEN OMITTED BECAUSE ZIOPHARM ONCOLOGY, INC. HAS DETERMINED THE INFORMATION (I) IS NOT MATERIAL AND (II) WOULD LIKELY CAUSE COMPETITIVE HARM TO ZIOPHARM ONCOLOGY, INC. IF PUBLICLY DISCLOSED.

PUBLIC HEALTH SERVICE

Amendment

This **Agreement** is based on the model Amendment Agreement adopted by the U.S. Public Health Service ("**PHS**") Technology Transfer Policy Board for use by components of the National Institutes of Health ("**NIH**"), the Centers for Disease Control and Prevention ("**CDC**"), and the Food and Drug Administration ("**FDA**"), which are agencies of the **PHS** within the Department of Health and Human Services ("**HHS**").

This Cover Page identifies the Parties to this **Agreement**:

The U.S. Department of Health and Human Services, as represented by

National Cancer Institute

an Institute or Center (hereinafter referred to as the "**IC**") of the

NIH

and

Ziopharm Oncology, Inc.,

hereinafter referred to as the "**Licensee**",

having offices at One First Avenue, Parris Building #34, Navy Yard Plaza, Boston, MA 02129,

created and operating under the laws of Delaware.

Tax ID No.: 84-1475642

A-362-2020

SECOND AMENDMENT TO L-190-2019/0

This is the second amendment ("**Second Amendment**") of the agreement by and between the **IC** and **Licensee** having an effective date of May 28, 2019 and having **IC** Reference Number L-190-2019/0 ("**Agreement**"). This **Second Amendment**, having **IC** Reference Number L-190-2019/2 includes, in addition to the amendments made below, 1) a Signature Page, 2) Attachment 1 (Royalty Payment Information), and 3) Appendix A - Patent(s) or Patent Application(s).

WHEREAS, the **IC** and the **Licensee** desire that the **Agreement** be amended a second time as set forth below in order to bring additional patent rights within the scope of the **Agreement**.

NOW, THEREFORE, in consideration of the mutual covenants and promises contained herein, the **IC** and the **Licensee**, intending to be bound, hereby mutually agree to the following:

1) The cover page's "Serial Number(s) of Licensed Patent(s) or Patent Application(s)" section of the **Agreement**, including the complete list of Licensed Patent(s) or Patent Application(s) in this section, shall be deleted. Appendix A of this Second Amendment is hereby incorporated by reference herein.

2) Appendix A - Patent(s) or Patent Application(s) of the **Agreement** shall be deleted and replaced with Appendix A - Patent(s) or Patent Application(s) of this **Second Amendment**.

3) Section 2.2 of the **Agreement** shall be deleted and replaced with the following:

"Additional T Cell Receptor" means an identified human, murine or human-murine hybrid T cell receptor which immunologically recognizes a tumor-mutated peptide derived from epidermal growth factor receptor (EGFR), Kirsten rat sarcoma viral oncogene homolog (KRAS), or p53 (also known as TP53 or tumor protein p53) presented by a human leukocyte antigen ("**HLA**") allele group (e.g., HLA-A*02, HLA-A*03) (the peptide and the HLA allele group together, "**Peptide-HLA Complex**").

4) Appendix C – VII of the **Agreement** shall be deleted and replaced with the following:

VII. As used herein, a "[***]" means an **Additional T Cell Receptor** that immunologically recognizes a **Peptide-HLA Complex** that is [***].

As used herein, a "[***]" means any **Additional T Cell Receptor** that is [***].

In the event an amendment adds several **Additional T Cell Receptors** that [***].

Subject to Paragraph 14.4 of this **Agreement**, for each **Additional T Cell Receptor** added by written amendment, the non-creditable, non-refundable amendment issue royalty shall be as follows for each such **Additional T Cell Receptor**:

Type of Additional T Cell Receptor	Royalty amount
[***] recognizing mutated [***] peptide	$ [***]
Each [***] recognizing mutated [***] peptide	$ [***]
[***] recognizing mutated [***] peptide	$ [***]
[***] recognizing mutated [***] peptide	$ [***]
Each [***] recognizing mutated [***] peptide	$ [***]

In addition, for each **Additional T Cell Receptor** added by written amendment, Licensee shall pay the same earned royalty, benchmark royalty and sublicensing royalty rates that are applicable to **Licensed Fields of Use** 1-3.

5) Section 14.6 of the **Agreement** shall be deleted and replaced with the following:

14.6 All **Agreement** notices required or permitted by this **Agreement** shall be given by (i) prepaid, first class, registered or certified mail or by an express/overnight delivery service provided by a commercial carrier, properly addressed to the other party at the address designated on the following Signature Page, or to another address as may be designated in writing by the other party, or (ii) by email to the address designated on the following Signature Page, with receipt confirmed by return email from the recipient. **Agreement** notices shall be considered timely if the notices are received on or before the established deadline date or sent on or before the deadline date by email or as verifiable by U.S. Postal Service postmark or dated receipt from a commercial carrier. Parties should request a legibly dated U.S. Postal Service postmark or obtain a dated receipt from a commercial carrier or the U.S. Postal Service. Private metered postmarks shall not be acceptable as proof of timely mailing.

6) Within sixty (60) days of the execution of this **Second Amendment**, the **Licensee** shall pay the **IC** an amendment issue royalty in the sum of four hundred eleven thousand US Dollars ($411,000.00). Payment options may be found in Attachment 1. The parties agree that the foregoing payment obligation shall be in lieu of the non-creditable, non-refundable amendment issue royalty set forth in Paragraph VII of Appendix C of the **Agreement** for all **Additional T Cell Receptors** added pursuant to this **Second Amendment**.

7) In the event any provision(s) of the **Agreement** is/are inconsistent with Attachment 1, such provision(s) is/are hereby amended to the extent required to avoid such inconsistency and to give effect to the payment information in such Attachment 1.

8) All terms and conditions of the **Agreement** not herein amended remain binding and in effect.

9) The terms and conditions of this **Second Amendment** shall, at the **IC's** sole option, be considered by the **IC** to be withdrawn from the **Licensee's** consideration and the terms and conditions of this **Second Amendment**, and the **Second Amendment** itself, to be null and void, unless this **Second Amendment** is executed by the **Licensee** and a fully executed original is received by the **IC** within sixty (60) days from the date of the **IC's** signature found at the Signature Page.

10) This **Second Amendment** is effective upon execution by all parties.

SIGNATURES BEGIN ON NEXT PAGE

SECOND AMENDMENT TO L-190-2019/0

SIGNATURE PAGE

In Witness Whereof, the parties have executed this **Second Amendment** on the dates set forth below. Any communication or notice to be given shall be forwarded to the respective addresses listed below.

For the **IC**:

/s/ Richard U. Rodriguez — 9-21-20
Richard U. Rodriguez, MBA — Date
Associate Director
Technology Transfer Center, National Cancer Institute

National Institutes of Health Mailing Address or E-mail Address for **Agreement** notices and reports:

License Compliance and Administration
Monitoring & Enforcement
Office of Technology Transfer
National Institutes of Health
6011 Executive Boulevard, Suite 325
Rockville, Maryland 20852-3804 U.S.A.

E-mail: LicenseNotices_Repor@mail.nih.gov

For the **Licensee** (Upon information and belief, the undersigned expressly certifies or affirms that the contents of any statements of the **Licensee** made or referred to in this document are truthful and accurate.):

/s/ Robert Hadfield — 9/28/20
Signature of Authorized Official — Date

Name: Robert Hadfield
Title: General Counsel

I. Official and Mailing Address for **Agreement** notices:

Rob Hadfield
Name

General Counsel
Title

A-362-2020

Mailing Address:

One First Avenue, Parris Building #34

Navy Yard Plaza

Boston, MA 02129

Email Address: [***]

Phone: [***]

Fax: [***]

II. Official and Mailing Address for Financial notices (the Licensee's contact person for royalty payments):

Eshane Dupree
Name

Accounts Payable
Title

Mailing Address:

One First Avenue, Parris Building #34

Navy Yard Plaza

Boston, MA 02129

Email Address: [***]

Phone: [***]

Fax: [***]

Any false or misleading statements made, presented, or submitted to the **Government**, including any relevant omissions, under this **Agreement** and during the course of negotiation of this **Agreement** are subject to all applicable civil and criminal statutes including Federal statutes 31 U.S.C. §§3801-3812 (civil liability) and 18 U.S.C. §1001 (criminal liability including fine(s) or imprisonment).

A-362-2020

ATTACHMENT 1 – ROYALTY PAYMENT INFORMATION

New Payment Options Effective March 2018

The License Number MUST appear on payments, reports and correspondence.

[***]

A-362-2020

CONFIDENTIAL -NIH

APPENDIX A – PATENT(S) OR PATENT APPLICATION(S)

Patent(s) or Patent Application(s):

Group A

[***]

Group B

[***]

Group C

[***]

Group D

[***]

Group E

[***]

Group F

[***]

A-362-2020

CERTAIN CONFIDENTIAL INFORMATION CONTAINED IN THIS DOCUMENT, MARKED BY [***], HAS BEEN OMITTED BECAUSE ZIOPHARM ONCOLOGY, INC. HAS DETERMINED THE INFORMATION (I) IS NOT MATERIAL AND (II) WOULD LIKELY CAUSE COMPETITIVE HARM TO ZIOPHARM ONCOLOGY, INC. IF PUBLICLY DISCLOSED.

AMENDMENT NO. 1 TO THE EXCLUSIVE LICENSE AGREEMENT

THIS AMENDMENT NO. 1 TO THE EXCLUSIVE LICENSE AGREEMENT, (the "**Amendment No. 1**") effective as of October 15, 2020 (the "**Effective Date of Amendment No. 1**"), is made by and between **Ziopharm Oncology, Inc.**, a Delaware corporation, with its principal place of business at One First Avenue, Parris Building 34, Navy Yard Plaza, Boston, MA 02129 ("**Ziopharm**"), and **PGEN Therapeutics, Inc.** (formerly known as Precigen, Inc.), a Delaware corporation, with its principal place of business at 20358 Seneca Meadows Parkway, Germantown, MD 20876 ("**Precigen**"). Ziopharm and Precigen are sometimes referred to herein individually as a "**Party**" and collectively as the "**Parties**".

WHEREAS, Ziopharm and Precigen entered into the Exclusive License Agreement, dated as of October 5, 2018, (the "**Agreement**");

WHEREAS, Ziopharm and Precigen have a dispute regarding the delivery of certain information and materials under the Agreement, and have agreed to settle this dispute by entering into this Amendment No. 1; and

WHEREAS, through this Amendment No. 1, the Parties wish to compromise and fully and finally settle the above-referenced dispute, including all related matters in controversy and causes of action between them relating to the above-referenced dispute, without any admission of liability.

NOW, THEREFORE, in consideration of the mutual covenants and promises of the Parties contained herein, the sufficiency of which is hereby acknowledged, the Parties hereby agree as follows:

1. **Defined Terms**. The terms in this Amendment No. 1 with initial letters capitalized shall have the meanings set forth in this Amendment No. 1 and, if not defined in this Amendment No. 1, shall have the meaning set forth in the Agreement.

2. **Immediate Transfers and Deliveries**. A new Section 4.1(e) of the Agreement is hereby added to read as follows:

"**(e) Immediate Transfers and Deliveries**. Notwithstanding any other provision of the Agreement, this Section 4.1(e) together with Section 4.1(f), set forth the Parties' sole obligations and rights with respect to Precigen's provision or transfer to Ziopharm of Licensed Know-How relating to the IL-12 Program and Precigen's inventory of IL-12 Products (including all final product, drug substance, intermediates, works-in-process, formulation materials, reference

standards, drug product clinical reserve samples, cell banks, viral banks, packaged retention samples, and the like):

(i) Within [***] of the Effective Date of Amendment No. 1, Precigen will transfer and deliver to Ziopharm each of the items described on Schedule 1 attached to Amendment No. 1. Precigen represents and warrants to Ziopharm that it has Control over, and the right to transfer and deliver, each of these items described on such Schedule 1 as contemplated hereby.

(ii) Ziopharm may send follow-up notice(s) to Precigen to request additional items related to the items on Schedule 1 (the "**Additional Items**") within [***] from such transfer and delivery by Precigen to Ziopharm pursuant to Section 4.1(e)(i). Precigen will transfer and deliver the Additional Items to Ziopharm within [***] of Ziopharm's request; provided that (i) Ziopharm represents it cannot obtain the Additional Items without the participation of Precigen, and (ii) Precigen is in possession of and can reasonably locate the Additional Items. Notwithstanding the prior sentence, Precigen may transfer and deliver such Additional Items to Ziopharm within sixty [***] of Ziopharm's request provided that Precigen represents that production of the Additional Items within [***] would materially interfere with Precigen's (or the relevant individual or individuals') ongoing business or other material obligations and commitments. Ziopharm shall reimburse Precigen for its out-of-pocket expenses and FTE costs incurred to perform the transfer provided solely under this Section 4.1(e)(ii) as set forth in Section 4.2, except that the applicable FTE rate shall be [***] per hour.

(iii) Within [***] of the Effective Date of Amendment No. 1, Precigen shall prepare and send a letter to each vendor listed on Schedule 2 attached to this Amendment No. 1 (each, "**Vendor**") authorizing the Vendor to provide Ziopharm with information and documentation regarding work performed by the Vendor relating to the IL-12 Program prior to October 5, 2018, as identified by the batch/lot numbers or other specific identifying information specified on Schedule 2 (each, "**Vendor Authorization Letter**"). A model Vendor Authorization Letter is attached as Schedule 3 to Amendment No. 1. In preparing the letter, Precigen will consider whether information concerning the identified batch/lot numbers contains information that is not related to the IL-12 Program (the "**Unrelated Information**"), in which case Precigen may add language to the letter instructing the Vendor to redact or otherwise withhold from Ziopharm such Unrelated Information. Precigen will provide Ziopharm with a copy of each Vendor Authorization Letter, with redactions as determined by Precigen in its sole discretion. When requesting specific information from a Vendor pursuant to a Vendor Authorization Letter, Ziopharm shall send Precigen via email a copy of the request at least [***] prior to sending the request to the Vendor, so that Precigen may consider whether and how Ziopharm's request may implicate any Unrelated Information and provide further instructions to the Vendor as appropriate. If despite Ziopharm's good faith efforts to compile a comprehensive list in Schedule 2, Ziopharm later identifies additional relevant vendors and/or batch/lot numbers relating to the IL-12 Program prior to October 5, 2018, Precigen shall at Ziopharm's request prepare and send additional Vendor Authorization Letters within [***] of such request. In the event Ziopharm discovers that information it receives from a Vendor contains Unrelated Information, including but not limited to as a result of Precigen pointing out the existence of such information, Ziopharm shall immediately destroy all copies of such Unrelated Information that are in Ziopharm's possession or control and shall confirm to Precigen in writing that Ziopharm has destroyed all such copies. However, where a document contains both information relating to the IL-12 Program and Unrelated Information, Ziopharm shall be permitted to retain copies of the document provided that all Unrelated Information is redacted and inaccessible, and Ziopharm has confirmed to Precigen in writing that Ziopharm has redacted all Unrelated Information. Ziopharm shall reimburse Precigen for its out-of-pocket expenses and FTE costs incurred in relation to the Vendor Authorization Letters under this Section 4.1(e)(iii) as set forth in Section 4.2, except that the FTE rate shall be [***] per hour."

(iv) For the avoidance of doubt, any items provided to Ziopharm by a Vendor pursuant to this Amendment No. 1 shall be subject to the confidentiality provisions of the Agreement (including Article 10) as if the items were provided by Precigen directly.

3. **Future IL-12 Transfers and Deliveries**. A new Section 4.1(f) of the Agreement is hereby added to read as follows:

"**(f) Future IL-12 Transfers and Deliveries**. For future requests, other than requests expressly authorized pursuant to Section 4.1(e)(ii), for Precigen's provision or transfer to Ziopharm of Licensed Know-How relating to the IL-12 Program or any of Precigen's inventory of IL-12 Products (including all final product, drug substance, intermediates, works-in-process, formulation materials, reference standards, drug product clinical reserve samples, packaged retention samples, cell banks, viral banks, and the like):

(i) Ziopharm's first recourse for obtaining such items will be from the applicable Vendor through the respective Vendor Authorization Letter.

(ii) Ziopharm may request such items from Precigen only if (x) the applicable Vendor does not have or cannot provide the requested items to Ziopharm without further authorization from Precigen, (y) Ziopharm demonstrates a need for the requested items in connection with a request or requirement of a government regulator; and (z) Ziopharm represents that it cannot obtain those items without the participation of Precigen. In the event Ziopharm satisfies each of these requirements, Precigen will produce such items to Ziopharm provided that Precigen is in Control of and can reasonably locate the requested items. Precigen will produce any such items to Ziopharm within [***], except that Precigen may transfer and deliver such items to Ziopharm within [***] of Ziopharm's request provided that Precigen represents that production of the items within [***] would materially interfere with Precigen's (or the relevant individual or individuals') ongoing business or other material obligations and commitments. Ziopharm shall reimburse Precigen for its out-of-pocket expenses and FTE costs incurred to perform the transfer provided solely under this Section 4.1(f) as set forth in Section 4.2, except that the applicable FTE rate shall be [***] per hour.

(iii) Except as expressly set forth in Section 4.1(e) and 4.1(f), Precigen shall have no obligations with respect to the provision or transfer to Ziopharm of any Licensed Know-How relating to the IL-12 Program or any of Precigen's inventory of IL-12 Products. For the avoidance of doubt, any items requested by Ziopharm pursuant to Section 4.1(e) and 4.1(f) must have been in existence at the time the Agreement was signed. For the further avoidance of doubt, nothing herein shall require Precigen to disclose any confidential or proprietary information except insofar as such information relates to the IL-12 Program or IL-12 Products and otherwise meets the requirements of the Agreement as amended herein."

4. **Release**. In consideration for the Parties agreeing to the terms of this Amendment No. 1, (a) Ziopharm, on behalf of itself and its Affiliates, hereby releases and discharges Precigen and its respective subsidiaries, divisions, parents, Affiliates, agents and each of their respective officers, directors, employees, representatives and agents, and (b) Precigen, on behalf of itself and its Affiliates, hereby releases and discharges Ziopharm and its subsidiaries, divisions, parents,

Affiliates, agents and each of their respective officers, directors, employees, representatives and agents, in each foregoing case ((a) and (b)), from any and all Disputes existing as of the Effective Date of this Amendment No. 1 concerning the Parties' respective rights and obligations for the transfer or delivery of Licensed Know-How relating to the IL-12 Program or IL-12 Products as provided for under Sections 4.1, 4.3 and 4.5 of the Agreement.

5. **Amendment**. This Amendment No. 1 shall not amend or modify the covenants, terms, conditions, rights and obligations of the Parties under the Agreement, except as specifically set forth herein. The Agreement shall continue in full force and effect in accordance with its terms as amended by this Amendment No. 1.

* * *

IN WITNESS WHEREOF, the Parties have entered into this Amendment No. 1 in multiple counterparts as of the Effective Date of Amendment No. 1.

Ziopharm Oncology, Inc.

By: /s/ Laurence Cooper
Title: CEO

PGEN Therapeutics, Inc.

By: /s/ Donald Lehr
Title: Manager, Board of Managers

Schedule 1 to Amendment No. 1 – Specified Transfers and Deliveries

Item Number	Item	Location	Ziopharm Request
1	[***]	[***]	[***]
2	[***]	[***]	[***]
3	[***]	[***]	[***]
4	[***]	[***]	[***]
5	[***]	[***]	[***]
6	[***]	[***]	[***]
7	[***]	[***]	[***]
8	[***]	[***]	[***]
9	[***]	[***]	[***]
10	[***]	[***]	[***]
11	[***]	[***]	[***]
12	[***]	[***]	[***]
13	[***]	[***]	[***]
14	[***]	[***]	[***]
15	[***]	[***]	[***]
16	[***]	[***]	[***]
17	[***]	[***]	[***]

Schedule 2 to Amendment No. 1 – List of Vendors and Batch/Lot Numbers

Veledimex & Maisine Formulated Veledimex:

Vendor	Batches
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]

Ad-RTS-hIL12 and Ad-RTS-mIL12:

Vendor	Batches
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]
[***]	[***]

Schedule 3 to Amendment No. 1 – Model Vendor Authorization Letter

[Insert Date]

[Insert vendor name, address]

Re: Release of data, information and records to Ziopharm

Dear [Insert name],

[***]

Sincerely,

PGEN Therapeutics, Inc.

_____

[Name]

[Title]

CERTIFICATION

I, Laurence J.N. Cooper, certify that:

1. I have reviewed this quarterly report on Form 10-Q of ZIOPHARM Oncology, 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: November 5, 2020

/s/ Laurence J.N. Cooper
Laurence J.N. Cooper, M.D., Ph.D.
Chief Executive Officer
(Principal Executive Officer)

CERTIFICATION

I, Satyavrat Shukla, certify that:

1. I have reviewed this quarterly report on Form 10-Q of ZIOPHARM Oncology, 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: November 5, 2020

/s/ Satyavrat Shukla
Satyavrat Shukla
Executive Vice President and Chief Financial Officer
(Principal Financial Officer)

CERTIFICATION

In connection with the Quarterly Report on Form 10-Q of ZIOPHARM Oncology, Inc. (the "Company") for the quarter ended September 30, 2020, as filed with the Securities and Exchange Commission on the date hereof (the "Report"), we Laurence J.N. Cooper, the Principal Executive Officer of the Company and Satyavrat Shukla, the Principal Financial Officer of the Company, hereby each certifies, pursuant to 18 U.S.C. 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, that, to the best of his knowledge:

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

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

Dated: November 5, 2020

/s/ Laurence J.N. Cooper

Laurence J.N. Cooper, M.D., Ph.D.
Chief Executive Officer
(Principal Executive Officer)

/s/ Satyavrat Shukla

Satyavrat Shukla
Executive Vice President and Chief Financial Officer
(Principal Financial Officer)