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

Form 10-Q

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

For the quarterly period ended March 31, 2019

OR

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

For the transition period from to

Commission file number: 001-36829

Rocket Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**04-3475813**
(State or other jurisdiction of incorporation or organization)	**(I.R.S. Employer Identification No.)**

350 Fifth Avenue, Suite 7530
New York, NY 10118
(Address of principal executive office) (Zip Code)

Registrant's telephone number, including area code:
(646) 440-9100

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 (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

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

Large accelerated filer ☐ Accelerated filer ☒

Non-accelerated filer ☐ 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 ☒

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, $0.01 par value	RCKT	Nasdaq Global Market

As of May 6, 2019, there were 50,289,437 shares of common stock, $0.01 par value per share, outstanding.

PART I — FINANCIAL INFORMATION

Item 1. Consolidated Financial Statements

Rocket Pharmaceuticals, Inc.
Consolidated Balance Sheets
(in thousands, except share and per share amounts)

	March 31, 2019	December 31, 2018
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 124,464	$ 111,355
Investments	72,126	94,375
Prepaid expenses and other assets	3,029	3,358
Total current assets	199,619	209,088
Property and equipment, net	7,077	2,027
Goodwill	30,815	30,815
Restricted cash	1,525	1,436
Deposits	455	545
Operating lease right-of-use assets	2,647	-
Investments	-	7,402
Total assets	$ 242,138	$ 251,313
Liabilities and shareholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 19,680	$ 15,372
Operating lease liabilities, current	892	-
Total current liabilities	20,572	15,372
Convertible notes, net of unamortized discount	42,297	41,447
Operating lease liabilities, non-current	2,169	-
Other liabilities	23	457
Total liabilities	65,061	57,276
Commitments and contingencies (Note 11)		
Shareholders' equity:		
Preferred shares, $0.01 par value, authorized 1,000,000 shares:		
Series A convertible preferred shares; 300,000 shares designated as Series A; 0 shares issued and outstanding at March 31, 2019 and December 31, 2018	-	-
Series B convertible preferred shares; 300,000 shares designated as Series B; 0 shares issued and outstanding at March 31, 2019 and December 31, 2018	-	-
Common stock, $0.01 par value, 120,000,000 shares authorized; 45,114,437 and 45,194,736 shares issued and outstanding at March 31, 2019 and December 31, 2018, respectively	451	452
Treasury stock, at cost, 0 and 50,000 common shares at March 31, 2019 and December 31, 2018, respectively	-	(668)
Additional paid-in capital	302,039	300,253
Accumulated other comprehensive loss	(89)	(127)
Accumulated deficit	(125,324)	(105,873)
Total shareholders' equity	177,077	194,037
Total liabilities and shareholders' equity	$ 242,138	$ 251,313

The accompanying notes are an integral part of these consolidated financial statements.

Rocket Pharmaceuticals, Inc.
Consolidated Statements of Operations
(in thousands, except share and per share amounts)
(unaudited)

	Three Months Ended March 31, 2019	2018
Revenue	$ -	$ -
Operating expenses:		
Research and development	15,137	5,743
General and administrative	3,808	8,662
Total operating expenses	18,945	14,405
Loss from operations	(18,945)	(14,405)
Research and development incentives	250	186
Interest expense	(1,604)	(1,427)
Interest and other income net	601	288
Accretion of discount on investments	247	15
Net loss	$ (19,451)	$ (15,343)
Net loss per share attributable to common shareholders - basic and diluted	$ (0.43)	$ (0.42)
Weighted-average common shares outstanding - basic and diluted	45,122,815	36,137,120

The accompanying notes are an integral part of these consolidated financial statements.

Rocket Pharmaceuticals, Inc.
Consolidated Statements of Comprehensive Loss
(in thousands)
(unaudited)

	Three Months Ended March 31,	
	2019	2018
Net loss	$ (19,451)	$ (15,343)
Other comprehensive loss		
Net unrealized gain on investments	38	10
Total comprehensive loss	$ (19,413)	$ (15,333)

The accompanying notes are an integral part of these consolidated financial statements.

Rocket Pharmaceuticals, Inc.
Consolidated Statement of Shareholders' Equity
For the Three Months Ended March 31, 2019 and 2018
(in thousands except share amounts)
(unaudited)

	Common Stock		Treasury Stock	Additional Paid-In Capital	Accumulated Other Comprehensive Loss	Accumulated Deficit	Total Shareholders' Equity
	Shares	Amount					
Balance at December 31, 2018	45,194,736	$ 452	$ (668)	$ 300,253	$ (127)	$ (105,873)	$ 194,037
Issuance of common stock pursuant to exercise of stock options	19,701	-	-	-	-	-	-
Share repurchase	-	-	(725)	(2)	-	-	(727)
Retirement of treasury stock	(100,000)	(1)	1,393	(1,392)	-	-	-
Unrealized comprehensive gain on marketable securities	-	-	-	-	38	-	38
Share-based compensation	-	-	-	3,180	-	-	3,180
Net loss	-	-	-	-	-	(19,451)	(19,451)
Balance at March 31, 2019	45,114,437	$ 451	-	$ 302,039	$ (89)	$ (125,324)	$ 177,077

	Series A Convertible Preferred Shares		Series B Convertible Preferred Shares		Common Stock		Additional Paid-In Capital	Accumulated Deficit	Accumulated Other Comprehensive Income	Total Shareholders' Equity
	Shares	Amount	Shares	Amount	Shares	Amount				
Balance at December 31, 2017	128,738	$ 16,060	126,909	$ 25,406	6,795,627	$ 68	$ 5,340	$ (31,355)	$ -	$ 15,519
Conversion of convertible preferred shares into common shares	(128,738)	(16,060)	(126,909)	(25,406)	19,475,788	194	41,272	-	-	-
Exchange of common shares in connection with the Merger	-	-	-	-	6,805,608	68	85,992	-	-	86,060
Issuance of common shares, net of issuance costs of $5.3 million	-	-	-	-	6,325,000	63	78,455	-	-	78,518
Issuance of common shares pursuant to settlement of restricted stock units	-	-	-	-	1,875	1	(1)	-	-	-
Unrealized gain on short term investments	-	-	-	-	-	-	-	-	10	10
Share-based compensation	-	-	-	-	-	-	5,382	-	-	5,382
Net loss	-	-	-	-	-	-	-	(15,343)	-	(15,343)
Balance at March 31, 2018	-	$ -	-	$ -	39,403,898	$ 394	$ 216,440	$ (46,698)	$ 10	170,146

The accompanying notes are an integral part of these consolidated financial statements.

Rocket Pharmaceuticals, Inc.
Consolidated Statements of Cash Flows
(in thousands)
(unaudited)

	Three Months Ended March 31,	
	2019	**2018**
Operating Activities:		
Net loss	$ (19,451)	$ (15,343)
Adjustments to reconcile net loss to net cash used in operating activities:		
Accretion of discount on convertible notes	850	696
Amortization of operating lease right-of-use assets	190	(20)
Depreciation expense	102	83
Share-based compensation expense	3,180	5,382
Amortization of premium on short term investments	-	12
Accretion of discount on investments	(247)	-
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(307)	(266)
Accounts payable and accrued expenses	(84)	(2,145)
Operating lease liabilities	(210)	-
Net cash used in operating activities	(15,977)	(11,601)
Investing activities:		
Cash acquired in connection with the Reverse Merger	-	76,348
Proceeds from maturities of investments	29,935	9,718
Proceeds from sale of property and equipment	-	20
Purchases of property and equipment	(760)	(8)
Net cash provided by investing activities	29,175	86,078
Financing activities:		
Proceeds from issuance of common stock, net of issuance costs	-	78,518
Net cash provided by financing activities	-	78,518
Net change in cash, cash equivalents and restricted cash	13,198	152,995
Cash, cash equivalents and restricted cash at beginning of period	112,791	18,349
Cash, cash equivalents and restricted cash at end of period	$ 125,989	$ 171,344
Supplemental disclosure of non-cash financing and investing activities:		
Accrued purchases of property and equipment	$ 4,392	$ -
Retirement of treasury stock	$ 1,395	$ -
Conversion of convertible preferred stock into common stock	$ -	$ 41,466
Unrealized gain on investments	$ 38	$ -
Supplemental cash flow information:		
Cash paid for interest	$ 1,495	$ 1,495

The accompanying notes are an integral part of these consolidated financial statements.

ROCKET PHARMACEUTICALS, INC.
Notes to Consolidated Financial Statements
(Amounts in thousands, except share and per share data)
(Unaudited)

1. Nature of Business

Rocket Pharmaceuticals, Inc., together with its subsidiaries (collectively, "Rocket" or the "Company"), is a clinical-stage, multi-platform biotechnology company focused on the development of first or best-in-class gene therapies, with direct on-target mechanism of action and clear clinical endpoints, for rare and devastating pediatric diseases. The Company has clinical-stage lentiviral vector ("LVV") programs currently undergoing clinical testing for Fanconi Anemia ("FA"), a genetic defect in the bone marrow that reduces production of blood cells or promotes the production of faulty blood cells and Leukocyte Adhesion Deficiency-I ("LAD-I"), a genetic disorder that causes the immune system to malfunction. FA has been in clinical stage testing in the European Union ("EU") since 2016, and in the United States ("U.S."), Rocket received investigational new drug ("IND") clearance for both FA and LAD-I in late 2018. Two additional pre-clinical stage LVV programs include Pyruvate Kinase Deficiency ("PKD"), a rare red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia; and Infantile Malignant Osteopetrosis ("IMO"), a genetic disorder characterized by increased bone density and bone mass secondary to impaired bone resorption. In addition, the Company has an adeno-associated virus ("AAV"), program for Danon disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. An IND filing was cleared in Danon disease in early 2019 and human clinical studies are anticipated to commence in the second quarter of 2019. The Company has global commercialization and development rights to all of its product candidates under royalty-bearing license agreements, with the exception of the CRISPR/Cas9 development program for which the Company currently only has development rights.

2. Risks and Liquidity

The Company has not generated any revenue and has incurred losses since inception. The Company's operations are subject to certain risks and uncertainties, including, among others, uncertainty of drug candidate development, technological uncertainty, uncertainty regarding patents and proprietary rights, lack of commercial manufacturing experience, a lack of marketing or sales capability or experience, dependency on key personnel, compliance with government regulations and the need to obtain additional financing. Drug candidates currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure and extensive compliance-reporting capabilities.

The Company's drug candidates are in the development and clinical stage. There can be no assurance that the Company's research and development will be successfully completed, that adequate protection for the Company's intellectual property will be obtained, that any products developed will obtain necessary government approval or that any approved products will be commercially viable. Even if the Company's product development efforts are successful, it is uncertain when, if ever, the Company will generate significant revenue from product sales. The Company operates in an environment of rapid change in technology and substantial competition from pharmaceutical and biotechnology companies.

The Company's consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities in the ordinary course of business. The Company has experienced negative cash flows from operations and had an accumulated deficit of $125.3 million as of March 31, 2019. As of March 31, 2019, the Company has $196.6 million of cash, cash equivalents and investments.

On April 18, 2019, the Company completed a public offering of 5,175,000 shares of common stock, for net proceeds of $86.0 million. See Note 16 "Subsequent Events" for additional disclosures regarding the public offering. Considering the proceeds from the public offering, Rocket expects such resources would be sufficient to fund its operating expenses and capital expenditure requirements into the first half of 2021.

In the longer term, the future viability of the Company is dependent on its ability to generate cash from operating activities or to raise additional capital to finance its operations. The Company's failure to raise capital as and when needed could have a negative impact on its financial condition and ability to pursue its business strategies.

3. Basis of Presentation, Principles of Consolidation and Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited interim consolidated financial statements should be read in conjunction with the Company's consolidated financial statements for the year ended December 31, 2018 included in the Annual Report on Form 10-K filed with the Securities and Exchange Commission ("SEC") on March 8, 2019. The unaudited interim consolidated financial statements have been prepared on the same basis as the audited annual financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for the fair statement of the Company's financial position as of March 31, 2019 and the results of its operations and its cash flows for the three months ended March 31, 2019 and 2018. The financial data and other information disclosed in these consolidated notes related to the three months ended March 31, 2019 and 2018 are unaudited. The results for the three months ended March 31, 2019 are not necessarily indicative of results to be expected for the year ending December 31, 2019 and any other interim periods or any future year or period.

Principles of Consolidation

The consolidated financial statements represent the consolidation of the accounts of the Company and its subsidiaries in conformity with accounting principles generally accepted in the U.S ("US GAAP"). All intercompany accounts have been eliminated in consolidation.

Use of Estimates

The preparation of the consolidated financial statements in conformity with US GAAP requires management to make estimates and assumptions that affect the reported amounts of assets and liabilities and disclosure of contingent assets and liabilities at the date of the financial statements and the reported amounts of expenses during the reporting period. Significant estimates and assumptions reflected in these consolidated financial statements include but are not limited to goodwill impairment, the accrual of research and development expenses, the valuation of equity transactions and share-based awards. Changes in estimates and assumptions are reflected in reported results in the period in which they become known. Actual results could differ from those estimates.

Significant Accounting Policies

The significant accounting policies used in the preparation of these consolidated financial statements for the three months ended March 31, 2019 are consistent with those disclosed in Note 3 to the consolidated financial statements in the Company's Annual Report on Form 10-K for the year ended December 31, 2018, except as noted below.

Cash, Cash Equivalents and Restricted Cash

Cash, cash equivalents and restricted cash consists of bank deposits, certificates of deposit and money market accounts with financial institutions. Cash equivalents are carried at cost which approximates fair value due to their short-term nature and which the Company believes do not have a material exposure to credit risk. The Company considers all highly liquid investments with maturities of three months or less from the date of purchase to be cash equivalents. The Company's cash and cash equivalent accounts, at times, may exceed federally insured limits. The Company has not experienced any losses in such accounts.

Restricted cash consists of deposits collateralizing letters of credit issued by a bank in connection with the Company's operating leases (See Note 11) and a deposit collateralizing a letter of credit issued by a bank supporting the Company's Corporate Credit Card. Cash, cash equivalents and restricted cash consist of the following:

	March 31, 2019	December 31, 2018
Cash and cash equivalents	$ 124,464	$ 111,355
Restricted cash	1,525	1,436
	$ 125,989	$ 112,791

Leases

The Company adopted ASU 2016-02, *Leases ("ASU 2016-02")*, as amended on January 1, 2019, which supersedes the current leasing guidance and upon adoption, requires lessees to recognize right-of-use assets and lease liabilities on the balance sheet for all leases with terms longer than 12 months. Leases are classified as either finance or operating, with classification affecting the pattern of expense recognition in the income statement. Upon the adoption of the guidance, operating leases are capitalized on the balance sheet at the present value of lease payments. The balance sheet amount recorded for existing leases at the date of adoption of ASU 2016-02 was calculated using the applicable incremental borrowing rate at the date of adoption.

The Company adopted ASU 2016-02, including several practical expedients on January 1, 2019. The Company elected the available package of practical expedients which allows the Company to not reassess previous accounting conclusions around whether arrangements are or contain leases, the classification of leases, and the treatment of initial direct costs. The Company also made an accounting policy election to utilize the short-term lease exemption, whereby leases with a term of 12 months or less will not follow the recognition and measurement requirements of the new standard. Upon adoption, the Company recognized total right-of-use assets of $2.6 million, with corresponding liabilities of $3.1 million on the consolidated balance sheets, including the reclassification of $0.5 million from deferred rent to right-of-use assets.

See Note 11 "Commitments and Contingencies" for additional disclosures in accordance with the new lease standard.

4. Fair Value of Financial Instruments

Items measured at fair value on a recurring basis are the Company's investments. The following table sets forth the Company's financial instruments that were measured at fair value on a recurring basis by level within the fair value hierarchy:

	Fair Value Measurements as of March 31, 2019 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Money market mutual funds (included in cash and cash equivalents)	$ 61,125	$ -	$ -	$ 61,125
United States Treasury securities	72,126	-	-	72,126
Investments	72,126	-	-	72,126
	$ 133,251	$ -	$ -	$ 133,251

	Fair Value Measurements as of December 31, 2018 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Money market mutual funds (included in cash and cash equivalents)	$ 30,552	$ -	$ -	$ 30,552
United States Treasury securities	101,777	-	-	101,777
Investments	101,777	-	-	101,777
	$ 132,329	$ -	$ -	$ 132,329

The Company classifies its money market mutual funds and U.S. Treasury securities as Level 1 assets under the fair value hierarchy, as these assets have been valued using quoted market prices in active markets without any valuation adjustment.

5. Property and Equipment

The Company's property and equipment consisted of the following:

	March 31, 2019	December 31, 2018
Laboratory equipment	$ 1,556	$ 1,556
Leasehold improvements	282	29
Furniture and fixtures	273	273
Computer equipment	179	179
Construction in progress	5,368	469
	7,658	2,506
Less: accumulated depreciation	(581)	(479)
	$ 7,077	$ 2,027

Construction in progress comprises costs associated with the one of the facilities under an operating lease. See Note 11. During the three months ended March 31, 2019 and 2018, the Company recognized $102 and $83 of depreciation expense, respectively.

6. Accounts Payable and Accrued Expenses

At March 31, 2019 and December 31, 2018, the Company's accounts payable and accrued expenses consisted of the following:

	March 31, 2019	December 31, 2018
Research and development	$ 12,573	$ 10,414
Construction in progress	4,475	-
Government grant payable	541	534
Professional fees	536	690
Accrued interest	493	1,241
Bonus	471	1,774
Other	374	589
Accrued vacation	217	123
Severance and benefits	-	7
	$ 19,680	$ 15,372

7. Debt

On January 4, 2018, in connection with the Reverse Merger, the Company assumed the obligations of Inotek Pharmaceuticals Corporation ("Inotek") under its outstanding convertible notes, with an aggregate principal value of $52.0 million, (the "2021 Convertible Notes"). The 2021 Convertible Notes were issued in 2016 and mature on August 1, 2021 (the "Maturity Date"). The 2021 Convertible Notes are unsecured, and accrue interest at a rate of 5.75% per annum, interest is payable semi-annually on February 1 and August 1 of each year. Each holder of the 2021 Convertible Notes ("Holder") has the option until the close of business on the second business day immediately preceding the Maturity Date to convert all, or any portion, of the 2021 Convertible Notes held by it at a conversion rate of 31.1876 shares of the Company's common stock per $1.00 principal amount of 2021 Convertible Notes (the "Conversion Rate") which is $32.08 per share. The Conversion Rate is subject to adjustment from time to time upon the occurrence of certain events, including the issuance of stock dividends and payment of cash dividends.

The Company, at its option, may redeem for cash all or any portion of the 2021 Convertible Notes if the last reported sale price of a share of the Company's common stock is equal to or greater than 200% of the conversion price for the 2021 Convertible Notes then in effect for at least 20 trading days (whether or not consecutive) during any 30 consecutive trading day period (including the last trading day of such period) ending within the five trading days immediately preceding the date on which the Company provides notice of redemption, at a redemption price equal to 100% of the principal amount of the 2021 Convertible Notes to be redeemed, plus accrued and unpaid interest to, but excluding, the redemption date.

The 2021 Convertible Notes are considered a hybrid financial instrument consisting of a fixed interest rate "host" and various embedded features that required evaluation as potential embedded derivatives under FASB ASC 815, *Derivatives and Hedging* ("ASC 815"). Based on the nature of the host instrument and the embedded features, management concluded that none of the conversion, put and redemption features required bifurcation and separate accounting from the host instrument. The Company determined that the Additional Interest was an embedded derivative that contains non-credit related events of default. As a result, the Additional Interest feature required bifurcation and separate accounting under ASC 815. Based on the amount of Additional Interest that would be owed and the likelihood of occurrence, Rocket estimated the fair value of the Additional Interest feature to be insignificant upon issuance and as of March 31, 2019 and December 31, 2018.

The Company recorded the 2021 Convertible Notes at their fair value of $38,388 on January 4, 2018, the date of the Reverse-Merger. The difference between the fair value of the 2021 Convertible Notes and the principal value represents a discount on the notes that is being accreted to interest expense over the remaining term using the effective interest method. As of March 31, 2019, the stated interest rate was 5.75%, and the effective interest rate was 15.3%.

The table below summarizes the carrying value of the 2021 Convertible Notes as of March 31, 2019:

Principal amount	$ 52,000
Discount	(9,703)
Carrying value as of March 31, 2019	$ 42,297

Accretion of the 2021 Convertible Notes discount was $0.9 million and $0.7 million for the three months ended March 31, 2019 and 2018, respectively.

8. Share Based Compensation

Share Option Valuation

The weighted average assumptions that the Company used in the Black-Scholes pricing model to determine the fair value of the share options granted to employees, non-employees and directors for the three months ended March 31, 2019 and the share options granted to employees and directors for the three months ended March 31, 2018 were as follows:

	Three Months Ended March 31,	
	2019	2018
Risk-free interest rate	2.61%	2.57%
Expected term (in years)	5.77	5.76
Expected volatility	74.60%	88.60%
Expected dividend yield	0.00%	0.00%
Exercise price	$ 14.61	$ 17.52
Fair value of common stock	$ 14.61	$ 17.52

The weighted average assumptions that the Company used in the Black-Scholes pricing model to determine the fair value of the share options granted to non-employees for the three months ended March 31, 2018 were as follows:

	Three Months Ended March 31, 2019
Risk-free interest rate	2.74%
Expected term (in years)	10.00
Expected volatility	83.79%
Expected dividend yield	0.00%
Exercise price	$ 18.75
Fair value of common stock	$ 18.75

The Company recognizes compensation expense for only the portion of awards that are expected to vest.

The following table summarizes stock option activity for the three months ended March 31, 2019 under the Second Amended and Restated 2014 Stock Option and Incentive Plan:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Contractual Term (Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2018	8,615,997	$ 4.48	7.51	$ 94,474
Granted	1,023,366	14.61	9.79	
Exercised	(19,701)	1.92		801
Forfeited	(226,841)	8.67		
Outstanding as of March 31, 2019	9,392,821	$ 5.56	7.55	$ 114,173
Options vested and exercisable as of March 31, 2019	6,777,244	$ 2.17	6.92	$ 104,547
Options unvested as of March 31, 2019	2,617,828	$ 14.35	9.20	$ 9,626

The aggregate intrinsic value of stock options is calculated as the difference between the exercise price of the stock options and the fair value of the Company's common stock for those stock options that had exercise prices lower than the fair value of the Company's common stock. The intrinsic value of options exercised and exercisable as of March 31, 2019 and 2018 was $114 and $130.

The weighted average grant-date fair value per share of stock options granted during the three months ended March 31, 2019 and 2018 was $9.60 and $12.84, respectively.

The total fair value of options vested during the three months ended March 31, 2019 and 2018 was $20,493 and $24,724, respectively.

Share-Based Compensation

	Three Months Ended March 31, 2019	2018
Research and development	$ 1,565	$ 2,207
General and administrative	1,615	3,175
Total share based compensation expense	$ 3,180	$ 5,382

As of March 31, 2019, the Company had an aggregate of $24.9 million of unrecognized share-based compensation cost, which is expected to be recognized over the weighted average period of 2.07 years.

9. Shareholders' Equity

On December 27 and 28, 2018, the Company repurchased 100,000 shares of its common stock for aggregate consideration of approximately $1.4 million. The repurchases were made on the Nasdaq Stock Market at prevailing market prices in accordance with SEC Rule 10b-18. 50,000 of the shares repurchased at an average price of $13.36 by the Company settled on December 31, 2018 and the remaining 50,000 shares repurchased at an average price of $14.50 settled on January 2, 2019. As of December 31, 2018, the Company recorded a prepaid expense of $0.7 million related to the 50,000 shares that settled on January 2, 2019 and recorded treasury stock of $0.7 million relating to the 50,000 shares that settled as of December 31, 2018. These shares were subsequently retired in January 2019.

The Company has 14,102 warrants outstanding as of March 31, 2019, convertible into 14,102 shares of common shares at an exercise price of $24.82 per share which expire on June 28, 2023.

10. Net Loss Per Share

Basic and diluted net loss per share attributable to common shareholders was calculated as follows:

	Three Months Ended March 31, 2019	2018
Numerator:		
Net loss	$ (19,451)	$ (15,343)
Denominator:		
Weighted-average common shares outstanding - basic and diluted	45,122,815	36,137,120
Net loss per share- basic and diluted	$ (0.43)	$ (0.42)

The Company excluded the following potential common shares, presented based on amounts outstanding at each period end, from the computation of diluted net loss per share attributable to common shareholders for the periods indicated because including them would have had an anti-dilutive effect:

	Three Months Ended March 31, 2019	2018
Shares issuable upon conversion of the 2021 Convertible Notes	1,620,848	1,620,948
Warrants exercisable for common shares	14,102	14,102
Options to purchase common shares	9,392,821	8,635,089
	11,027,771	10,270,139

11. Commitments and Contingencies

Operating Leases

On August 14, 2018, Rocket entered into a lease for approximately 92,000 rentable square feet in Cranbury, New Jersey, for office space, process development, research activities and manufacturing to support the Company's pipeline (the "NJ Lease Agreement"). The term of the NJ Lease Agreement will commence for 72,000 rentable square feet upon substantial completion of leasehold improvements (the "Commencement Date"), and the remaining 20,000 square feet will commence upon the earlier of the Company's election to commence the lease of such additional space or thirty months from the Commencement Date. The NJ Lease Agreement has a term of fifteen years from the Commencement Date, with an option to renew for two consecutive five-year renewal terms. Estimated rent payments are $1.2 million per annum, payable in monthly installments, depending upon the nature of the leased space, and subject to annual base rent increases of 3%. The total commitment under the lease is estimated to be approximately $26.5 million over the 15 year term of the lease. The Company delivered a cash security deposit of $0.3 million to the landlord in connection with the NJ Lease Agreement which has been reflected in deposits in the consolidated balance sheets. The Company entered into the lease prior to the building being available for use as the building construction was not complete. The Company has determined it does not control the leased asset prior to the commencement date, but is involved with the design and construction of the space in selecting building designs, general contractors, and funding certain construction costs.

The total restricted cash balance for the Company's operating leases at March 31, 2019 and December 31, 2018 was $1.0 million and $1.4 million, respectively.

The Company determines if an arrangement is a lease at inception. Operating leases are included in our balance sheet as right-of-use assets from operating leases, current operating lease liabilities and long-term operating lease liabilities. Certain of the Company's lease agreements contain renewal options; however, the Company does not recognize right-of-use assets or lease liabilities for renewal periods unless it is determined that the Company is reasonably certain of renewing the lease at inception or when a triggering event occurs. As the Company's leases do not provide an implicit rate, the Company estimated the incremental borrowing rate in calculating the present value of the lease payments. The Company has utilized its incremental borrowing rate based on the long -term borrowing costs of comparable companies in the biotechnology industry. Since the Company elected to account for each lease component and its associated non-lease components as a single combined lease component, all contract consideration was allocated to the combined lease component. Some of the Company's lease agreements contain rent escalation clauses (including index-based escalations). The Company recognizes the minimum rental expense on a straight-line basis based on the fixed components of a lease arrangement. The Company amortizes this expense over the term of the lease beginning with the date of initial possession, which is the date the Company can enter the leased space and begin to make improvements in preparation for its intended use. Variable lease components represent amounts that are not fixed in nature and are not tied to an index or rate, and are recognized as incurred.

Lease cost	**March 31, 2019**
Operating lease cost	$ 251
Total lease cost	$ 251

The following table summarizes the maturity of the Company's lease liabilities on an undiscounted cash flow basis and a reconciliation to the operating lease liabilities recognized on our balance sheet as of March 31, 2019:

Maturity of lease liabilities	**March 31, 2019**
2019 (remaining nine months)	$ 818
2020	1,103
2021	894
2022	572
2023	74
Total lease payments	$ 3,460
Less: interest	(399)
Total operating lease liabilities	$ 3,061

The following disclosure is provided for periods prior to adoption of ASU 2016-02. Future annual minimum lease payment commitments as of March 31, 2019 were as follows:

2019 (remaining nine months)	$ 1,188
2020	1,970
2021	1,898
2022	1,757
2023	1,618
Thereafter	20,144
Total	$ 28,575

Leases	March 31, 2019
Operating right-of-use assets	$ 2,647
Operating current lease liabilities	892
Operating noncurrent lease liabilities	2,169
Total operating lease liabilities	$ 3,061

Other information

Cash paid for amounts included in the measurement of lease liabilities:	
Operating cash flows from operating leases	270
Weighted-average remaining lease term - operating leases	3.3 years
Weighted-average discount rate - operating leases	7.77%

Rent expense was $0.2 million and $0.1 million for the three months ended March 31, 2019 and 2018, respectively.

Litigation

From time to time, the Company may be subject to other various legal proceedings and claims that arise in the ordinary course of its business activities. Although the results of litigation and claims cannot be predicted with certainty, the Company does not believe it is party to any other claim or litigation the outcome of which, if determined adversely to the Company, would individually or in the aggregate be reasonably expected to have a material adverse effect on its business. Regardless of the outcome, litigation can have an adverse impact on the Company because of defense and settlement costs, diversion of management resources and other factors.

Indemnification Arrangements

Pursuant to its bylaws and as permitted under Delaware law, the Company has indemnification obligations to directors, officers, employees or agents of the Company or anyone serving in these capacities. The maximum potential amount of future payments the Company could be required to pay is unlimited. The Company has insurance that reduces its monetary exposure and would enable it to recover a portion of any future amounts paid. As a result, the Company believes that the estimated fair value of these indemnification commitments is minimal.

Throughout the normal course of business, the Company has agreements with vendors that provide goods and services required by the Company to run its business. In some instances, vendor agreements include language that requires the Company to indemnify the vendor from certain damages caused by the Company's use of the vendor's goods and/or services. The Company has insurance that would allow it to recover a portion of any future amounts that could arise from these indemnifications. As a result, the Company believes that the estimated fair value of these indemnification commitments is minimal.

12. Agreements Related to Intellectual Property

The Company has various license and research and collaboration arrangements. The transactions principally resulted in the acquisition of rights to intellectual property which is in the preclinical phase and has not been tested for safety or feasibility. In all cases, the Company did not acquire tangible assets, processes, protocols or operating systems. The Company expenses the acquired intellectual property rights as of the acquisition date on the basis that the cost of intangible assets purchased from others for use in research and development activities, has no alternative future uses.

13. Strategic Research Collaboration

On May 16, 2018, Rocket and the Stanford University School of Medicine ("Stanford University") entered into a strategic collaboration agreement to support the advancement of FA and PKD gene therapy research. Under the terms of the collaboration agreement, Stanford University will serve as the lead clinical trial research center in the U.S. for the planned FA registrational trial and would also be the lead U.S. site for PKD clinical trials. The project will also separately evaluate the potential for non-myeloablative, non-genotoxic antibody-based conditioning regimens as a future development possibility that may be applied across bone marrow-derived disorders. In addition, Rocket agreed to support expansion of Stanford University's Laboratory for Cell and Gene Therapy ("LCGM") in order to further enhance the development of Rocket's internal pipeline. Rocket agreed to contribute up to $3.5 million for the LCGM expansion of which 40% or $1.4 million was due upon execution of the collaboration agreement and the remaining $2.1 million balance is due upon the achievement of certain milestones. In January 2019, the Company and Stanford University signed a Clinical Trial Agreement for the treatment of FA. Upon the signing of the Clinical Trial Agreement, the second milestone of $1.4 million for the LGCM became due and was accrued and expensed in January 2019, when the milestone was met, and paid in April 2019. During the three months ended March 31, 2019, none of the remaining milestones were met with regard to the LCGM.

14. Related Party Transactions

During March 2018, the Company entered into a consulting agreement with a member of the Board of Directors for strategic and corporate consulting services to be provided to the Company. The Company incurred expenses of $4 and $30 during the three months ended March 31, 2019 and 2018, respectively relating to services provided under this consulting agreement.

During April 2018, the Company entered into a consulting agreement with a member of the Board of Directors for business development consulting services. Payments for the services under the agreement are $28 per quarter, and the Company may terminate the agreement with 14 days' notice. The Company incurred expenses of $28 and $0 during the three months ended March 31, 2019 and 2018, relating to services provided under this consulting agreement.

15. 401(k) Savings Plan

The Company has a defined contribution savings plan (the "Plan") under Section 401(k) of the Internal Revenue Code of 1986. This Plan covers substantially all employees who meet minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre-tax basis. Company contributions to the Plan may be made at the discretion of the Company's board of directors. The Company has elected to match 4% of employee contributions to the Plan, subject to certain limitations. The Company's matching contribution for the three months ended March 31, 2019 and 2018 was $81 and $30, respectively.

16. Subsequent Events

On April 18, 2019, the Company completed a public offering of 5,175,000 shares of common stock, which includes the full exercise of the underwriters' option to purchase 675,000 additional shares of its common stock, at a public offering price of $17.50 per share. The gross proceeds to Rocket from the public offering were $90.6 million, less $4.6 million of offering costs, commissions, legal and other expenses for net proceeds from the offering of $86.0 million.

On April 30, 2019, the Company announced the California Institute for Regenerative Medicine ("CIRM") has awarded Rocket a $6.5 million CLIN2 grant award to support the clinical development of gene therapy for LAD-I. Rocket's IND application for RP-L201 was accepted by the U.S. Food and Drug Administration in November 2018. Proceeds from the grant will help fund clinical trial costs as well as manufactured drug product for Phase I/II patients enrolled in the U.S. clinical site, University of California, Los Angeles Mattel Children's Hospital, led by PI Donald Kohn, M.D., UCLA Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics (Hematology/Oncology), Molecular and Medical Pharmacology and member of the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at UCLA.

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

The information set forth below should be read in conjunction with the consolidated financial statements and the notes thereto included elsewhere in this Quarterly Report on Form 10-Q as well as the audited financial statements and the notes thereto contained in our Annual Report on Form 10-K filed with the Securities and Exchange Commission (the "SEC") on March 8, 2019. Unless stated otherwise, references in this Quarterly Report on Form 10-Q to "us," "we," "our," or our "Company" and similar terms refer to Rocket Pharmaceuticals, Inc. References to "Inotek" refer to the company prior to the Reverse Merger.

Recent Developments

Rocket Pharmaceuticals, Inc., together with its subsidiaries (collectively, "Rocket" or the "Company"), is a clinical-stage, multi-platform biotechnology company focused on the development of first or best-in-class gene therapies, with direct on-target mechanism of action and clear clinical endpoints, for rare and devastating pediatric diseases. We have clinical-stage lentiviral vector ("LVV") programs currently undergoing clinical testing for Fanconi Anemia ("FA"), a genetic defect in the bone marrow that reduces production of blood cells or promotes the production of faulty blood cells and Leukocyte Adhesion Deficiency-I ("LAD-I"), a genetic disorder that causes the immune system to malfunction. FA has been in clinical stage testing in the European Union ("EU") since 2016, and in the United States ("U.S."), Rocket received investigational new drug ("IND") clearance for both FA and LAD-I in late 2018. Two additional pre-clinical stage LVV programs include Pyruvate Kinase Deficiency ("PKD"), a rare red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia; and Infantile Malignant Osteopetrosis ("IMO"), a genetic disorder characterized by increased bone density and bone mass secondary to impaired bone resorption. In addition, we have an adeno-associated virus ("AAV") program for Danon disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. An IND filing was cleared in Danon disease in early 2019, and human clinical studies will begin in the second quarter of 2019. We have global commercialization and development rights to all of our product candidates under royalty-bearing license agreements, with the exception of the CRISPR/Cas9 development program (described below) for which we currently only have development rights.

On April 18, 2019, we announced the closing of a public offering of 5,175,000 shares of common stock, which includes the full exercise of the underwriters' option to purchase 675,000 additional shares of our common stock, at a public offering price of $17.50 per share. The gross proceeds to Rocket from the public offering were approximately $90.6 million, less $4.6 million of offering costs, commissions, legal and other expenses for a net proceeds from the offering of $86.0 million. SVB Leerink LLC, Evercore Group L.L.C. and William Blair & Company, L.L.C., acted as joint book-running managers for the offering. Oppenheimer & Co. acted as lead manager for the offering. Pursuant to the underwriting agreement executed in connection with the offering, the Company's executive officers and directors, and certain other shareholders entered into agreements providing for a 90-day "lock-up" period with respect to sales of the Company's common stock, subject to certain exceptions.

On April 30, 2019, the Company announced the California Institute for Regenerative Medicine ("CIRM") has awarded Rocket a $6.5 million CLIN2 grant award to support the clinical development of gene therapy for LAD-I. Rocket's IND for RP-L201 was accepted by the U.S. Food and Drug Administration in November 2018. Proceeds from the grant will help fund clinical trial costs as well as manufactured drug product for Phase I/II patients enrolled in the U.S. clinical site, University of California, Los Angeles Mattel Children's Hospital, led by PI Donald Kohn, M.D., UCLA Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics (Hematology/Oncology), Molecular and Medical Pharmacology and member of the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at UCLA. The trial will evaluate the safety and efficacy of the infusion of autologous hematopoietic stem cells transduced with a lentiviral vector encoding the *ITGB2* gene.

Gene Therapy Overview

Genes are composed of sequences of deoxyribonucleic acid ("DNA"), which code for proteins that perform a broad range of physiologic functions in all living organisms. Although genes are passed on from generation to generation, genetic changes, also known as mutations, can occur in this process. These changes can result in the lack of production of proteins or the production of altered proteins with reduced or abnormal function, which can in turn result in disease.

Gene therapy is a therapeutic approach in which an isolated gene sequence or segment of DNA is administered to a patient, most commonly for the purpose of treating a genetic disease that is caused by genetic mutations. Currently available therapies for many genetic diseases focus on administration of large proteins or enzymes and typically address only the symptoms of the disease. Gene therapy aims to address the disease-causing effects of absent or dysfunctional genes by delivering functional copies of the gene sequence directly into the patient's cells, offering the potential for curing the genetic disease, rather than simply addressing symptoms.

We are using modified non-pathogenic viruses for the development of our gene therapy treatments. Viruses are particularly well suited as delivery vehicles because they are adept at penetrating cells and delivering genetic material inside a cell. In creating our viral delivery vehicles, the viral (pathogenic) genes are removed and are replaced with a functional form of the missing or mutant gene that is the cause of the patient's genetic disease. The functional form of a missing or mutant gene is called a therapeutic gene, or the "transgene." The process of inserting the transgene is called "transduction." Once a virus is modified by replacement of the viral genes with a transgene, the modified virus is called a "viral vector." The viral vector delivers the transgene into the targeted tissue or organ (such as the cells inside a patient's bone marrow). We have two types of viral vectors in development, LVV and AAV. We believe that our LVV and AAV-based programs have the potential to offer a significant therapeutic benefit to patients that is durable (long-lasting).

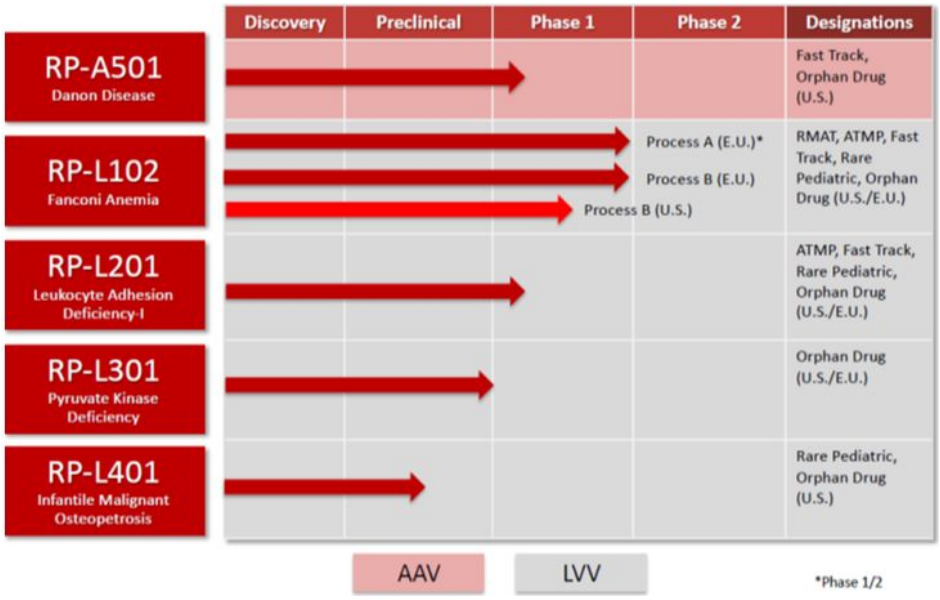
The gene therapies can be delivered either (1) *ex vivo* (outside the body), in which case the patient's cells are extracted and the vector is delivered to these cells in a controlled, safe laboratory setting, with the modified cells then being reinserted into the patient, or (2) *in vivo* (inside the body), in which case the vector is injected directly into the patient, either intravenously ("IV") or directly into a specific tissue at a targeted site, with the aim of the vector delivering the transgene to the targeted cells.

We believe that scientific advances, clinical progress, and the greater regulatory acceptance of gene therapy have created a promising environment to advance gene therapy products as these products are being designed to restore cell function and improve clinical outcomes, which in many cases include prevention of death at an early age. The U.S. Food and Drug Administration ("FDA") approval of Novartis's treatment for pediatric acute lymphoblastic leukemia indicates that there is a regulatory pathway forward for gene therapy products.

Pipeline Overview

LVV Programs. Rocket's LVV-based programs utilize third-generation, self-inactivating lentiviral vectors to target selected rare diseases. Currently, Rocket is developing LVV programs to treat FA, LAD-I, PKD, and IMO.

The chart below shows the current phases of development of Rocket's programs and product candidates:



Fanconi Anemia Complementation Group A (FANCA):

FA, a rare and life-threatening DNA-repair disorder, generally arises from a mutation in a single FA gene. An estimated 60 to 70% of cases arise from mutations in the Fanconi-A ("FANCA") gene, which is the focus of the current Rocket program. FA results in bone marrow failure, developmental abnormalities, myeloid leukemia and other malignancies, often during the early years and decades of life. Bone marrow aplasia, which is bone marrow that no longer produces any or very few red and white blood cells and platelets leading to infections and bleeding, is the most frequent cause of early morbidity and mortality in FA, with a median onset before 10 years of age. Leukemia is the next most common cause of mortality, ultimately occurring in about 20% of patients later in life. Solid organ malignancies, such as head and neck cancers, can also occur, although at lower rates during the first two to three decades of life.

Although improvements in allogeneic (donor-mediated) hematopoietic stem cell transplant ("HSCT"), currently the most frequently utilized therapy for FA, have resulted in more frequent hematologic correction of the disorder, HSCT is associated with both acute and long-term risks, including transplant-related mortality, graft versus host disease ("GVHD"), a sometimes fatal side effect of allogeneic transplant characterized by painful ulcers in the GI tract, liver toxicity and skin rashes, as well as increased risk of subsequent cancers. Rocket's gene therapy program in FA is designed to enable a minimally toxic hematologic correction using a patient's own stem cells during the early years of life. Rocket believes that the development of a broadly applicable autologous gene therapy can be transformative for these patients.

Rocket's LVV-based programs utilize third-generation, self-inactivating lentiviral vectors to correct defects in patients' HSCs, which are the cells found in bone marrow that are capable of generating blood cells over a patient's lifetime. Defects in the genetic coding of HSCs can result in severe, and potentially life-threatening anemia, which is when a patient's blood lacks enough properly functioning red blood cells to carry oxygen throughout the body. Stem cell defects can also result in severe and potentially life-threatening decreases in white blood cells resulting in susceptibility to infections, and in platelets responsible for blood clotting, which may result in severe and potentially life-threatening bleeding episodes. Patients with FA have a genetic defect that prevents the normal repair of genes and chromosomes within blood cells in the bone marrow, which frequently results in the development of acute myeloid leukemia ("AML"), a type of blood cancer, as well as bone marrow failure and congenital defects. The average lifespan of an FA patient is estimated to be 30 to 40 years. The prevalence of FA in the U.S. and European Union ("EU") is estimated to be about 2,000, and given the efficacy seen in non-conditioned patients, the addressable annual market opportunity is now thought to be in the 400-500 range, or at least double previous estimates.

We currently have one LVV-based program targeting FA, RP-L102. RP-L102 is our lead lentiviral vector based program that we in-licensed from Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas ("CIEMAT"), which is a leading research institute in Madrid, Spain. RP-L102 is currently being studied in a Rocket-sponsored Phase 1 clinical trial treating FA patients initially at the Center for Definitive and Curative Medicine at Stanford University School of Medicine ("Stanford University"). A modified process under an Investigational Medicinal Product Dossier ("IMPD"), is being studied in a trial sponsored by CIEMAT. We are entitled to the data from this clinical study and have the commercial rights to the drug being studied under this IMPD.

We submitted an IND to initiate clinical studies of RP-L102 in the U.S. in September 2018 and were notified by the FDA of its clearance in November 2018. The clinical trial will evaluate "Process B" RP-L102 which incorporates a modified cell enrichment process, transduction enhancers, and commercial-grade vector manufacturing and cell processing. In March 2019, the Company announced that the first patient has been dosed in the open-label, Phase 1 clinical trial of RP-L102, the Company's LVV based gene therapy for the treatment of FA. The patient was dosed at Stanford University, the lead U.S. clinical site.

The Phase 1 clinical trial of "Process B" RP-L102 is expected to enroll 2 FA pediatric patients at the Center for Definitive and Curative Medicine at Stanford University. The initial two FA pediatric patients have received treatment on the Phase 1 clinical trial of "Process B" RP-L102 at the Center for Definitive and Curative Medicine at Stanford. "Process B" incorporates a modified cell enrichment process, transduction enhancers, and commercial-grade vector manufacturing and cell processing. The study is designed to assess the safety and tolerability of a single infusion of RP-L102, as well as efficacy endpoints. Preliminary data is expected by the end of 2019.

On April 15, 2019, we announced updated long-term clinical data from the ongoing Phase 1/2 trial of RP-L102, sponsored by Ciemat, for FA that utilizes 'Process A' without the use of myeloablative conditioning was presented at the American Society of Cell and Gene Therapy ("ASCGT") annual meeting. Results for the four patients who have been followed for at least one year and up to three years show increasing and durable engraftment in peripheral blood and bone marrow, and restoration of bone marrow hematopoietic stem cell function, which we believe suggests patients are approaching an FA mosaic phenotype. These long-term data further support the clinical development of RP-L102 as a potential therapeutic option for FA patients as a means of averting bone marrow failure and avoiding the need for a more toxic bone marrow transplantation.

Leukocyte Adhesion Deficiency-I (LAD-I):

Overview of LAD-I

LAD-I is a rare autosomal recessive disorder of white blood cell adhesion and migration, resulting from mutations in the ITGB2 gene encoding for the Beta-2 Integrin component, CD18. Deficiencies in CD18 result in an impaired ability for neutrophils (a subset of infection-fighting white blood cells) to leave blood vessels and enter into tissues where these cells are needed to combat infections. As is the case with many rare diseases, true estimates of incidence are difficult; however, several hundred cases (both living and deceased) have been reported to date.

Most LAD-I patients are believed to have the severe form of the disease. Severe LAD-I is notable for recurrent, life-threatening infections and substantial infant mortality in patients who do not receive an allogeneic HSCT. Mortality for severe LAD-I has been reported as 60 to 75% by age two in the absence of allogeneic HCST.

Rocket currently has one program targeting LAD-I, RP-L201. RP-L201 is a clinical program that Rocket in-licensed from CIEMAT. The planned open-label, single-arm, Phase 1/2 registration enabling clinical trial of RP-L201 is expected to enroll two severe LAD-I patients in the U.S. or E.U. We anticipate receiving initial Phase I clinical data from this trial in the second half of 2019.

Rocket partners with UCLA to lead U.S. clinical development efforts for LAD-I and program. UCLA and its Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research is serving as the lead U.S. clinical research center for the planned registrational clinical trial for LAD-I.

Pyruvate Kinase Deficiency (PKD):

Red blood cell ("RBC") PKD is a rare autosomal recessive disorder resulting from mutations in the pyruvate kinase L/R ("PKLR") gene encoding for a component of the RBC glycolytic pathway. PKD is characterized by chronic non-spherocytic hemolytic anemia, a disorder in which RBCs do not assume a normal spherical shape and are broken down, leading to decreased ability to carry oxygen to cells, with anemia severity that can range from mild (asymptomatic) to severe forms that may result in childhood mortality or a requirement for frequent, lifelong RBC transfusions. The pediatric population is the most commonly and severely affected subgroup of patients with PKD, and PKD often results in splenomegaly (abnormal enlargement of the spleen), jaundice and chronic iron overload which is likely the result of both chronic hemolysis and the RBC transfusions used to treat the disease. The variability in anemia severity is believed to arise in part from the large number of diverse mutations that may affect the PKLR gene. Estimates of disease incidence have ranged between 3.2 and 51 cases per million in the white U.S. and EU population. Industry estimates suggest at least 2,500 cases in the U.S. and EU have already been diagnosed despite the lack of FDA-approved molecularly targeted therapies.

We currently have one LVV-based program targeting PKD, RP-L301. RP-L301 is a preclinical program that we in-licensed from CIEMAT. This program is currently being developed through an ongoing collaboration with CIEMAT. New market research indicates the application of gene therapy to broader populations could increase the market opportunity from approximately 250 to 500 per year.

In the EU, the PKD program has been discussed with the EMA via a Scientific Advisory meeting in 2016. This program has been granted EMA orphan drug disease designation and FDA orphan drug disease designation ("ODD"). A rolling IMPD is expected to be filed in the next few months. We expect to initiate a Phase 1 clinical trial of RP-L301 in the second half of 2019.

Infantile Malignant Osteopetrosis (IMO):

IMO is a genetic disorder characterized by increased bone density and bone mass secondary to impaired bone resorption. Normally, small areas of bone are constantly being broken down by special cells called osteoclasts, then made again by cells called osteoblasts. In IMO, the cells that break down bone (osteoclasts) do not work properly, which leads to the bones becoming thicker and not as healthy. Untreated IMO patients may suffer from a compression of the bone-marrow space, which results in bone marrow failure, anemia and increased infection risk due to the lack of production of white blood cells. Untreated IMO patients may also suffer from a compression of cranial nerves, which transmit signals between vital organs and the brain, resulting in blindness, hearing loss and other neurologic deficits.

Rocket currently has one LVV-based program targeting IMO, RP-L401. RP-L401 is a preclinical program that Rocket in-licensed from Lund University, Sweden. This program has been granted ODD from the FDA.

In March 2019, Rocket received Rare Pediatric Disease designation from the FDA for RP-L401 for the treatment of IMO. The FDA defines a "rare pediatric disease" as a serious and life-threatening disease that affects less than 200,000 people in the U.S. that are aged between birth to 18 years. The Rare Pediatric Disease designation program allows for a Sponsor who receives an approval for a product to potentially qualify for a voucher that can be redeemed to receive a priority review of a subsequent marketing application for a different product. Rocket has partnered with UCLA to lead U.S clinical development efforts for the IMO program and UCLA is serving as the lead U.S clinical site for IMO.

AAV Program:

Overview of Danon Disease

Danon disease is a multi-organ lysosomal-associated disorder leading to early death due to heart failure. RP-A501 is in preclinical development as an *in vivo* therapy for Danon disease, which is estimated to have a prevalence of 15,000 to 30,000 patients in the U.S. and the EU, however new market research is being performed and the prevalence of patients may be updated in the future. Danon disease is caused by mutations in the gene encoding lysosome-associated membrane protein 2 ("LAMP-2"), a mediator of autophagy. This mutation results in the accumulation of autophagic vacuoles, predominantly in cardiac and skeletal muscle. Male patients often require heart transplantation and typically die in their teens or twenties from progressive heart failure. Along with severe cardiomyopathy, other Danon disease symptoms can include skeletal muscle weakness, liver disease, and intellectual impairment. There are no specific therapies available for the treatment of Danon disease.

In November 2018, we announced our AAV-based RP-A501 program for the treatment of Danon disease, along with animal study data providing preclinical proof-of-concept for the RP-A501 program. Preclinical efficacy studies were performed in LAMP-2 knockout mice. Four doses of vector were tested for optimal transduction of the heart, skeletal muscle, and liver. Toxicology studies were conducted in wild-type mice and non-human primates. The results from these studies are summarized as follows:

- Increased survival rates were observed at higher doses of RP-A501 along with dose-dependent improvements and restoration of cardiac function.
- RP-A501 elicited phenotypic reversals at a structural and molecular level in cardiac, liver, and skeletal muscle tissue.
- There were no treatment-related adverse events or deaths associated with RP-A501. All doses were observed to be well-tolerated in Good Laboratory Practice biodistribution and toxicology studies in both wildtype mice and additional studies in non-human primates.

In January 2019, we announced the clearance of our IND application by the FDA for RP-A501. We anticipate initiating our Phase 1 clinical study of RP-A501 for Danon disease in the second quarter of 2019. University of California San Diego Health will be the initial and lead center for the planned Phase 1 clinical trial. In February 2019, we were notified by the FDA that we were granted Fast Track designation for RP-A501.

On April 15, 2019, we announced additional preclinical data at the ASCGT annual meeting, indicating that high vector copy number ("VCN"), in Danon disease-relevant organs in both mice and non-human primates ("NHN's"), with high concentrations in heart and liver tissue (for NHP, cardiac VCN was approximately 10 times higher on average than in skeletal muscle and central nervous system), which is consistent with reported results in several studies of heart tissue across different species. There were no treatment-related adverse events or safety issues up to the highest dose.

CRISPR/Cas9 gene editing in Fanconi Anemia:

In addition to its LVV and AAV programs, Rocket also has a program evaluating CRISPR/Cas9-based gene editing for FA. This program is currently in the discovery phase. CRISPR/Cas9-based gene editing is a different method of correcting the defective genes in a patient, where the editing is very specific and targeted to a particular gene sequence. "CRISPR/Cas9" stands for Clustered, Regularly Interspaced Short Palindromic Repeats ("CRISPR") Associated protein-9. The CRISPR/Cas9 technology can be used to make "cuts" in DNA at specific sites of targeted genes, making it potentially more precise in delivering gene therapies than traditional vector-based delivery approaches. CRISPR/Cas9 can also be adapted to regulate the activity of an existing gene without modifying the actual DNA sequence, which is referred to as gene regulation.

Strategy

We seek to bring hope and relief to patients with devastating, undertreated, rare pediatric diseases through the development and commercialization of potentially curative first-in-class gene therapies. To achieve these objectives, we intend to develop into a fully-integrated biotechnology company. In the near- and medium-term, we intend to develop our first-in-class product candidates, which are targeting devastating diseases with substantial unmet need. In the medium and long-term, we expect to develop proprietary in-house analytics and manufacturing capabilities, commence registration trials for our currently planned programs and submit our first biologics license applications ("BLAs"), and establish our gene therapy platform and expand our pipeline to target additional indications that we believe to be potentially compatible with our gene therapy technologies. In addition, during that time, we believe that our currently planned programs will become eligible for priority review vouchers from the FDA that provide for expedited review. We have assembled a leadership and research team with expertise in cell and gene therapy, rare disease drug development and commercialization.

We believe that our competitive advantage lies in our disease-based selection approach, a rigorous process with defined criteria to identify target diseases. We believe that this approach to asset development differentiates us as a gene therapy company and potentially provides us with a first-mover advantage.

Financial Overview

Since our inception, we have devoted substantially all of our resources to organizing and staffing the company, business planning, raising capital, acquiring or discovering product candidates and securing related intellectual property rights, conducting discovery, research and development activities for the programs and planning for potential commercialization. We do not have any products approved for sale and have not generated any revenue from product sales. From inception through March 31, 2019, Rocket raised net cash proceeds of approximately $195.2 million from investors through both equity and convertible debt financing to fund operating activities. On April 18, 2019, Rocket closed a public offering of common stock of 5,175,000 shares of common stock and received net proceeds of approximately $86.0 million. As of April 30, 2019, we had cash, cash equivalents and investments of $273.1 million.

Since inception, we have incurred significant operating losses. Our ability to generate product revenue sufficient to achieve profitability will depend heavily on the successful development and eventual commercialization of one or more of the current or future product candidates and programs. Rocket had net losses of $74.5 million for the year ended December 31, 2018 and $19.5 million for the three months ended March 31, 2019. As of March 31, 2019, we had an accumulated deficit of $125.3 million. We expect to continue to incur significant expenses and higher operating losses for the foreseeable future as we advance our current product candidates from discovery through preclinical development and clinical trials and seek regulatory approval of our product candidates. In addition, if we obtain marketing approval for any of their product candidates, we expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution. Furthermore, we expect to incur additional costs as a public company. Accordingly, we will need additional financing to support continuing operations and potential acquisitions of licensing or other rights for product candidates.

Until such a time as we can generate significant revenue from product sales, if ever, we will seek to fund our operations through public or private equity or debt financings or other sources, which may include collaborations with third parties and government programs or grants. Adequate additional financing may not be available to us on acceptable terms, or at all. We can make no assurances that we will be able to raise the cash needed to fund our operations and, if we fail to raise capital when needed, we may have to significantly delay, scale back or discontinue the development and commercialization of one or more product candidates or delay pursuit of potential in-licenses or acquisitions.

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. Even if we are able to generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

Financial Overview

Revenue

To date, we have not generated any revenue from any sources, including from product sales, and we do not expect to generate any revenue from the sale of products in the near future. If our development efforts for product candidates are successful and result in regulatory approval or license agreements with third parties, we may generate revenue in the future from product sales.

Operating Expenses

Research and Development Expenses

Our research and development program ("R&D") expenses consist primarily of external costs incurred for the development of our product candidates. These expenses include:

- expenses incurred under agreements with research institutions that conduct research and development activities including, process development, preclinical, and clinical activities on Rocket's behalf;
- costs related to process development, production of preclinical and clinical materials, including fees paid to contract manufacturers and manufacturing input costs for use in internal manufacturing processes;
- consultants supporting process development and regulatory activities; and
- costs related to in-licensing of rights to develop and commercialize our product candidate portfolio.

We recognize external development costs based on contractual payment schedules aligned with program activities, invoices for work incurred, and milestones which correspond with costs incurred by the third parties. Nonrefundable advance payments for goods or services to be received in the future for use in research and development activities are recorded as prepaid expenses.

Our direct research and development expenses are tracked on a program-by-program basis for product candidates and consist primarily of external costs, such as research collaborations and third party manufacturing agreements associated with our preclinical research, process development, manufacturing, and clinical development activities. Our direct research and development expenses by program also include fees incurred under license agreements. Our personnel, non-program and unallocated program expenses include costs associated with activities performed by our internal research and development organization and generally benefit multiple programs. These costs are not separately allocated by product candidate and consist primarily of:

- salaries and personnel-related costs, including benefits, travel and share-based compensation, for our scientific personnel performing research and development activities;
- facilities and other expenses, which include expenses for rent and maintenance of facilities, and depreciation expense and;
- laboratory supplies and equipment used for internal research and development activities.

Our research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development. As a result, we expect that research and development expenses will increase substantially over the next several years as we increase personnel costs, including share-based compensation, supports ongoing clinical studies, seeks to achieve proof-of-concept in one or more product candidates, advances preclinical programs to clinical programs, and prepares regulatory filings for product candidates.

We cannot determine with certainty the duration and costs to complete current or future clinical studies of product candidates or if, when, or to what extent we will generate revenues from the commercialization and sale of any of its product candidates that obtain regulatory approval. We may never succeed in achieving regulatory approval for any of our product candidates. The duration, costs, and timing of clinical studies and development of product candidates will depend on a variety of factors, including:

- the scope, rate of progress, and expense of ongoing as well as any clinical studies and other research and development activities that we undertake;
- future clinical study results;
- uncertainties in clinical study enrollment rates;
- changing standards for regulatory approval; and
- the timing and receipt of any regulatory approvals.

We expect research and development expenses to increase for the foreseeable future as we continue to invest in research and development activities related to developing product candidates, including investments in manufacturing, as our programs advance into later stages of development and as we conduct additional clinical trials. The process of conducting the necessary clinical research to obtain regulatory approval is costly and time-consuming, and the successful development of product candidates is highly uncertain. As a result, we are unable to determine the duration and completion costs of research and development projects or when and to what extent we will generate revenue from the commercialization and sale of any of our product candidates.

Our future research and development expenses will depend on the clinical success of our product candidates, as well as ongoing assessments of the commercial potential of such product candidates. In addition, we cannot forecast with any degree of certainty which product candidates may be subject to future collaborations, when such arrangements will be secured, if at all, and to what degree such arrangements would affect our development plans and capital requirements. We expect our research and development expenses to increase in future periods for the foreseeable future as we seek to complete development of our product candidates.

The successful development and commercialization of our product candidates is highly uncertain. This is due to the numerous risks and uncertainties associated with product development and commercialization, including the uncertainty of:

- the scope, progress, outcome and costs of our clinical trials and other research and development activities;
- the efficacy and potential advantages of our product candidates compared to alternative treatments, including any standard of care;
- the market acceptance of our product candidates;
- obtaining, maintaining, defending and enforcing patent claims and other intellectual property rights;
- significant and changing government regulation; and
- the timing, receipt and terms of any marketing approvals.

A change in the outcome of any of these variables with respect to the development of our product candidates that we may develop could mean a significant change in the costs and timing associated with the development of our product candidates. For example, if the FDA or another regulatory authority were to require us to conduct clinical trials or other testing beyond those that we currently contemplate for the completion of clinical development of any of our product candidates that we may develop or if we experience significant delays in enrollment in any of our clinical trials, we could be required to expend significant additional financial resources and time on the completion of clinical development of that product candidate.

General and Administrative Expenses

General and administrative (“G&A”) expenses consist primarily of salaries and related benefit costs for personnel, including share-based compensation and travel expenses for our employees in executive, operational, finance, legal, business development, and human resource functions. In addition, other significant general and administrative expenses include professional fees for legal, patents, consulting, investor and public relations, auditing and tax services as well as other expenses for rent and maintenance of facilities, insurance and other supplies used in general and administrative activities. We expect general and administrative expenses to increase for the foreseeable future due to anticipated increases in headcount to support the continued advancement of our product candidates. We also anticipate that we will incur increased accounting, audit, legal, regulatory, compliance and director and officer insurance costs as well as investor and public relations expenses.

Interest Expense

Interest expense is related to Rocket’s 2021 Convertible Notes, which are due in August 2021.

Interest Income

Interest income is related to interest earned from investments.

Critical Accounting Policies and Significant Judgments and Estimates

Our consolidated financial statements are prepared in accordance with generally accepted accounting principles in the U.S.. The preparation of our financial statements and related disclosures requires us to make estimates and judgments that affect the reported amounts of assets, liabilities, costs and expenses, and the disclosure of contingent assets and liabilities in our financial statements. We base our estimates on historical experience, known trends and events and various other factors that we believe are reasonable under the circumstances, the results of which form the basis for making judgments about the carrying values of assets and liabilities that are not readily apparent from other sources. We evaluate estimates and assumptions on an ongoing basis. Actual results may differ from these estimates under different assumptions or conditions.

While our significant accounting policies are described in more detail in our Annual Report on Form 10-K filed for the year ended December 31, 2018, which was filed with the SEC on March 8, 2019, except as otherwise described below.

Refer to Part 1, Item 1, Note 3 and Note 10 of the Notes to our Consolidated Financial Statements for disclosures regarding estimates and judgments relating to leases.

Results of Operations

Comparison of the Three Months Ended March 31, 2019 and 2018

The following table summarizes the results of operations for the three months ended March 31, 2019 and 2018 ($ in thousands):

	Three Months Ended March 31,		
	2019	**2018**	**Change**
Operating expenses:			
Research and development	$ 15,137	$ 5,743	$ 9,394
General and administrative	3,808	8,662	(4,854)
Total operating expenses	18,945	14,405	4,540
Loss from operations	(18,945)	(14,405)	(4,540)
Research and development incentives	250	186	64
Interest expense	(1,604)	(1,427)	(177)
Interest and other income net	601	288	313
Accretion of discount on investments	247	15	232
Total other expense net	(506)	(938)	432
Net loss	$ (19,451)	$ (15,343)	$ (4,108)

Research and Development Expenses

R&D expenses increased from $9.4 million to $15.1 million for the three months ended March 31, 2019 compared to the three months ended March 31, 2018. The increases was primarily a result of increases in manufacturing and process development expenses of $5.5 million, an increase in research agreement expenses of $1.9 million, primarily due to the $1.4 million milestone expense on the Stanford Laboratory for Cell and Gene Therapy (“LCGM”), and an increase in compensation and benefits of $1.0 million due to increased R&D headcount.

General and Administrative Expenses

G&A expenses decreased from $4.9 million to $3.8 million for the three months ended March 31, 2019 compared to the three months ended March 31, 2018. The decrease in G&A was primarily driven by a decrease in stock compensation expense of $2.3 million and a decrease in compensation and benefits of $1.9 million. The decreases in stock compensation expense and compensation and benefits as compared to March 31, 2018 is due to compensation, severance and other expenses associated with the merger which were incurred in the three months ended March 31, 2018.

Other Expense

Other expense decreased by $0.4 million to $0.5 million for the three months ended March 31, 2019 compared to the three months ended March 31, 2018, which was primarily due to an increase in interest and accretion income of $0.5 million, offset by an increase in interest expense of $0.2 million. The increase in interest expense is due to the assumption by the Company of the 2021 Convertible Notes in connection with the Reverse Merger. The increase in interest income is due to interest on the Company’s investments.

Liquidity, Capital Resources and Plan of Operations

Since inception, we have not generated any revenue from any sources, including from product sales, and have incurred significant operating losses and negative cash flows from our operations. We have funded operations to date primarily with proceeds from the sale of preferred shares, common stock and the issuance of convertible notes.

Cash Flows

The following table summarizes our cash flows for each of the periods presented:

	Three Months Ended March 31,	
	2019	**2018**
Cash used in operating activities	$ (15,977)	$ (11,601)
Cash provided by investing activities	29,175	86,078
Cash provided by financing activities	-	78,518
Net change in cash, cash equivalents and restricted cash	$ 13,198	$ 152,995

Operating Activities

During the three months ended March 31, 2019, operating activities used $16.0 million of cash, primarily resulting from our net loss of $19.5 million offset by net changes in our operating assets and liabilities of $0.6 million and net non-cash charges of $4.1 million, including share-based compensation expense of $3.2 million. Changes in Rocket's operating assets and liabilities for the three months ended March 31, 2019 consisted of increases in operating lease liabilities of $0.2 million, prepaid expenses and other current assets of $0.3 million and accounts payable and accrued expenses of $0.1 million.

During the three months ended March 31, 2018, operating activities used $11.6 million of cash, primarily resulting from our net loss of $15.3 million and net changes in our operating assets and liabilities of $2.4 million, partially offset by net non-cash charges of $6.2 million, including share-based compensation expense of $5.4 million. Changes in our operating assets and liabilities for the three months ended March 31, 2018 consisted primarily of an increase in prepaid expenses of $0.3 million and a decrease in accounts payable and accrued expenses of $2.3 million.

Investing Activities

During the three months ended March 31, 2019, net cash provided by investing activities was $29.2 million, consisting of proceeds of $29.9 million from the maturities of investments, offset by purchases of property and equipment of $0.8 million.

During the three months ended March 31, 2018, net cash provided by investing activities was $86.1 million, consisting primarily of $76.3 million of cash acquired in connection with the Reverse Merger and $9.7 million from the maturities of short-term investments.

Financing Activities

During the three months ended March 31, 2019, there was no net cash provided by financing activities.

During the three months ended March 31, 2018, net cash provided by financing activities was $78.5 million, consisting entirely of proceeds from the issuance of common stock.

Funding Requirements

We expect expenses to increase substantially in connection with our ongoing activities, particularly as we advance our preclinical activities, initiate additional clinical trials and manufacturing of our product candidates. In addition, we expect to incur additional costs associated with operating as a public company. Our expenses will also increase as we:

- leverage our programs to advance other product candidates into preclinical and clinical development;
- seek regulatory agreements to initiate clinical trials in the EU, US and ROW;
- establish a sales, marketing, medical affairs and distribution infrastructure to commercialize any product candidates for which Rocket may obtain marketing approval and intend to commercialize on its own or jointly;
- hire additional preclinical, clinical, regulatory, quality and scientific personnel;
- expand our operational, financial and management systems and increase personnel, including personnel to support our clinical development, manufacturing and commercialization efforts and our operations as a public company;
- maintain, expand and protect our intellectual property portfolio; and
- acquire or in-license other product candidates and technologies.

As of March 31, 2019, we had cash, cash equivalents and investments of $196.6 million. On April 18, 2019, Rocket closed a public offering of common stock of 5,175,000 shares of common stock and received net proceeds of approximately $86.0 million. Considering the proceeds from the public offering, Rocket expects such resources would be sufficient to fund its operating expenses and capital expenditure requirements into the first half of 2021.

Because of the numerous risks and uncertainties associated with research, development and commercialization of pharmaceutical product candidates, we are unable to estimate the exact amount of working capital requirements. Our future funding requirements will depend on, and could increase significantly as a result of, many factors, including:

- the scope, progress, results and costs of researching and developing our product candidates, and conducting preclinical studies and clinical trials;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;
- the costs of manufacturing commercial-grade product to support commercial launch;
- the ability to receive additional non-dilutive funding, including grants from organizations and foundations;
- the revenue, if any, received from commercial sale of its products, should any of its product candidates receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending intellectual property-related claims;
- our ability to establish and maintain collaborations on favorable terms, if at all;
- the extent to which we acquire or in-license other product candidates and technologies; and
- the timing, receipt and amount of sales of, or milestone payments related to our royalties on, current or future product candidates, if any.

Until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of public or private equity offerings, debt financings, collaborations, strategic partnerships or marketing, distribution or licensing arrangements with third parties. To the extent that we raise additional capital through the sale of equity or convertible debt securities, our ownership interest may be materially diluted, and the terms of such securities could 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 restrictive covenants that limit our ability to take specified actions, such as incurring additional debt, making capital expenditures or declaring dividends. In addition, additional debt financing would result in increased fixed payment obligations.

If we raise funds through governmental funding, collaborations, strategic partnerships 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. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, reduce or eliminate our product development or future commercialization efforts or grant rights to develop and market product candidates that it would otherwise prefer to develop and market themselves.

Contractual Obligations and Commitments

Information regarding contractual obligations and commitments may be found in Note 11 of our “Consolidated Unaudited Financial Statements in this Quarterly Report on Form 10-Q.

Off-Balance Sheet Arrangements

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

JOBS Act

Under Section 107(b) of the Jumpstart Our Business Startups Act of 2012 (the “JOBS Act”), an “emerging growth company” can delay the adoption of new or revised accounting standards until such time as those standards would apply to private companies. We have irrevocably elected not to avail ourselves of this exemption and, as a result, we will adopt new or revised accounting standards at the same time as other public companies that are not emerging growth companies. There are other exemptions and reduced reporting requirements provided by the JOBS Act that we are currently evaluating. For example, as an emerging growth company, we are exempt from Sections 14A(a) and (b) of the Securities Exchange Act of 1934 (the “Exchange Act”), which would otherwise require us to (i) submit certain executive compensation matters to stockholder advisory votes, such as “say-on-pay,” “say-on-frequency” and “golden parachutes” and (ii) disclose certain executive compensation related items such as the correlation between executive compensation and performance and comparisons of our Chief Executive Officer’s compensation to our median employee compensation. We also intend to rely on an exemption from the rule requiring us to provide an auditor’s attestation report on our internal controls over financial reporting pursuant to Section 404(b) of the Sarbanes-Oxley Act and the rule requiring us to comply with any requirement that may be adopted by the Public Company Accounting Oversight Board (“PCAOB”) regarding mandatory audit firm rotation or a supplement to the auditor’s report providing additional information about the audit and the financial statements as the auditor discussion and analysis. We will continue to remain an “emerging growth company” until the earliest of the following: December 31, 2020; the last day of the fiscal year in which our total annual gross revenue is equal to or more than $1.07 billion; the date on which we have issued more than $1.0 billion in nonconvertible debt during the previous three years; or the date on which we are deemed to be a large accelerated filer under the rules of the SEC.

Recently Issued Accounting Pronouncements

A description of recently issued accounting pronouncements that may potentially impact our financial position and results of operations is disclosed in Note 3 of our "Consolidated Unaudited Financial Statements," in this Quarterly Report on Form 10-Q.

Item 3. Quantitative and Qualitative Disclosures About Market Risk

We are exposed to market risks in the ordinary course of our business. These market risks are principally limited to interest rate fluctuations. We had cash, cash equivalents and investments of $196.6 million at March 31, 2019, consisting primarily of funds in a money market account, and United States Treasury securities. The primary objective of our investment activities is to preserve principal and liquidity while maximizing income without significantly increasing risk. We do not enter into investments for trading or speculative purposes. Due to the short-term nature of our investment portfolio, we do not believe an immediate 1.0% increase in interest rates would have a material effect on the fair market value of our portfolio, and accordingly we do not expect a sudden change in market interest rates to affect materially our operating results or cash flows.

Our 2021 Convertible Notes bear interest at a fixed rate and therefore a change in interest rates would not impact the amount of interest we would have to pay on this indebtedness.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our principal executive officer and our principal financial officer, evaluated, as of the end of the period covered by this Quarterly Report on Form 10-Q, the effectiveness of our disclosure controls and procedures. Based on that evaluation of our disclosure controls and procedures as of March 31, 2019, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures as of such date are effective at the reasonable assurance level. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act are recorded, processed, summarized and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and principal financial and accounting officer, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Inherent Limitations of Internal Controls

Because of its inherent limitations, internal control over financial reporting may not prevent or detect misstatements. Therefore, even those systems determined to be effective can provide only reasonable assurance with respect to financial statement preparation and presentation. Projections of any evaluation of effectiveness to future periods are subject to the risk that controls may become inadequate because of changes in conditions, or that the degree of compliance with the policies or procedures may deteriorate.

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the quarter ended March 31, 2019, 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

From time to time, we may be subject to various legal proceedings and claims that arise in the ordinary course of our business activities. Although the results of litigation and claims cannot be predicted with certainty, we do not believe we are party to any other claim or litigation the outcome of which, if determined adversely to us, would individually or in the aggregate be reasonably expected to have a material adverse effect on our business. Regardless of the outcome, litigation can have an adverse impact on us because of defense and settlement costs, diversion of management resources and other factors.

Item 1A. Risk Factors

We operate in an industry that involves numerous risks and uncertainties. You should carefully consider the following information about these risks, together with the other information appearing elsewhere in this Quarterly Report on Form 10-Q, including our financial statements and related notes hereto. The occurrence of any of the following risks could have a material adverse effect on our business, financial condition, results of operations and future growth prospects. The risks and uncertainties described below may change over time and other risks and uncertainties, including those that we do not currently consider material, may impair our business. In these circumstances, the market price of our common stock could decline. The following Risk Factors are consistent with those previously disclosed in the 2018 Form 10-K.

Risks Related to Our Financial Position

We have a history of operating losses, and we may not achieve or sustain profitability. We anticipate that we will continue to incur losses for the foreseeable future. If we fail to obtain additional funding to conduct our planned research and development effort, we could be forced to delay, reduce or eliminate our product development programs or commercial development efforts.

We are an early-stage gene therapy company with a limited operating history on which to base your investment decision. Gene therapy product development is a highly speculative undertaking and involves a substantial degree of risk. Our operations to date have been limited primarily to organizing and staffing our company, business planning, raising capital, acquiring and developing product and technology rights and conducting preclinical research and development activities for our product candidates. We have never generated any revenue from product sales. We have not obtained regulatory approvals for any of our product candidates, and have funded our operations to date through proceeds from sales of our stock.

We have incurred net losses since our inception. We incurred net losses of $19.5 million and $15.3 million for the three months ended March 31, 2019 and 2018, respectively, and $74.5 million and $19.6 million for the years ended December 31, 2018 and 2017, respectively. As of March 31, 2019, we had an accumulated deficit of $125.3 million. Substantially all of our operating losses have resulted from costs incurred in connection with our research and development programs and from G&A costs associated with our operations. We expect to continue to incur significant expenses and operating losses over the next several years and for the foreseeable future as we intend to continue to conduct research and development, clinical testing, regulatory compliance activities, manufacturing activities, and, if any of our product candidates is approved, sales and marketing activities that, together with anticipated G&A expenses, will likely result in us incurring significant losses for the foreseeable future. Our prior losses, combined with expected future losses, have had and will continue to have an adverse effect on our accumulated deficit and working capital.

We may need to raise additional funding, which may not be available on acceptable terms, or at all. Failure to obtain this necessary capital when needed may force us to delay, limit or terminate certain of our licensing activities, product development efforts or other operations.

We expect to require substantial future capital in order to seek to broaden licensing of our gene therapy platforms, complete preclinical and clinical development for our current product candidates and other future product candidates, if any, and potentially commercialize these product candidates. We expect our spending levels to increase in connection with our preclinical and clinical trials. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant expenses related to product sales, medical affairs, marketing, manufacturing and distribution. Furthermore, we expect to incur additional costs associated with operating as a public company. Accordingly, we will need to obtain substantial additional funding in connection with our continuing operations. If we are unable to raise capital when needed or on acceptable terms, we could be forced to delay, reduce or eliminate certain of our licensing activities, our research and development programs or other operations.

Our operations have consumed significant amounts of cash since inception. As of March 31, 2019, our cash, cash equivalents and investments was $196.6 million. Our future capital requirements will depend on many factors, including:

- the timing of enrollment, commencement, completion and results of our clinical trials;
- the production of LVV and AAV gene therapy products to support preclinical and clinical needs
- the results of our preclinical studies for our current product candidates and any subsequent clinical trials;
- the scope, progress, results and costs of drug discovery, laboratory testing, preclinical development and clinical trials, if any, for our internal product candidates; the costs associated with building out additional laboratory and research capacity;
- the costs, timing and outcome of regulatory review of our product candidates;
- the costs of future activities, including product sales, medical affairs, marketing, manufacturing and distribution, for any of our product candidates for which we receive marketing approval;
- the costs of preparing, filing and prosecuting patent applications, maintaining and enforcing our intellectual property rights and defending any intellectual property-related claims;
- our current licensing agreements or collaborations remaining in effect;
- our ability to establish and maintain additional licensing agreements or collaborations on favorable terms, if at all;
- the extent to which we acquire or in-license other product candidates and technologies; and
- the costs associated with being a public company.

Many of these factors are outside of our control. Identifying potential product candidates and conducting preclinical testing and clinical trials is a time-consuming, expensive and uncertain process that takes years to complete, and we may never generate the necessary data or results required to obtain regulatory and marketing approval and achieve product sales. In addition, our product candidates, if approved, may not achieve commercial success. Accordingly, we will need to continue to rely on additional financing to achieve our business objectives.

To the extent that additional capital is raised through the sale of equity or equity-linked securities, the issuance of those securities could result in substantial dilution for our current stockholders and the terms may include liquidation or other preferences that adversely affect the rights of our current stockholders. Adequate additional financing may not be available to us on acceptable terms, or at all. We also could be required to seek funds through arrangements with partners or otherwise that may require us to relinquish rights to our intellectual property, our product candidates or otherwise agree to terms unfavorable to us.

Our limited operating history may make it difficult for us to evaluate the success of our business to date and to assess our future viability.

Our operations to date have predominantly focused on organizing and staffing our company, business planning, raising capital, acquiring our technology, administering and expanding our gene therapy platforms, identifying potential product candidates, undertaking research, preclinical studies and clinical trials of our product candidates and establishing licensing arrangements and collaborations. We have not yet completed clinical trials of our product candidates, obtained marketing approvals, manufactured a commercial-scale product or conducted sales and marketing activities necessary for successful commercialization. Consequently, any predictions made about our future success or viability may not be as accurate as they could be if we had a longer operating history.

In addition, as a relatively new business, we may encounter unforeseen expenses, difficulties, complications, delays and other known and unknown factors. We are still in the early stages of transitioning from a drug-discovery company with a licensing and research focus to a clinical-stage company that is supporting clinical development activities, and we may need to transition to supporting commercial activities in the future. We cannot guarantee that we will be successful in these transitions.

We have never generated any revenue from product sales and may never be profitable.

Our ability to generate revenue and achieve profitability depends on our ability, alone or with strategic collaboration partners, to successfully complete the development of, and obtain the regulatory, pricing and reimbursement approvals necessary to commercialize our product candidates. We do not anticipate generating revenues from product sales for the foreseeable future, if ever. Our ability to generate future revenues from product sales depends heavily on our success in:

- completing research and preclinical and clinical development of our product candidates;
- seeking and obtaining regulatory and marketing approvals for product candidates for which we complete clinical studies;
- developing a sustainable, commercial-scale, reproducible, and transferable manufacturing process for our vectors and product candidates;
- establishing and maintaining supply and manufacturing relationships with third parties that can provide adequate (in amount and quality) products and services to support clinical development and the market demand for our product candidates, if approved;
- launching and commercializing product candidates for which we obtain regulatory and marketing approval, either by collaborating with a partner or, if launched independently, by establishing a sales force, marketing and distribution infrastructure;
- obtaining sufficient pricing and reimbursement for our product candidates from private and governmental payors
- obtaining market acceptance of our product candidates and gene therapy as a viable treatment option;
- addressing any competing technological and market developments;
- identifying and validating new gene therapy product candidates;
- negotiating favorable terms in any collaboration, licensing or other arrangements into which we may enter; and
- maintaining, protecting and expanding our portfolio of intellectual property rights, including patents, trade secrets and know-how.

Even if one or more of the product candidates that we will develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing any approved product candidate. Our expenses could increase beyond expectations if we are required by the FDA, the EMA, or other regulatory agencies, domestic or foreign, to perform clinical and other studies in addition to those that we currently anticipate. Even if we are able to generate revenues from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations.

Risks Related to Product Regulatory Matters

Our gene therapy product candidates are based on novel technology, which makes it difficult to predict the time and cost of product candidate development and subsequently obtaining regulatory approval. Currently, only a few gene therapy products have been approved in the U.S. and the E.U.

We have concentrated our research and development efforts to date on a gene therapy platform, and our future success depends on the successful development of viable gene therapy product candidates. We cannot guarantee that we will not experience problems or delays in developing current or future product candidates or that such problems or delays will not cause unanticipated costs, or that any such development problems or delays can be resolved. We may also experience unanticipated problems or delays in developing our manufacturing capacity or transferring our manufacturing process to commercial partners, which may prevent us from completing our clinical studies or commercializing our products 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 more extensively studied pharmaceutical or other product candidates. Currently, only a few gene therapy products have received marketing authorization in the U.S. or the EU, including Novartis Pharmaceuticals' Kymriah, Kite Pharma's Yescarta, GlaxoSmithKline's Strimvelis and Spark Therapeutics' Luxturna. It is therefore difficult to determine how long it will take or how much it will cost to obtain regulatory approvals for our product candidates in the U.S., the EU or other jurisdictions. Approvals by the EMA may not be indicative of what the FDA may require for approval. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approvals necessary to bring a potential product to market could decrease our ability to generate sufficient product revenue and our business, financial condition, results of operations and prospects could be materially harmed.

Regulatory requirements governing gene therapy products have evolved and may continue to change in the future. For example, CBER may require us to perform additional nonclinical studies or clinical trials that may increase our development costs, lead to changes in regulatory positions and interpretations, delay or prevent approval and commercialization of our gene therapy product candidates or lead to significant post-approval limitations or restrictions.

In addition, the EMA's Committee for Advanced Therapies ("CAT") and other regulatory review committees and advisory groups and any 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 our product candidates or lead to significant post-approval limitations or restrictions. As we advance our product candidates, we will be required to consult with these regulatory and advisory groups, and comply with applicable guidelines. If we fail to do so, we may be required to delay or discontinue development of certain of our product candidates. These additional processes may result in a review and approval process that is longer than we otherwise would have expected. Delay or failure to obtain, or unexpected costs in obtaining, the regulatory approval necessary to bring a potential product to market could decrease our ability to generate product revenue, and our business, financial condition, results of operations and prospects would be materially harmed.

We may encounter substantial delays in commencement, enrollment or completion of our clinical trials or may fail to demonstrate safety and efficacy to the satisfaction of applicable regulatory authorities, which could prevent us from commercializing our current and future product candidates on a timely basis, if at all.

Before obtaining marketing approval from regulatory authorities for the sale of our current and future product candidates, we must conduct extensive clinical trials to demonstrate the safety and efficacy of our product candidates. Clinical trials are expensive, time-consuming, and outcomes are uncertain.

Our experience with clinical trials has been limited. Our clinical programs to date have been performed under an IMPD, in Spain sponsored by CIEMAT, and in the U.S. sponsored by Hutch. The clinical trials performed by these sponsors were for a lentiviral treatment for FA, a rare mutation of the FANC-A gene. We have now initiated Rocket-sponsored clinical trials for FA and LAD-I, but have not completed any clinical trials to date. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. A clinical trial may be delayed or halted at any stage of testing for various reasons, including:

- failure of patients to enroll in the studies at the rate we expect;
- ineffectiveness of our product candidates;
- patients experiencing unexpected side effects or other safety concerns being raised during treatment;
- changes in governmental regulations or administrative actions;
- failure to conduct studies in accordance with required clinical practices;
- inspection of clinical study operations or study sites by the FDA, the EMA or other regulatory authorities, resulting in a clinical hold;
- insufficient financial resources;
- insufficient supplies of drug product to treat the patients in the studies;
- political unrest at foreign clinical sites;
- a shutdown of the U.S. government, including the FDA; or
- natural disasters at any of our clinical sites.

In addition, to the extent we seek to obtain regulatory approval for our product candidates in foreign countries, our ability to successfully initiate, enroll and complete a clinical study in any foreign country is subject to numerous risks unique to conducting business in foreign countries, including:

- difficulty in establishing or managing relationships with CROs, and physicians;
- different standards for the conduct of clinical trials;
- absence in some countries of established groups with sufficient regulatory expertise for review of LVV and AAV gene therapy protocols;
- our inability to locate qualified local partners or collaborators for such clinical trials; and
- the potential burden of complying with a variety of foreign laws, medical standards and regulatory requirements, including the regulation of pharmaceutical and biotechnology products and treatment.

Moreover, we intend to rely on the nonclinical studies and clinical trials performed by CIEMAT, and the FDA or the regulatory authority in any other country in which we decide to perform clinical trials or seek approval may not accept that results of the CIEMAT studies and trials. Any inability to successfully complete preclinical studies and clinical trials could result in additional costs to us or impair our ability to generate revenues from product sales, regulatory and commercialization milestones and royalties.

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

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

- severity of the disease under investigation;
- design of the study protocol;
- size of the patient population;
- eligibility criteria for the study in question;
- perceived risks and benefits of the product candidate under study, including as a result of adverse effects observed in similar or competing therapies;
- proximity and availability of clinical study sites for prospective patients;
- availability of competing therapies and clinical studies;
- efforts to facilitate timely enrollment in clinical studies;
- patient referral practices of physicians; and
- ability to monitor patients adequately during and after treatment.

In particular, each of the conditions for which we plan to evaluate our current product candidates are rare genetic diseases with limited patient pools from which to draw for clinical studies. Additionally, the process of finding and diagnosing patients may prove costly. In some cases, potential patients may be located outside of the U.S., and immigration related issues, including government policy changes, may introduce additional delays into the enrollment process. Finally, the treatment process requires that the cells be obtained from patients and then shipped to a transduction facility within the required timelines, and this may introduce unacceptable shipping-related delays to the process.

We have not completed any clinical studies of our current product candidates. Initial or interim results in our ongoing clinical studies may not be indicative of results obtained when these studies are completed. Furthermore, success in early clinical studies may not be indicative of results obtained in later studies.

Our FA gene therapy treatment was studied in clinical trials conducted by our partners. We have now initiated Rocket sponsored clinical trials for FA and LAD-I, but have not completed any clinical trials to date. We cannot guarantee that any clinical trials will be conducted as planned or completed on schedule, if at all. Our other gene therapy programs are in the preclinical stages. Study designs and results from previous or ongoing studies and clinical trials are not necessarily predictive of future study or clinical trial results, and initial or interim results may not continue or be confirmed upon completion of the study or trial. Positive data may not continue or occur for subjects in our clinical studies or for any future subjects in our ongoing or future clinical studies, and may not be repeated or observed in ongoing or future studies involving our product candidates. Furthermore, our product candidates may also fail to show the desired safety and efficacy in later stages of clinical development despite having successfully advanced through initial clinical studies. We cannot guarantee that any of these studies will ultimately be successful or that preclinical or early stage clinical studies will support further clinical advancement or regulatory approval of our product candidates.

Data obtained from preclinical and clinical activities are subject to varying interpretations, which may delay, limit or prevent regulatory approval. In addition, regulatory delays or rejections may be encountered as a result of many factors, including changes in regulatory policy during the period of product development.

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

We cannot commercialize a product candidate until the appropriate regulatory authorities have reviewed and approved the product candidate. We have not received approval from regulatory authorities in any jurisdiction to market any of our product candidates. Even if our product candidates meet their safety and efficacy endpoints in clinical trials, the regulatory authorities may not complete their review processes in a timely manner, issue a complete response letter, or ultimately, we may not be able to obtain regulatory approval. In addition, we may experience delays or rejections if an FDA Advisory Committee recommends disapproval or restrictions on use. In addition, we may experience delays or rejections based upon additional government regulation from future legislation or administrative actions, or changes in regulatory authority policy during the period of product development, clinical trials and the review process. Regulatory authorities have substantial discretion in the approval process and may refuse to accept any application or may decide that our data are insufficient for approval and require additional preclinical, clinical or other studies. In addition, varying interpretations of data obtained from preclinical and clinical testing could delay, limit or prevent the receipt of marketing approval for a product candidate.

Regulatory authorities also may approve a product candidate for more limited indications than requested or they may impose significant limitations in the form of narrow indications, warnings or REMS. These regulatory authorities may require precautions or contra-indications with respect to conditions of use or they may grant approval subject to the performance of costly post-marketing clinical trials. In addition, regulatory authorities may not approve the labeling claims that are necessary or desirable for the successful commercialization of our product candidates. Any of the foregoing scenarios could materially harm the commercial prospects for our product candidates and materially harm our business, financial condition, results of operations and prospects.

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

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

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

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

- issue a warning letter asserting that we are in violation of the law;
- seek an injunction or impose civil or criminal penalties or monetary fines;
- suspend any ongoing clinical studies;
- refuse to approve a pending marketing application, such as a BLA or supplements to a BLA submitted by us;
- seize products; or
- refuse to allow us to enter into supply contracts, including government contracts.

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

In addition, the FDA’s policies, and those of comparable foreign regulatory authorities, may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative actions, either in the U.S. or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval which we may have obtained and we may not achieve or sustain profitability, which would materially harm our business, financial condition, results of operations and prospects.

We may never obtain FDA or EMA approval for any of our product candidates in the U.S. or the EU, and even if we do, we may never obtain approval for or commercialize any of our product candidates in any other jurisdiction, which would limit our ability to realize our full market potential.

In order to eventually market any of our product candidates in any particular jurisdiction, we must establish and comply with numerous and varying regulatory requirements regarding safety and efficacy on a jurisdiction-by-jurisdiction basis. Approval by the FDA in the U.S. or the EMA in the EU, if obtained, does not ensure approval by regulatory authorities in other countries or jurisdictions. In addition, preclinical studies and clinical trials conducted in one country may not be accepted by regulatory authorities in other countries, and regulatory approval in one country does not guarantee regulatory approval in any other country. Approval processes vary among countries and can involve additional product testing and validation and additional administrative review periods. Seeking foreign regulatory approval could result in difficulties and costs for us and require additional preclinical studies or clinical trials which could be costly and time-consuming. Regulatory requirements can vary widely from country to country and could delay or prevent the introduction of our products in those countries. The foreign regulatory approval process involves similar risks to those associated with FDA and EMA approval. We do not have any product candidates approved for sale in any jurisdiction, including international markets, nor have we attempted to obtain such approval. If we fail to comply with regulatory requirements in international markets or to obtain and maintain required approvals, or if regulatory approvals in international markets are delayed, our target market will be reduced and our ability to realize the full market potential of our products will be unrealized.

Our product candidates may cause undesirable and unforeseen side effects or be perceived by the public as unsafe, which could delay or prevent their advancement into clinical trials or regulatory approval, limit the commercial potential or result in significant negative consequences.

Gene therapy is still a relatively new approach to disease treatment and adverse side effects could develop with our product candidates. There also is the potential risk of delayed adverse events following exposure to gene therapy products due to persistent biologic activity of the genetic material or other components of products used to carry the genetic material.

Possible adverse side effects that could occur with treatment with gene therapy products include an immunologic reaction soon after administration which could substantially limit the effectiveness and durability of the treatment. If certain side effects are observed in testing of our potential product candidates, we may decide or be required to halt or delay further clinical development of our product candidates.

In addition to side effects caused by the product candidate, the administration process or related procedures associated with a given product candidate also can cause adverse side effects. If any such adverse events occur, our clinical trials could be suspended or terminated. Under certain circumstances, the FDA, the European Commission, the EMA or other regulatory authorities could order us to cease further development of, or deny approval of, our product candidates for any or all targeted indications. Moreover, if we elects or are required, to not initiate or to delay, suspend or terminate any future clinical trial of any of our product candidates, the commercial prospects of such product candidates may be harmed and our ability to generate product revenues from any of these product candidates may be delayed or eliminated. Any of these occurrences may harm our ability to develop other product candidates, and may harm our business, financial condition and prospects significantly.

Furthermore, if undesirable side effects caused by our product candidate are identified following regulatory approval of a product candidate, several potentially significant negative consequences could result, including:

- regulatory authorities may suspend or withdraw approvals of such product candidate;
- regulatory authorities may require additional warnings on the label;
- we may be required to change the way a product candidate is administered or conduct additional clinical trials; and
- our reputation may suffer.

Any of these occurrences may harm our business, financial condition and prospects significantly.

Risks Related to Manufacturing, Development and Commercialization of Our Product Candidates

Products intended for use in gene therapies are novel, complex and difficult to manufacture. We could experience production problems that result in delays in our development or commercialization programs, limit the supply of our products or otherwise harm our business.

We currently have development, manufacturing and testing agreements with third parties to manufacture supplies of our product candidates. Several factors could cause production interruptions, including equipment malfunctions, facility contamination, raw material shortages or contamination, natural disasters, disruption in utility services, human error or disruptions in the operations of suppliers.

Our product candidates require processing steps that are more complex than those required for small molecule pharmaceuticals.

We may encounter problems contracting with, hiring and retaining the experienced scientific, quality control and manufacturing personnel needed to operate our manufacturing process which could result in delays in our production or difficulties in maintaining compliance with applicable regulatory requirements.

Any problems in our manufacturing process or the facilities with which we contract could make us a less attractive collaborator for potential partners, including larger pharmaceutical companies and academic research institutions, which could limit our access to attractive development programs. Any such problems could also result in a decision to abandon or write off prior investments, which could result in significant accounting charges for impairment of previously capitalized expenditures. Problems in third-party manufacturing processes or facilities also could restrict our ability to meet market demand for our products. Additionally, should our manufacturing agreements with third parties be terminated for any reason, there may be a limited number of manufacturers who would be suitable replacements and it could take a significant amount of time to transition the manufacturing to a replacement.

Even if approved, we may not successfully commercialize our drug candidates.

Our gene therapy product candidates are subject to the risks of failure inherent in the development of pharmaceutical products based on new technologies, and our failure to develop safe, commercially viable products would severely limit our ability to become profitable or to achieve significant revenues. Even if one or more of our drug candidates is approved, we may be unable to successfully commercialize our product candidates for several reasons, including:

some or all of our product candidates may be found to be unsafe or ineffective or otherwise fail to meet applicable regulatory standards or receive necessary regulatory clearances;

- our product candidates, if safe and effective, may nonetheless not be able to be developed into commercially viable products;
- it may be difficult to manufacture or market our product candidates on a scale that is necessary to ultimately deliver our products to end-users;
- proprietary rights of third parties may preclude us from marketing our product candidates;
- the nature of our indications as rare diseases means that the potential market size may be limited; and
- third parties may market superior or equivalent drugs which could adversely affect the commercial viability and success of our product candidates.

Our ability to successfully develop and commercialize our product candidates will substantially depend upon the availability of reimbursement funds for the costs of the resulting drugs and related treatments.

Market acceptance and sales of our product candidates may depend on coverage and reimbursement policies and health care reform measures. Decisions about formulary coverage as well as levels at which government authorities and third-party payors, such as private health insurers and health maintenance organizations, reimburse patients for the price they pay for our products as well as levels at which these payors pay directly for our products, where applicable, could affect whether we are able to successfully commercialize these products. We cannot guarantee that reimbursement will be available for any of our product candidates, nor can we guarantee that coverage or reimbursement amounts will not reduce the demand for, or the price of, our product candidates. We have not commenced efforts to have our product candidates reimbursed by government or third-party payors. If coverage and reimbursement are not available or are available only at limited levels, we may not be able to successfully commercialize our products. In March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act, or the PPACA, was signed into law, and in recent years, numerous proposals to change the health care system in the U.S. have been made. These reform proposals include measures that would limit or prohibit payments for certain medical treatments or subject the pricing of drugs to government control. In addition, in many foreign countries, particularly the countries of the EU, the pricing of prescription drugs is subject to government control. If our products are or become subject to government regulation that limits or prohibits payment for our products, or that subjects the price of our products to governmental control, we may not be able to generate revenue, attain profitability or commercialize our products.

In addition, third-party payors are increasingly limiting both coverage and the level of reimbursement of new drugs. They may also impose strict prior authorization requirements and/or refuse to provide any coverage of uses of approved products for medical indications other than those for which the FDA has granted market approvals. As a result, significant uncertainty exists as to whether and how much third-party payors will reimburse patients for their use of newly-approved drugs. If we are unable to obtain adequate levels of reimbursement for our product candidates, our ability to successfully market and sell our product candidates will be harmed. The manner and level at which reimbursement is provided for services related to our product candidates (e.g., for administration of our product to patients) is also important to successful commercialization of our product candidates. Inadequate reimbursement for such services may lead to physician resistance and limit our ability to market or sell our products.

We face intense competition and rapid technological change and the possibility that our competitors may develop therapies that are more advanced or effective than ours, which may adversely affect our financial condition and our ability to successfully commercialize our product candidates.

We are engaged in gene therapy for severe genetic and rare diseases, which is a competitive and rapidly changing field. Although we are not currently aware of any gene therapy competitors addressing any of the same indications as those in our pipeline, we may have competitors both in the U.S. States and internationally, including major multinational pharmaceutical companies, biotechnology companies and universities and other research institutions.

Our potential competitors may have substantially greater financial, technical and other resources, such as larger research and development staff, manufacturing capabilities and experienced marketing and manufacturing organizations. These competitors may succeed in developing, acquiring or licensing on an exclusive basis, products that are more effective or less costly than any product candidate that we may develop, or achieve earlier patent protection, regulatory approval, product commercialization and market penetration than us. Additionally, technologies developed by our competitors may render our potential product candidates uneconomical or obsolete, and we may not be successful in marketing our product candidates against those of our competitors.

In addition, if our patent rights were to expire or be successfully challenged, we could face increased litigation with respect to the validity and/or scope of patents relating to our competitors' products. The availability of our competitors' products could limit the demand, and the price we are able to charge, for any products that we may develop and commercialize, thereby causing harm to our business, financial condition, results of operations and prospects.

We may not be successful in our efforts to build a pipeline of additional product candidates.

Our business model is centered on applying our expertise in rare genetic diseases by establishing focused selection criteria to develop and advance a portfolio of gene therapy product candidates through development into commercialization. We may not be able to continue to identify and develop new product candidates in addition to the pipeline of product candidates that our research and development efforts to date have resulted in. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development. If we do not successfully develop and commercialize product candidates based upon our approach, we will not be able to obtain product revenue in future periods, which would likely result in significant harm to our financial position and results of operations.

The success of our research and development activities, clinical testing and commercialization, upon which we primarily focus, is uncertain.

Our primary focus is on our research and development activities and the clinical testing and commercialization of our product candidates and we anticipate that we will remain principally engaged in these activities for an indeterminate, but substantial, period of time. Research and development was our most significant operating expense for the year ended December 31, 2018. Research and development activities, including the conduct of clinical studies, by their nature, preclude definitive statements as to the time required and costs involved in reaching certain objectives. Actual research and development costs, therefore, could significantly exceed budgeted amounts and estimated time frames may require significant extension. Cost overruns, unanticipated regulatory delays or demands, unexpected adverse side effects or insufficient therapeutic efficacy will prevent or substantially slow our research and development effort and our business could ultimately suffer.

Risks Related to Third Parties

We rely on third parties to conduct certain aspects of our preclinical studies and clinical trials and perform other tasks for us. If these third parties do not successfully carry out their contractual duties, meet expected deadlines, or comply with regulatory requirements, we may not be able to obtain regulatory approval for or commercialize our product candidates and our business, financial condition and results of operations could be substantially harmed.

We have relied upon and plan to continue to rely upon third parties, including CROs, medical institutions, and contract laboratories for certain aspects of our ongoing preclinical and clinical programs. Nevertheless, we maintain responsibility for ensuring that each of our clinical trials and preclinical studies is conducted in accordance with the applicable protocol, legal, regulatory, and scientific standards and our reliance on these third parties does not relieve us of our regulatory responsibilities. We and our vendors are required to comply with current requirements on GMP, good clinical practice ("GCP"), and good laboratory practice ("GLP"), which are a collection of laws and regulations enforced by the FDA, the EMA or comparable foreign authorities for all of our drug candidates in clinical development.

Regulatory authorities enforce these regulations through periodic inspections of preclinical study and clinical trial sponsors, principal investigators, preclinical study and clinical trial sites, and other contractors. If we or any of our vendors fail to comply with applicable regulations, the data generated in our preclinical studies and clinical trials may be deemed unreliable and the FDA, the EMA or comparable foreign authorities may require us to perform additional preclinical studies and clinical trials before approving our marketing applications. We cannot assure you that upon inspection by a given regulatory authority, such regulatory authority will determine that any of our clinical trials comply with GCP regulations. In addition, our clinical trials must be conducted with products produced consistent with GMP regulations. Our failure to comply with these regulations may require us to repeat clinical trials, which would delay the development and regulatory approval processes.

If any of our relationships with these third parties, medical institutions, clinical investigators or contract laboratories terminate, we may not be able to enter into arrangements with alternative CROs on commercially reasonable terms, or at all. In addition, our CROs are not our employees, and except for remedies available to us under our agreements with such CROs, we cannot control whether or not they devote sufficient time and resources to our ongoing preclinical and clinical programs.

If our CROs do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the data they obtain is compromised due to the failure to adhere to our protocols, regulatory requirements, or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to obtain regulatory approval for or successfully commercialize our product candidates. CROs may also generate higher costs than anticipated. As a result, our business, financial condition and results of operations and the commercial prospects for our product candidates could be materially and adversely affected, our costs could increase, and our ability to generate revenue could be delayed.

Switching or adding additional CROs, medical institutions, clinical investigators or contract laboratories involves additional cost and requires management time and focus. In addition, there is a natural transition period when a new CRO commences work replacing a previous CRO. As a result, delays may occur, which can materially impact our ability to meet our desired clinical development timelines. Though we carefully manage our relationships with our CROs, we cannot guarantee that we will not encounter similar challenges or delays in the future or that these delays or challenges will not have a material adverse effect on our business, financial condition or results of operations.

We expect to rely on third parties to conduct some or all aspects of our drug product manufacturing, research and preclinical and clinical testing, and these third parties may not perform satisfactorily.

We do not expect to independently conduct all aspects of our gene therapy production, product manufacturing, research and preclinical and clinical testing. We currently rely, and expect to continue to rely, on third parties with respect to these items. In some cases, these third parties are academic, research or similar institutions that may not apply the same quality control protocols utilized in certain commercial settings.

Our reliance on these third parties for research and development activities will reduce our control over these activities but will not relieve us of our responsibility to ensure compliance with all required regulations and study protocols. If these third parties do not successfully carry out their contractual duties, meet expected deadlines or conduct our studies in accordance with regulatory requirements or our stated study plans and protocols, we will not be able to complete, or may be delayed in completing, the preclinical and clinical studies required to support future product submissions and approval of our product candidates.

Generally, these third parties may terminate their engagements with us at will upon notice. If we need to enter into alternative arrangements, it could delay our product development activities.

We expect to rely solely on third-party manufacturers to manufacture supplies of our product candidates. Reliance on third-party manufacturers entails risks to which we would not be subject if we manufactured the product candidates ourselves, including:

- the inability to negotiate manufacturing agreements with third parties under commercially reasonable terms;
- reduced control as a result of using third-party manufacturers for all aspects of manufacturing activities;
- the risk that these activities are not conducted in accordance with our study plans and protocols;
- termination or nonrenewal of manufacturing agreements with third parties in a manner or at a time that is costly or damaging to us; and
- disruptions to the operations of our third-party manufacturers or suppliers caused by conditions unrelated to our business or operations, including the bankruptcy of the manufacturer or supplier.

Any of these events could lead to clinical study delays or failure to obtain regulatory approval, or impact our ability to successfully commercialize future products. Some of these events could be the basis for FDA action, including an injunction, recall, seizure or total or partial suspension of production.

We may not be successful in finding strategic collaborators for continuing development of certain of our product candidates or successfully commercializing our product candidates.

We may seek to establish strategic partnerships for developing and/or commercializing certain of our product candidates due to relatively high capital costs required to develop the product candidates, manufacturing constraints or other reasons. We may not be successful in our efforts to establish such strategic partnerships or other alternative arrangements for our product candidates for several reasons, including because our research and development pipeline may be insufficient, our product candidates may be deemed to be at too early of a stage of development for collaborative effort or third parties may not view our product candidates as having the requisite potential to demonstrate efficacy or market opportunity. In addition, we may be restricted under existing agreements from entering into future agreements with potential collaborators.

If we are unable to reach agreements with suitable licensees or collaborators on a timely basis, on acceptable terms or at all, we may have to curtail the development of a product candidate, reduce or delay our development program, delay our potential commercialization, reduce the scope of any sales or marketing activities or increase our expenditures and undertake development or commercialization activities at our own expense. If we elect to independently fund development or commercialization activities, we may need to obtain additional expertise and additional capital, which may not be available on acceptable terms or at all. If we fail to enter into collaboration arrangements and does not have sufficient funds or expertise to undertake necessary development and commercialization activities, we may not be able to further develop our product candidates and our business, financial condition, results of operations and prospects may be materially harmed.

The commercial success of any of our product candidates will depend upon our degree of market acceptance by physicians, patients, third-party payors and others in the medical community.

Ethical, social, legal and other concerns about gene therapy could result in additional regulations restricting or prohibiting our products. Even with the requisite approvals from the FDA in the U.S., the EMA in the EU and other regulatory authorities internationally, the commercial success of our product candidates will depend, in part, on the acceptance of physicians, patients and health care payors of gene therapy products in general, and our product candidates in particular, as medically beneficial, cost-effective and safe. Any product that we commercialize may not gain acceptance by physicians, patients, health care payors and others in the medical community. If these products do not achieve an adequate level of acceptance, we may not generate significant product revenue and may not become profitable. The degree of market acceptance of gene therapy products and, in particular, our product candidates, if approved for commercial sale, will depend on several factors, including:

- the efficacy and safety of such product candidates as demonstrated in preclinical studies and clinical trials;
- the potential and perceived advantages of product candidates over alternative treatments;
- the cost of our treatment relative to alternative treatments;
- the clinical indications for which the product candidate is approved by the FDA or the EMA;
- patient awareness of, and willingness to seek, gene therapy;
- the willingness of physicians to prescribe new therapies;
- the willingness of physicians to undergo specialized training with respect to administration of our product candidates;
- the willingness of the target patient population to try new therapies;
- the prevalence and severity of any side effects;
- product labeling or product insert requirements of the FDA, the EMA or other regulatory authorities, including any limitations or warnings contained in a product's approved labeling;
- relative convenience and ease of administration;
- the strength of marketing and distribution support;
- the timing of market introduction of competitive products;
- publicity concerning our products or competing products and treatments; and
- sufficient third-party payor coverage and reimbursement.

Even if a potential product displays a favorable efficacy and safety profile in preclinical studies and clinical trials, market acceptance of the product will not be fully known until after it is approved and launched. The failure of any of our product candidates to achieve market acceptance could materially harm our business, financial condition, results of operations and prospects.

Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.

We rely on third parties to manufacture our products and to perform quality testing, and because we collaborate with various organizations and academic institutions for the advancement of our gene therapy platform, we must, at times, share our proprietary technology and confidential information, including trade secrets, with them. We seek to protect our proprietary technology, in part, by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees and consultants prior to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information. Despite the contractual provisions employed when working with third parties, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our proprietary technology and confidential information or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business, financial condition, results of operations and prospects.

Despite our efforts to protect our trade secrets, our competitors may discover our trade secrets, either through breach of these agreements, independent development or publication of information including our trade secrets by third parties. A competitor's discovery of our trade secrets would impair our competitive position and have an adverse impact on our business, financial condition, results of operations and prospects.

Risks Related to Personnel and Other Risks Related to Our Business

Our business could suffer if it loses the services of, or fails to attract, key personnel.

We are highly dependent upon the efforts of our senior management, including our Chief Executive Officer, Gaurav Shah, MD; our Chief Medical Officer and Head of Clinical Development, Jonathan Schwartz, MD; and our Chief Operating Officer and Head of Development, Kinnari Patel, PharmD, MBA. The loss of the services of these individuals and other members of our senior management could delay or prevent the achievement of research, development, marketing, or product commercialization objectives. Our employment arrangements with the key personnel are "at-will." We do not maintain any "key-man" insurance policies on any of the key employees nor do we intend to obtain such insurance. In addition, due to the specialized scientific nature of our business, we are highly dependent upon our ability to attract and retain qualified scientific and technical personnel and consultants. There is intense competition among major pharmaceutical and chemical companies, specialized biotechnology firms and universities and other research institutions for qualified personnel in the areas of our operations, and we may be unsuccessful in attracting and retaining these personnel.

We may need to expand our organization and may experience difficulties in managing this growth, which could disrupt our operations.

As of May 3, 2019, we had 40 full-time employees. As our business activities expand, we may expand our full-time employee base and hire more consultants and contractors. Our management may need to divert a disproportionate amount of its attention away from day-to-day activities and devote a substantial amount of time to managing these growth activities. We may not be able to effectively manage the expansion of our operations, which may result in weaknesses in our infrastructure, operational setbacks, loss of business opportunities, loss of employees and reduced productivity among remaining employees. Our expected growth could require significant capital expenditures and may divert financial resources from other projects, such as the development of additional product candidates. If our management is unable to effectively manage our growth, our expenses may increase more than expected, our ability to generate and/or grow revenues could be reduced and we may not be able to implement our business strategy.

Our employees, principal investigators, consultants and commercial partners may engage in misconduct or other improper activities, including non-compliance with regulatory standards and requirements and insider trading.

We are exposed to the risk of fraud or other misconduct by our employees, consultants and commercial partners. Misconduct by these parties could include intentional failures to comply with the regulations of the FDA and non-U.S. regulators, provide accurate information to the FDA and non-U.S. regulators, comply with healthcare fraud and abuse laws and regulations in the U.S. and abroad, report financial information or data accurately or disclose unauthorized activities to us. In particular, sales, marketing and business arrangements in the healthcare industry are subject to extensive laws and regulations intended to prevent fraud, misconduct, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, sales commission, customer incentive programs and other business arrangements. Such misconduct could also involve the improper use of information obtained in the course of clinical studies, which could result in regulatory sanctions and cause serious harm to our reputation or could cause regulatory agencies not to approve our product candidates. We have a code of business ethics and conduct applicable to all employees, but it is not always possible to identify and deter employee or third-party misconduct, and the precautions we take to detect and prevent this activity may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to comply with these laws or regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of significant fines or other sanctions.

Our internal computer systems, or those of our third-party collaborators or other contractors, may fail or suffer security breaches, which could result in a material disruption of our development programs.

Our internal computer systems and those of our current and any future collaborators and other consultants are vulnerable to damage from computer viruses, unauthorized access, cyberattacks, data breaches, natural disasters, terrorism, war and telecommunication and electrical failures. While we have not experienced any such material system failure, accident, attack 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, whether due to a loss of our trade secrets or other proprietary information or other similar disruptions. 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. 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, our competitive position could be harmed and the further development and commercialization of our product candidates could be delayed.

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

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

Given our commercial relationships outside of the United States, in particular in the European Union, a variety of risks associated with international operations could harm our business.

We engage in various commercial relationships outside the U.S. and we may commercialize our product candidates outside of the U.S. In many foreign countries, it is common for others to engage in business practices that are prohibited by U.S. laws and regulations applicable to us, including the Foreign Corrupt Practices Act. Although we may implement policies and procedures specifically designed to comply with these laws and policies, there can be no assurance that our employees, contractors and agents will comply with these laws and policies. If we are unable to successfully manage the challenges of international expansion and operations, our business and operating results could be harmed.

We may be, and to the extent we commercialize our product candidates outside the United States, expect to be subject to various risks associated with operating internationally, including:

- different regulatory requirements for approval of drugs and biologics in foreign countries;
- reduced protection for intellectual property rights;
- unexpected changes in tariffs, trade barriers and regulatory requirements;
- economic weakness, including inflation, or political instability in particular foreign economies and markets;
- compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- foreign currency fluctuations, which could result in increased operating expenses and reduced revenues, and other obligations incident to doing business in another country;
- workforce uncertainty in countries where labor unrest is more common than in the United States;
- shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad;
- business interruptions resulting from geopolitical actions, including war and terrorism or natural disasters including earthquakes, typhoons, floods and fires, or from economic or political instability;
- compliance with foreign laws, regulations, standards and regulatory guidance governing the collection, use, disclosure, retention, security and transfer of personal data, including the European Union General Data Privacy Regulation (“GDPR”); and
- greater difficulty with enforcing our contracts in jurisdictions outside of the United States.

These and related risks could materially harm our business, financial condition, results of operations and prospects.

Affordable Care Act and Other Reform Initiatives

In the United States and some foreign jurisdictions, there have been, and likely will continue to be, a number of legislative and regulatory changes and proposed changes regarding the healthcare system directed at broadening the availability of healthcare and containing or lowering the cost of healthcare.

By way of example, in March 2010, the ACA was enacted. The ACA includes measures that have or will significantly change the way healthcare is financed by both governmental and private insurers. Among the provisions of the ACA of greatest importance to the pharmaceutical industry are the following:

- The Medicaid Drug Rebate Program requires pharmaceutical manufacturers to enter into and have in effect a national rebate agreement with the Secretary of the U.S. Department of Health and Human Services in exchange for state Medicaid coverage of most of the manufacturer’s drugs. The ACA made several changes to the Medicaid Drug Rebate Program, including increasing pharmaceutical manufacturers’ rebate liability by raising the minimum basic Medicaid rebate on most branded prescription drugs and biologic agents to 23.1% of average manufacturer price (“AMP”) and adding a new rebate calculation for “line extensions” (i.e., new formulations, such as extended release formulations) of solid oral dosage forms of branded products, as well as potentially impacting their rebate liability by modifying the statutory definition of AMP.
- The ACA expanded the types of entities eligible to receive discounted 340B pricing, although, with the exception of children’s hospitals, these newly eligible entities will not be eligible to receive discounted 340B pricing on orphan drugs used in orphan indications. In addition, because 340B pricing is determined based on AMP and Medicaid drug rebate data, the revisions to the Medicaid rebate formula and AMP definition described above could cause the required 340B discounts to increase. The ACA imposed a requirement on manufacturers of branded drugs and biologic agents to provide a 50% discount off the negotiated price of branded drugs dispensed to Medicare Part D beneficiaries in the coverage gap (i.e., “donut hole”).

- The ACA imposed 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, although this fee would not apply to sales of certain products approved exclusively for orphan indications.
- The ACA included the Federal Physician Payments Sunshine Act, which requires certain pharmaceutical manufacturers of drugs, devices, biologics and medical supplies for which payment is available under Medicare, Medicaid, or the Children's Health Insurance Program, with specific exception, to track certain financial arrangements with physicians and teaching hospitals, including any "transfer of value" provided, as well as any ownership or investment interests held by physicians and their immediate family members. Covered manufacturers were required to begin collecting data on August 1, 2013 and submit reports on aggregate payment data to CMS for the first reporting period (August 1, 2013—December 31, 2013) by March 31, 2014, and were required to report detailed payment data for the first reporting period and submit legal attestation to the completeness and accuracy of such data by June 30, 2014. Thereafter, covered manufacturers must submit reports by the 90th day of each subsequent calendar year. The information reported was made publicly available on a searchable website in September 2014.
- The ACA established a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research. The research conducted by the Patient-Centered Outcomes Research Institute may affect the market for certain pharmaceutical products.
- The ACA created the Independent Payment Advisory Board which has the authority to recommend certain changes to the Medicare program to reduce expenditures by the program that could result in reduced payments for prescription drugs. Under certain circumstances, these recommendations will become law unless Congress enacts legislation that will achieve the same or greater Medicare cost savings.
- The ACA established the Center for Medicare and Medicaid Innovation within CMS to test innovative payment and service delivery models to improve quality of care and lower program costs of Medicare, Medicaid and the Children's Health Insurance Program, potentially including prescription drug spending. Funding has been allocated to support the mission of the Center for Medicare and Medicaid Innovation through 2019.

Many of the details regarding the implementation of the ACA are yet to be determined, and at this time, it remains unclear the full effect that the ACA will have on our business.

Other legislative changes have been proposed and adopted in the United States since the ACA was enacted. For example, in August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least $1.2 trillion for the years 2012 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers up to 2% per fiscal year, which went into effect in April 2013 and will remain in effect through 2024 unless additional Congressional action is taken. 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, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors, which may adversely affect our future profitability.

European Union Drug Development

In the EU, our products will also be subject to extensive regulatory requirements. As in the United States, medicinal products can only be marketed if a marketing authorization application ("MAA") from the competent regulatory agencies has been obtained, and the various phases of preclinical and clinical research in the EU are subject to significant regulatory controls. Although the EU Clinical Trials Directive 2001/20/EC has sought to harmonize the EU clinical trial regulatory framework, setting out common rules for the control and authorization of clinical trials in the EU, the EU Member States have transposed and applied the provisions of the Directive differently. This has led to significant variations in the member state regimes. Under the current regime, before a clinical trial can be initiated it must be approved by two distinct bodies in each of the EU countries where the trial is to be conducted: the National Competent Authority ("NCA") and one or more Ethics Committees ("ECs"). In addition, all serious adverse reactions to the investigated drug that occur during the clinical trial must be reported to the NCA and ECs of the Member State where they occurred.

The EU clinical trials legislation is currently undergoing a revision process mainly aimed at making more uniform and streamlining the clinical trials authorization process, simplifying adverse event reporting procedures, improving the supervision of clinical trials and increasing the transparency of clinical trials.

European Union Drug Review Approval

In the European Economic Area ("EEA"), which is comprised of the 28 Member States of the EU plus Norway, Iceland and Liechtenstein, medicinal products can only be commercialized after obtaining an MAA. There are two types of MAAs: (1) the Community MAA, which is issued by the European Commission through the Centralized Procedure based on the opinion of the Committee for Medicinal Products for Human Use, a body of the EMA, and which is valid throughout the entire territory of the EEA; and (2) the National MAA, which is issued by the competent authorities of the Member States of the EEA and only authorized marketing in that Member State's national territory and not the EEA as a whole.

The Centralized Procedure is mandatory for certain types of products, such as biotechnology medicinal products, orphan medicinal products and medicinal products containing a new active substance indicated for the treatment of AIDS, cancer, neurodegenerative disorders, diabetes, auto-immune and viral diseases. The Centralized Procedure is optional for products containing a new active substance not yet authorized in the EEA, or for products that constitute a significant therapeutic, scientific or technical innovation or which are in the interest of public health in the EU. The National MAA is for products not falling within the mandatory scope of the Centralized Procedure. Where a product has already been authorized for marketing in a Member State of the EEA, this National MAA can be recognized in another Member States through the Mutual Recognition Procedure. If the product has not received a National MAA in any Member State at the time of application, it can be approved simultaneously in various Member States through the Decentralized Procedure. Under the Decentralized Procedure an identical dossier is submitted to the competent authorities of each of the Member States in which the MAA is sought, one of which is selected by the applicant as the Reference Member State ("RMS"). If the RMS proposes to authorize the product, and the other Member States do not raise objections, the product is granted a national MAA in all the Member States where the authorization was sought. Before granting the MAA, the EMA or the competent authorities of the Member States of the EEA make an assessment of the risk-benefit balance of the product on the basis of scientific criteria concerning its quality, safety and efficacy.

In addition, in the EU, the EMA's CAT is responsible for assessing the quality, safety and efficacy of advanced therapy medicinal products. The role of the CAT is to prepare a draft opinion on an application for marketing authorization for a gene therapy medicinal candidate that is submitted to the EMA. The development and evaluation of a gene therapy medicinal product must be considered in the context of the relevant EU guidelines, and the EMA may issue new guidelines concerning the development and marketing authorization for gene therapy medicinal products and require that we comply with these new guidelines.

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

The United States and many foreign jurisdictions have enacted or proposed legislative and regulatory changes affecting the healthcare system that could prevent or delay marketing approval of our product candidates or any future product candidates, restrict or regulate post-approval activities and affect our ability to profitably sell any product for which we obtain marketing approval. Changes in regulations, statutes or the interpretation of existing regulations could impact our business in the future by requiring, for example: (i) changes to our manufacturing arrangements; (ii) additions or modifications to product labeling; (iii) the recall or discontinuation of our products; or (iv) additional record-keeping requirements. If any such changes were to be imposed, they could adversely affect the operation of our business.

In the United States, there have been and continue to be a number of legislative initiatives to contain healthcare costs. For example, in March 2010, the Patient Protection and Affordable Care Act, as amended by the Health Care and Education Reconciliation Act of 2010, or the Affordable Care Act, was passed, which substantially changed the way health care is financed by both governmental and private insurers, and significantly impacts the U.S. pharmaceutical industry. Since January 2017, the Trump administration has signed two Executive Orders designed to delay the implementation of certain provisions of the Affordable Care Act or otherwise circumvent some of the requirements for health insurance mandated by the Affordable Care Act. One Executive Order directs federal agencies with authorities and responsibilities under the Affordable Care Act to waive, defer, grant exemptions from, or delay the implementation of any provision of the Affordable Care Act that would impose a fiscal or regulatory burden on states, individuals, healthcare providers, health insurers, or manufacturers of pharmaceuticals or medical devices. The second Executive Order terminates the cost-sharing subsidies that reimburse insurers under the Affordable Care Act. Several state Attorneys General filed suit to stop the administration from terminating the subsidies, but their request for a restraining order was denied by a federal judge in California on October 25, 2017. Further, on June 14, 2018, U.S. Court of Appeals for the Federal Circuit ruled that the federal government was not required to pay more than $12.0 billion in Affordable Care Act risk corridor payments to third-party payors who argued were owed to them. The effects of this gap in reimbursement on third-party payors, the viability of the Affordable Care Act marketplace, providers, and potentially our business, are not yet known.

In July 2018, the CMS published a final rule permitting further collections and payments to and from certain Affordable Care Act-qualified health plans and health insurance issuers under the Affordable Care Act risk adjustment program in response to the outcome of federal district court litigation regarding the method CMS uses to determine this risk adjustment. In addition, CMS has recently published a final rule that would give states greater flexibility, starting in 2020, in setting benchmarks for insurers in the individual and small group marketplaces, which may have the effect of relaxing the essential health benefits required under the Affordable Care Act for plans sold through such marketplaces. On December 14, 2018, a U.S. District Court Judge in the Northern District of Texas, or the Texas District Court Judge, ruled that the individual mandate is a critical and inseverable feature of the Affordable Care Act, and therefore, because it was repealed as part of the Tax Cuts and Jobs Act of 2017, the remaining provisions of the Affordable Care Act are invalid as well. While the Trump Administration and CMS have both stated that the ruling will have no immediate effect, and on December 30, 2018 the Texas District Court Judge issued an order staying the judgment pending appeal, it is unclear how this decision, subsequent appeals and other efforts to repeal and replace the Affordable Care Act will impact the Affordable Care Act and our business.

Since its enactment, some of the provisions of the Affordable Care Act have yet to be fully implemented, while certain provisions have been subject to judicial, congressional, or executive challenges. As a result, there have been delays in the implementation of, and action taken to repeal or replace, certain aspects of the Affordable Care Act. The U.S. Supreme Court has upheld certain key aspects of the legislation, including a tax-based shared responsibility payment imposed on certain individuals who fail to maintain qualifying health coverage for all or part of a year or pay a penalty, which is commonly known as the "individual mandate." However, as a result of tax reform legislation passed in December 2017, the individual mandate has been eliminated effective January 1, 2019. On January 22, 2018, President Trump signed a continuing resolution on appropriations for fiscal year 2018 that delayed the implementation of certain Affordable Care Act-mandated fees, including the so-called "Cadillac" tax on certain high cost employer-sponsored insurance plans, the annual fee imposed on certain health insurance providers based on market share, and the medical device excise tax on non-exempt medical devices. The Bipartisan Budget Act of 2018, or the BBA, among other things, amends the Affordable Care Act, effective January 1, 2019, to close the coverage gap in most Medicare drug plans, commonly referred to as the "donut hole."

In addition, other legislative changes have been proposed and adopted since the Affordable Care Act was enacted. 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 to Congress proposals in spending reductions. The Joint Select Committee on Deficit Reduction did not achieve a targeted deficit reduction, which triggered the legislation's automatic reduction to several government programs. This includes aggregate reductions to Medicare payments to providers of, on average, 2% per fiscal year through 2025 unless Congress takes additional action. These reductions were extended through 2027 under the BBA. In January 2013, the American Taxpayer Relief Act of 2012, among other things, further reduced Medicare payments to several providers, including hospitals and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

There has been increasing legislative and enforcement interest in the United States with respect to specialty drug pricing practices. Specifically, there have been several recent U.S. Congressional inquiries and proposed federal and state legislation designed to, among other things, bring more transparency to drug pricing, reduce the cost of prescription drugs under Medicare, 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 2019 contains further drug price control measures that could be enacted during the 2019 budget process or in other future legislation, including, for example, measures to permit Medicare Part D plans to negotiate the price of certain drugs under Medicare Part B, to allow some states to negotiate drug prices under Medicaid, and to eliminate cost sharing for generic drugs for low-income patients. Additionally, the Trump administration released a "Blueprint" to lower drug prices and reduce out of pocket costs of drugs that contains additional proposals to increase manufacturer competition, increase the negotiating power of certain federal healthcare programs, incentivize manufacturers to lower the list price of their products and reduce the out of pocket costs of drug products paid by consumers. The U.S. Department of Health and Human Services, or HHS, has already started the process of soliciting feedback on some of these measures and, at the same time, is immediately implementing others under its existing authority. For example, in September 2018, CMS announced that it will allow Medicare Advantage Plans the option to use step therapy for Part B drugs beginning January 1, 2019, and in October 2018, CMS proposed a new rule that would require direct-to-consumer television advertisements of prescription drugs and biological products, for which payment is available through or under Medicare or Medicaid, to include in the advertisement the Wholesale Acquisition Cost, or list price, of that drug or biological product. Although a number of these, and other proposed measures will require authorization through additional legislation to become effective, Congress and the Trump administration have each indicated that it will continue to seek new legislative and/or administrative measures to control drug costs. At the state level, legislatures have increasingly passed legislation and implemented regulations designed to control pharmaceutical and biological product pricing, including price or patient reimbursement constraints, discounts, restrictions on certain product access and marketing cost disclosure and transparency measures, and, in some cases, designed to encourage importation from other countries and bulk purchasing.

We expect that the healthcare reform measures that have been adopted and may be adopted in the future, may result in more rigorous coverage criteria and in additional downward pressure on the price that we receive for any approved product and could seriously harm our future revenues. Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private third-party payors.

The delivery of healthcare in the European Union, including the establishment and operation of health services and the pricing and reimbursement of medicines, is almost exclusively a matter for national, rather than EU, law and policy. National governments and health service providers have different priorities and approaches to the delivery of health care and the pricing and reimbursement of products in that context. In general, however, the healthcare budgetary constraints in most EU member states have resulted in restrictions on the pricing and reimbursement of medicines by relevant health service providers. Coupled with ever-increasing EU and national regulatory burdens on those wishing to develop and market products, this could prevent or delay marketing approval of our product candidates, restrict or regulate post-approval activities and affect our ability to commercialize any products for which we obtain marketing approval.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our product. Such reforms could have an adverse effect on anticipated revenue from product candidates that we may successfully develop and for which we may obtain regulatory approval and may affect our overall financial condition and ability to develop product candidates.

Other Regulations

We are also subject to numerous federal, state and local laws relating to such matters as safe working conditions, manufacturing practices, environmental protection, fire hazard control and disposal of hazardous or potentially hazardous substances. We may incur significant costs to comply with such laws and regulations now or in the future.

If we fail to comply with applicable U.S. and foreign privacy and data protection laws and regulations, we may be subject to liabilities that adversely affect our business, operations and financial performance.

We may also be subject to or affected by foreign laws and regulation, including regulatory guidance, governing the collection, use, disclosure, security, transfer and storage of personal data, such as information that we collect about patients and healthcare providers in connection with clinical trials and our other operations in the U.S. and abroad. For example, the E.U. has adopted the GDPR, which introduces strict requirements for processing personal data. The GDPR is likely to increase the compliance burden on us, including by mandating potentially burdensome documentation requirements and granting certain rights to individuals to control how we collect, use, disclose, retain and leverage information about them. The processing of sensitive personal data, such as physical health conditions, may impose heightened compliance burdens under the GDPR and is a topic of active interest among foreign regulators. In addition, the GDPR provides for breach reporting requirements, more robust regulatory enforcement and fines of up to 20 million euros or up to 4% of annual global revenue. While the GDPR affords some flexibility in determining how to comply with the various requirements, significant effort and expense has been, and will continue to be, invested to ensure continuing compliance. Moreover, the requirements under the GDPR may change periodically or may be modified by EU national law and could have an effect on our business operations if compliance becomes substantially more costly than under current requirements.

The global legislative and regulatory landscape for privacy and data protection continues to evolve, and implementation standards and enforcement practices are likely to remain uncertain for the foreseeable future. This evolution may create uncertainty in our business, result in liability or impose additional costs on us. The cost of compliance with these laws, regulations and standards is high and is likely to increase in the future.

It is possible that each of these privacy laws may be interpreted and applied in a manner that is inconsistent with our practices. Any failure or perceived failure by us to comply with federal, state, or foreign laws or self-regulatory standards could result in negative publicity, diversion of management time and effort and proceedings against us by governmental entities or others. In many jurisdictions, enforcement actions and consequences for noncompliance are rising. As we continue to expand into other foreign countries and jurisdictions, we may be subject to additional laws and regulations that may affect how we conduct business.

Risks Related to Our Intellectual Property

Our rights to intellectual property for the development and commercialization of our product candidates are subject to the terms and conditions of licenses granted to us by others.

We are heavily reliant upon licenses to certain patent rights and proprietary technology from third parties that are important or necessary to the development of our technology and products, including technology related to our manufacturing process and our gene therapy product candidates. These and other licenses may not provide exclusive rights to use such intellectual property and technology in all relevant fields of use and in all territories in which we may wish to license our platform or develop or commercialize our technology and products in the future. As a result, we may not be able to prevent competitors from developing and commercializing competitive products in territories not included in all of our licenses.

Licenses to additional third-party technology that may be required for our licensing or development programs may not be available in the future or may not be available on commercially reasonable terms, or at all, which could materially harm our business and financial condition.

In some circumstances, we may not have the right to control the preparation, filing and prosecution of patent applications, or to maintain or enforce the patents, covering technology that we license from third parties. If our licensors fail to maintain such patents, or lose rights to those patents or patent applications, the rights we have licensed may be reduced or eliminated and our right to develop and commercialize any of our products that are the subject of such licensed rights could be impacted. In addition to the foregoing, the risks associated with patent rights that we license from third parties will also apply to patent rights we may own in the future.

Furthermore, the research resulting in certain of our licensed patent rights and technology was funded by the U.S. government. As a result, the government may have certain rights, or march-in rights, to such patent rights and technology. When new technologies are developed with government funding, the government generally obtains certain rights in any resulting patents, including a non-exclusive license authorizing the government to use the invention for non-commercial purposes. These rights may permit the government to disclose our confidential information to third parties and to exercise march-in rights to use or allow third parties to use our licensed technology. The government can exercise its march-in rights if it determines that action is necessary because we fail to achieve practical application of the government-funded technology, because action is necessary to alleviate health or safety needs, to meet requirements of federal regulations or to give preference to U.S. industry. In addition, our rights in such inventions may be subject to certain requirements to manufacture products embodying such inventions in the U.S. Any exercise by the government of such rights could harm our competitive position, business, financial condition, results of operations and prospects.

If we are unable to obtain and maintain patent protection for products and related technology, or if the scope of the patent protection obtained is not sufficiently broad, our competitors could develop and commercialize products and technology similar or identical to ours, and our ability to successfully commercialize our products may be harmed.

Our success depends, in large part, on our ability to obtain and maintain patent protection in the U.S. and other countries with respect to our product candidates and our manufacturing technology. Our licensors have sought, and we may intend to seek, to protect our proprietary position by filing patent applications in the U.S. and abroad related to many of our novel technologies and product candidates that are important to our business.

The patent prosecution process is expensive, time-consuming and complex, and we may not be able to file, prosecute, maintain, enforce or license all necessary or desirable patent applications at a reasonable cost or in a timely manner. In addition, certain patents in the field of gene therapy that may have otherwise potentially provided patent protection for certain of our product candidates may expire prior to commercial launch of our products; this patent expiration risk could be partially addressed by pursuing and receiving 10 years Biologics regulatory exclusivity from the FDA, which would grant protection in later years where patent expiration may not exist. In some cases, the work of certain academic researchers in the gene therapy field has entered the public domain, which we believe precludes our ability to obtain patent protection for certain inventions relating to such work. It is also possible that we will fail to identify patentable aspects of our research and development output before it is too late to obtain patent protection.

We are party to intellectual property license agreements with several entities, each of which is important to our business, and we expect to enter into additional license agreements in the future. Our patent portfolio consists primarily of patent applications in-licensed pursuant to those license agreements, and those agreements impose, and we expect that future license agreements will impose various diligence, development and commercialization timelines, milestone obligations, payments and other obligations on us. If we or our licensees fail to comply with our obligations under these agreements, or we are subject to a bankruptcy, the licensor may have the right to terminate the license, in which event we could lose certain rights provided by the licenses, including that we may not be able to market products covered by the license.

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. As a result, the issuance, scope, validity, enforceability and commercial value of our patent rights are highly uncertain. Pending and future patent applications may not result in patents being issued which protect our technology or product candidates or which effectively prevent others from commercializing competitive technologies and product candidates. Changes in either the patent laws or interpretation of the patent laws in the U.S. and other countries may diminish the value of our patent rights or narrow the scope of our patent protection.

While we believe our intellectual property allows us to pursue our current development programs, several companies and academic institutions are pursuing alternate approaches to gene therapy and have built intellectual property around these approaches and methods. In addition, we may not be aware of all third-party intellectual property rights potentially relating to our technology and product candidates. Publications of discoveries in the scientific literature often lag the actual discoveries, and patent applications in the U.S. and other jurisdictions are typically not published until 18 months after filing or, in some cases, not at all. Therefore, we cannot be certain that we were the first to make the inventions claimed in any owned or any licensed patents or pending patent applications, or that we were the first to file for patent protection of such inventions.

Even if the patent applications we license or may own in the future do issue as patents, they may not issue in a form that will provide us with any meaningful protection, prevent competitors or other third parties from competing with us or otherwise provide us with any competitive advantage. Our competitors or other third parties may avail themselves of safe harbor under the Drug Price Competition and Patent Term Restoration Act of 1984 (Hatch-Waxman Amendments) to conduct research and clinical trials and may be able to circumvent our patent rights 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 patent rights may be challenged in the courts or patent offices in the U.S. and abroad. Such challenges may result in loss of exclusivity or in patent claims being narrowed, invalidated or held unenforceable, 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 is technology and product candidates. 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 intellectual property may not provide sufficient rights to exclude others from commercializing products similar or identical to ours.

If we breach our license agreements, it could have a material adverse effect on our commercialization efforts for our product candidates.

If we breach any of the agreements under which we license intellectual property relating to the use, development and commercialization rights to our product candidates or technology from third parties, we could lose license rights that are important to our business. Licensing of intellectual property is of critical importance to our business and involves complex legal, business and scientific issues. Disputes may arise between us and our licensors regarding intellectual property subject to a license agreement, including:

- the scope of rights granted under the license agreement;
- whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- our right to sublicense patent and other intellectual property rights to third parties under collaborative development relationships;
- our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of is product candidates, and what activities satisfy those diligence obligations;
- the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners; and
- whether and the extent to which inventors are able to contest to the assignment of their rights to our licensors.

If disputes over intellectual property that we have in-licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates. In addition, if disputes arise as to ownership of licensed intellectual property, our ability to pursue or enforce the licensed patent rights may be jeopardized. If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize our products could suffer.

We may incur substantial costs as a result of litigation or other proceedings relating to patent and other intellectual property rights and we may be unable to protect our rights to, or use, our technology.

If we choose to engage in legal action to prevent a third-party from using the inventions claimed in our patents or patents which we license, that third-party has the right to ask the court to rule that these patents are invalid and/or should not be enforced against that third-party. These lawsuits are expensive and would consume time and other resources even if we were successful in stopping the infringement of these patents. In addition, there is a risk that the court will decide that these patents are not valid and that we do not have the right to stop the other party from using the inventions. There is also the risk that, even if the validity of these patents is upheld, the court will refuse to stop the other party on the ground that such other party's activities do not infringe our rights to these patents.

Furthermore, a third-party may claim that we are using inventions covered by the third-party's patent rights and may go to court to stop us from engaging in our normal operations and activities, including making or selling our product candidates. These lawsuits are costly and could affect our results of operations and divert the attention of managerial and technical personnel. There is a risk that a court would decide that we are infringing the third-party's patents and would order us to stop the activities covered by the patents. In addition, there is a risk that a court will order us to pay the other party damages for having violated the other party's patents. The biotechnology industry has produced a proliferation of patents, and it is not always clear to industry participants which patents cover various types of products or methods of use. The coverage of patents is subject to interpretation by the courts, and the interpretation is not always uniform. If we are sued for patent infringement, we would need to demonstrate that our products or methods of use either do not infringe the patent claims of the relevant patent and/or that the patent claims are invalid. Proving invalidity, in particular, is difficult since it requires a showing of clear and convincing evidence to overcome the presumption of validity enjoyed by issued patents. Our competitors have filed, and may in the future file, patent applications covering technology similar to ours. Any such patent application may have priority over our in-licensed patent applications and could further require us to obtain rights to issued patents covering such technologies. If another party has filed a U.S. patent application on inventions similar to ours, we may have to participate in an interference proceeding declared by the U.S. Patent and Trademark Office, to determine priority of invention in the U.S. The costs of these proceedings could be substantial, and it is possible that such efforts would be unsuccessful, resulting in a loss of our United States patent position with respect to such inventions.

Some of our competitors may be able to sustain the costs of complex patent litigation more effectively than we can because they have substantially greater resources. In addition, any uncertainties resulting from the initiation and continuation of any litigation could have a material adverse effect on our ability to raise the funds necessary to continue our operations.

If we are unable to protect the confidentiality of our trade secrets, our business and competitive position may be harmed.

In addition to the protection afforded by patents, we rely upon unpatented trade secret protection, unpatented know-how and continuing technological innovation to develop and maintain our competitive position. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our contractors, collaborators, employees and consultants. Nonetheless, we may not be able to prevent the unauthorized disclosure or use of our technical know-how or other trade secrets by the parties to these agreements, however, despite the existence generally of confidentiality agreements and other contractual restrictions. Monitoring unauthorized uses and disclosures is difficult and we do not know whether the steps we have taken to protect our proprietary technologies will be effective. If any of the contractors, collaborators, employees and consultants who are parties to these agreements breaches or violates the terms of any of these agreements, we may not have adequate remedies for any such breach or violation. As a result, we could lose our trade secrets. Enforcing a claim that a third-party illegally obtained and is using our trade secrets, like patent litigation, is expensive and time consuming and the outcome is unpredictable. In addition, courts outside the United States are sometimes less willing or unwilling to protect trade secrets.

Our trade secrets could otherwise become known or be independently discovered by our competitors. Competitors could purchase our product candidates and attempt to replicate some or all of the competitive advantages we derive from our development efforts, willfully infringe our intellectual property rights, design around our protected technology or develop their own competitive technologies that fall outside of our intellectual property rights. If any of our trade secrets 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 our trade secrets are not adequately protected or sufficient to provide an advantage over our competitors, our competitive position could be adversely affected, as could our business. Additionally, if the steps taken to maintain our trade secrets are deemed inadequate, we may have insufficient recourse against third parties for misappropriating our trade secrets.

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

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

If the patent applications we hold or have in-licensed with respect to our programs or product candidates fail to issue, if their breadth or strength of protection is threatened, or if they fail to provide meaningful exclusivity for our product candidates, it could dissuade companies from collaborating with us to develop product candidates, and threaten our ability to commercialize, future products. In addition to our existing patent application filings, we expect to continue to file additional patent applications covering our product candidates. Further, we intend to pursue additional activities to protect the patents, trade secrets and other intellectual property covering our product candidates. We cannot offer any assurances about which, if any, patents will issue, the breadth of any such patent or whether any issued patents will be found invalid and unenforceable or will be threatened by third parties. Any successful opposition to these patents or any other patents owned by or licensed to us could deprive us of rights necessary for the successful commercialization of any product candidates that we may develop. Further, if we or the relevant licensor encounter delays in regulatory approvals, the period of time during which we could market a product candidate under patent protection could be reduced. Since patent applications in the United States and most other countries are confidential for a period of time after filing, and some remain so until issued, we cannot be certain that we or the relevant licensor were the first to file any patent application related to a product candidate. Furthermore, if third parties have filed such patent applications, an interference proceeding in the United States can be initiated by a third-party to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. In addition, patents have a limited lifespan. In the United States, the natural expiration of a patent is generally 20 years after it is filed. Various extensions may be available however the life of a patent, and the protection it affords, is limited. Even if patents covering our product candidates are obtained, once the patent life has expired for a product, we may be open to competition from generic medications.

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

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

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

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

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

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents or patent applications with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover the manufacturing process of any of our product candidates, any molecules formed during the manufacturing process or any final product itself, the holders of any such patents may be able to block our ability to commercialize such product candidate unless we obtained a license under the applicable patents, or until such patents expire. Similarly, if any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations, processes for manufacture or methods of use, including combination therapy, the holders of any such patents may be able to block our ability to develop and commercialize the applicable product candidate unless we obtained a license or until such patent expires. In either case, such a license may not be available on commercially reasonable terms or at all. For example, Institute Pasteur controls a patent family related to vector elements for lentiviral-based gene therapy. These patents relate to an element that improves nuclear localization. While these patents expire from 2019 to 2023, if our products were to launch before these dates, we may need to secure a license.

Parties making claims against us may obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize one or more of our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, pay royalties, redesign our infringing products or obtain one or more licenses from third parties, which may be impossible or require substantial time and monetary expenditure.

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

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

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

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

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

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

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

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

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

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

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

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that a patent of ours or our licensors is not valid, is unenforceable and/or is not infringed, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated or interpreted narrowly and could put our patent applications at risk of not issuing.

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

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

Patent reform legislation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents.

On September 16, 2011, the Leahy-Smith America Invents Act (the "Leahy-Smith Act"), was signed into law. The Leahy-Smith Act includes a number of significant changes to U.S. patent law, including provisions that affect the way patent applications will be prosecuted and may also affect patent litigation. The U.S. PTO is currently developing regulations and procedures to govern administration of the Leahy-Smith Act, and many of the substantive changes to patent law associated with the Leahy-Smith Act, and in particular, the first to file provisions, were enacted March 16, 2013. It is not clear what, if any, impact the Leahy-Smith Act will have on the operation of our business. However, the Leahy-Smith Act and its implementation could increase the uncertainties and costs surrounding the prosecution of our patent applications and the enforcement or defense of our issued patents, all of which could have a material adverse effect on our business and financial condition.

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

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

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

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

Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court.

If we or one of our licensing partners initiated legal proceedings against a third-party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace. Grounds for a validity challenge could be an alleged failure to meet any of several statutory requirements, including patent eligible subject matter, lack of novelty, obviousness or non-enablement. Grounds for an unenforceability assertion could be an allegation that someone connected with prosecution of the patent withheld relevant information from the U.S. PTO, or made a misleading statement, during prosecution. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, and equivalent proceedings in foreign jurisdictions (e.g., opposition proceedings). Such proceedings could result in revocation or amendment to our or our licensing partners' patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art, of which we and the patent examiner were unaware during prosecution. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patent protection on our product candidates. Such a loss of patent protection would have a material adverse impact on our business.

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

As is the case with other biotechnology companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biotechnology industry involves both technological and legal complexity, and therefore obtaining and enforcing biotechnology patents is costly, time-consuming and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the U.S. PTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that it might obtain in the future.

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

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

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

Risks related to ownership of our common stock

A significant number of our total outstanding shares may be sold into the market in the near future, which could cause the market price of our common stock to drop significantly, even if our business is performing well.

Sales of a substantial number of shares of our common stock in the public market could occur at any time. These sales, or the perception or the perception that such sales may occur, could reduce the market price of our common stock. Our outstanding shares of common stock may be freely sold in the public market at any time to the extent permitted by Rules 144 and 701 under the Securities Act of 1933, as amended (the “Securities Act”), or to the extent such shares have already been registered under the Securities Act and are held by non-affiliates of ours. In addition, certain of our employees, executive officers, directors and affiliated stockholders have entered or may enter into Rule 10b5-1 plans providing for sales of shares of our common stock from time to time. Under a Rule 10b5-1 plan, a broker executes trades pursuant to parameters established by the employee, director or officer when entering into the plan, without further direction from the employee, officer, director or affiliated stockholder. A Rule 10b5-1 plan may be amended or terminated in some circumstances. Our employees, executive officers, directors and affiliated stockholders also may buy or sell additional shares outside of a Rule 10b5-1 plan when they are not in possession of material, nonpublic information.

If securities analysts do not publish research or reports about our business or if they publish negative evaluations of our stock, the price of our stock could decline.

The trading market for our common stock relies, in part, on the research and reports that industry or financial analysts publish about us or our business. Although we have obtained analyst coverage, if one or more of the analysts covering our business downgrade their evaluations of our stock, the price of our stock could decline. If one or more of these analysts cease to cover our stock or fail to regularly publish reports on us, we could lose visibility in the market for our stock, which in turn could cause our stock price to decline.

The price of our common stock may be volatile and fluctuate substantially, which could result in substantial losses for our stockholders.

Our stock price is likely to be volatile. The stock market in general, and the market for biopharmaceutical companies in particular, has experienced extreme volatility that has often been unrelated to the operating performance of particular companies. As a result of this volatility, our stockholders may not be able to sell their shares of common stock at or above the price they paid for their shares. The market price for our common stock may be influenced by many factors, including:

- results of clinical trials of our product candidates or those of our competitors;
- the success of competitive products or technologies;
- commencement or termination of collaborations;
- regulatory or legal developments in the United States and other countries;
- developments or disputes concerning patent applications, issued patents or other proprietary rights;
- the recruitment or departure of key personnel;
- the level of expenses related to any of our product candidates or clinical development programs;
- the results of our efforts to discover, develop, acquire or in-license additional product candidates;
- actual or anticipated changes in estimates as to financial results, development timelines or recommendations by securities analysts;
- negative publicity around gene therapy in general, or our product candidates;
- variations in our financial results or those of companies that are perceived to be similar to us;
- changes in the structure of healthcare payment systems;
- market conditions in the pharmaceutical and biotechnology sectors; and
- general economic, industry and market conditions.

If our quarterly operating results fall below the expectations of investors or securities analysts, the price of our common stock could decline substantially. Furthermore, any quarterly fluctuations in our operating results may, in turn, cause the price of our stock to fluctuate substantially. We believe that quarterly comparisons of our financial results are not necessarily meaningful and should not be relied upon as an indication of our future performance.

In the past, following periods of volatility in the market price of a company's securities, securities class-action litigation often has been instituted against that company. Such litigation, if instituted against us, could cause us to incur substantial costs to defend such claims and divert management's attention and resources, which could seriously harm our business, financial condition, results of operations and prospects.

RTW Investments, LP, our principal stockholder, may have the ability to significantly influence all matters submitted to stockholders for approval.

RTW Investments, LP ("RTW"), in the aggregate, beneficially owns approximately 36.50% of our outstanding shares of common stock. This concentration of voting power gives RTW the power to significantly influence all matters submitted to our stockholders for approval, as well as our management and affairs. For example, RTW could significantly influence the election of directors and approval of any merger, consolidation or sale of all or substantially all of our assets.

Because we do not anticipate paying any cash dividends on our capital stock in the foreseeable future, capital appreciation, if any, will be stockholders' sole source of gain.

We have never declared or paid cash dividends on our capital stock. We currently intend to retain all of our future earnings, if any, to finance the growth and development of our business. In addition, the terms of any future debt agreements may preclude us from paying dividends. As a result, capital appreciation, if any, of our common stock will be stockholders' sole source of gain for the foreseeable future.

If we are unable to implement and maintain effective internal control over financial reporting in the future, investors may lose confidence in the accuracy and completeness of our financial reports and the market price of our common stock may decline.

As a public company, we will be required to maintain internal control over financial reporting and to report any material weaknesses in such internal control. Once we are no longer an emerging growth company, we will be required to furnish a report by management on the effectiveness of our internal control over financial reporting pursuant to Section 404 of the Sarbanes-Oxley Act. We are in the process of designing, implementing, and testing the internal control over financial reporting required to comply with this obligation, which process is time consuming, costly, and complicated. In addition, our independent registered public accounting firm will be required to attest to the effectiveness of our internal control over financial reporting beginning with our annual report on Form 10-K following the date on which we are no longer an "emerging growth company." If we identify material weaknesses in our internal control over financial reporting, if we are unable to comply with the requirements of Section 404 in a timely manner or assert that our internal control over financial reporting is effective, or if our independent registered public accounting firm is unable to express an opinion as to the effectiveness of our internal control over financial reporting when required, investors may lose confidence in the accuracy and completeness of our financial reports and the market price of our common stock could be negatively affected, and we could become subject to investigations by the stock exchange on which our securities are listed, the Securities and Exchange Commission, or the SEC, or other regulatory authorities, which could require additional financial and management resources.

We are an "emerging growth company," and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.

We are an "emerging growth company," as defined in the Jumpstart Our Business Startups Act, or the JOBS Act. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in our periodic reports and proxy statements and exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company until December 31, 2020, although circumstances could cause us to lose that status earlier, including if the market value of our common stock held by non-affiliates exceeds $700 million as of any June 30 before that time, in which case we would no longer be an emerging growth company as of the following December 31. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds
None.

Item 3. Defaults Upon Senior Securities
None.

Item 4. Mine Safety Disclosures
Not applicable.

Item 5. Other Information
None.

Item 6. Exhibits

Exhibit Number	Description of Exhibit
2.1	Agreement and Plan of Merger and Reorganization, dated as of September 12, 2017, by and among Inotek Pharmaceuticals Corporation, Rocket Pharmaceuticals, Ltd. and Rome Merger Sub (1)
3.1	Seventh Amended and Restated Certificate of Incorporation of Rocket Pharmaceuticals, Inc., effective as of February 23, 2015 (2)
3.2	Certificate of Amendment (Reverse Stock Split) to the Seventh Amended and Restated Certificate of Incorporation of the Registrant, effective as of January 4, 2018 (3)
3.3	Certificate of Amendment (Name Change) to the Seventh Amended and Restated Certificate of Incorporation of the Registrant, effective January 4, 2018 (3)
3.4	Certificate of Amendment to the Seventh Amended and Restated Certificate of Incorporation of the Registrant, effective as of June 25, 2018. (4)
3.5	Amended and Restated By-Laws of Rocket Pharmaceuticals, Inc., effective as of March 29, 2018 (5)
4.1	Form of Common Stock Certificate of Rocket Pharmaceuticals, Inc. (3)
4.2	Base Indenture, dated as of August 5, 2016, by and between Inotek Pharmaceuticals Corporation and Wilmington Trust, National Association (6)
4.3	First Supplemental Indenture, dated as of August 5, 2016, by and between Inotek Pharmaceuticals Corporation and Wilmington Trust, National Association (6)
4.4	Form of 5.75% Convertible Senior Note due 2021 (6)
31.1*	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of 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 Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1*	Certification 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	XBRL Instance Document.
101.SCH	XBRL Taxonomy Extension Schema Document.
101.CAL	XBRL Taxonomy Extension Calculation Document.
101.DEF	XBRL Taxonomy Extension Definition Linkbase Document.
101.LAB	XBRL Taxonomy Extension Labels Linkbase Document.
101.PRE	XBRL Taxonomy Extension Presentation Link Document.

* Filed herewith.

(1) Filed as an Exhibit to the Company's current report on Form 8-K (001-36829), filed with the SEC on September 13, 2017, and incorporated herein by reference.

(2) Filed as an Exhibit to the Company's annual report on Form 10-K (001-36829), filed with the SEC on March 31, 2015, and incorporated herein by reference.

(3) Filed as an Exhibit to the Company's current report on Form 8-K (001-36829), filed with the SEC on January 5, 2018, and incorporated herein by reference.

(4) Filed as an Exhibit to the Company's current report on Form 8-K (001-36829), filed with the SEC on June 25, 2018, and incorporated herein by reference.

(5) Filed as an Exhibit to the Company's registration statement on Form 8-K, (001-36829), filed with the SEC on April 4, 2018, and incorporated herein by reference.

(6) Filed as an Exhibit to the Company's current report on Form 8-K (001-36829), filed with the SEC on August 5, 2016, and incorporated herein by reference.

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.

ROCKET PHARMACEUTICALS, INC.

May 9, 2019 By: /s/ Gaurav Shah, MD

Gaurav Shah, MD
President, Chief Executive Officer and Director
(Principal Executive Officer)

May 9, 2019 By: /s/ John Militello

John Militello
Controller
(Principal Financial and Accounting Officer)

Exhibit 31.1

CERTIFICATIONS

I, Gaurav Shah, MD, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended March 31, 2019 of Rocket Pharmaceuticals, 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: May 9, 2019

/s/ Gaurav Shah, MD

Gaurav Shah, MD
President, Chief Executive Officer and Director
(Principal Executive Officer)

Exhibit 31.2

CERTIFICATIONS

I, John Militello, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended March 31, 2019 of Rocket Pharmaceuticals, 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: May 9, 2019

/s/ John Militello

John Militello
Controller
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO
18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO
SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the quarterly report on Form 10-Q of Rocket Pharmaceuticals, Inc. (the "Company") for the period ended March 31, 2019, as filed with the United States Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers hereby certifies, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, 18 U.S.C. Section 1350, that to his knowledge:

1) the Report which this statement accompanies fully complies with the requirements of Section 13(a) or 15(d) 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.

Date: May 9, 2019

/s/ Gaurav Shah, MD
Gaurav Shah, MD
President, Chief Executive Officer and Director
(Principal Executive Officer)

Date: May 9, 2019

/s/ John Militello
John Militello
Controller
(Principal Financial Officer)