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 June 30, 2019

OR

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

For the transition period from to

Commission file number: 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 August 5, 2019, there were 50,348,435 shares of common stock, $0.01 par value per share, outstanding.

Forward-looking statements

This Quarterly Report on Form 10-Q for the Quarter ended June 30, 2019 ("Form 10-Q"), contains forward-looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended, or the Securities Act, and Section 21E of the Securities Exchange Act of 1934, as amended, or the Exchange Act. The words "believe," "will," "may," "estimate," "continue," "anticipate," "intend," "should," "plan," "expect," "predict," "could," "potentially" and variations of such words and similar expressions are intended to identify such forward-looking statements, which may include, but are not limited to, statements concerning the following:

federal, state, and non-U.S. regulatory requirements, including regulation of our current or any other future product candidates by the U.S. Food and Drug Administration ("FDA");

- the timing of and our ability to submit regulatory filings to the FDA and to obtain and maintain FDA or other regulatory authority approval of our product candidates;
- our competitors' activities, including decisions as to the timing of competing product launches, generic entrants, pricing and discounting;
- whether safety and efficacy results of our clinical trials and other required tests for approval of our product candidates provide data to warrant progression of clinical trials, potential regulatory approval or further development of any of our product candidates;
- our ability to develop, acquire and advance product candidates into, and successfully complete, clinical studies, and our ability to apply for and obtain regulatory approval for such product candidates, within currently anticipated timeframes, or at all;
- our ability to establish key collaborations and vendor relationships for our product candidates and any other future product candidates;
- our ability to successfully develop and commercialize any technology that we may in-license or products we may acquire;
- unanticipated delays due to manufacturing difficulties, supply constraints or changes in the regulatory environment;
- our ability to successfully operate in non-U.S. jurisdictions in which we currently or in the future may do business, including compliance with applicable regulatory requirements and laws;
- uncertainties associated with obtaining and enforcing patents to protect our product candidates, and our ability to successfully defend ourselves against unforeseen third-party infringement claims;
- anticipated trends and challenges in our business and the markets in which we operate;
- our estimates regarding our capital requirements; and
- our ability to obtain additional financing and raise capital as necessary to fund operations or pursue business opportunities.

These forward-looking statements are neither promises nor guarantees of future performance due to a variety of risks and uncertainties, many of which are beyond our control, which could cause actual results to differ materially from those indicated by these forward-looking statements, including, without limitation: the possibility that we may experience slower than expected clinical site initiation or slower than expected identification and enrollment of evaluable patients; the potential for delays or problems in analyzing data or the need for additional analysis, data or patients; the potential that future pre-clinical and clinical results may not support further development of our product candidates; the potential for unexpected adverse events in the conduct of one of our clinical trials to impact our ability to continue the clinical trial or further development of a product candidate; the risk that we may encounter other unexpected hurdles or issues in the development and manufacture of our product candidates that may impact our cost, timing or progress, as well as those risks more fully discussed in the "Risk Factors" section in this prospectus, the section of any accompanying prospectus supplement entitled "Risk Factors" and the risk factors and cautionary statements described in other documents that we file from time to time with the SEC, specifically under "Item 1A: Risk Factors" and elsewhere in our most recent Annual Report on Form 10-K for the period ended December 31, 2018, and our Current Reports on Form 8-K.

Given these uncertainties, readers should not place undue reliance on our forward-looking statements. These forward-looking statements speak only as of the date on which the statements were made and are not guarantees of future performance. Except as may be required by applicable law, we do not undertake to update any forward-looking statements after the date of this prospectus or the respective dates of documents incorporated by reference herein or therein that include forward-looking statements.

PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

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

	June 30, 2019	December 31, 2018
Assets	(unaudited)	
Current assets:		
Cash and cash equivalents	$ 64,833	$ 111,355
Investments	192,950	94,375
Prepaid expenses and other assets	5,846	3,358
Total current assets	263,629	209,088
Property and equipment, net	9,844	2,027
Goodwill	30,815	30,815
Restricted cash	1,525	1,436
Deposits	455	545
Operating lease right-of-use assets	2,452	-
Investments	-	7,402
Total assets	$ 308,720	$ 251,313
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 14,168	$ 15,372
Operating lease liabilities, current	913	-
Total current liabilities	15,081	15,372
Convertible notes, net of unamortized discount	43,180	41,447
Operating lease liabilities, non-current	1,933	-
Other liabilities	22	457
Total liabilities	60,216	57,276
Commitments and contingencies (Note 11)		
Stockholders' equity:		
Preferred stock, $0.01 par value, authorized 5,000,000 shares:		
Series A convertible preferred stock; 300,000 shares designated as Series A; 0 shares issued and outstanding at June 30, 2019 and December 31, 2018	-	-
Series B convertible preferred stock; 300,000 shares designated as Series B; 0 shares issued and outstanding at June 30, 2019 and December 31, 2018	-	-
Common stock, $0.01 par value, 120,000,000 shares authorized; 50,332,435 and 45,194,736 shares issued and outstanding at June 30, 2019 and December 31, 2018, respectively	503	452
Treasury stock, at cost, 22,308 and 50,000 common shares at June 30, 2019 and December 31, 2018, respectively	(344)	(668)
Additional paid-in capital	392,205	300,253
Accumulated other comprehensive income (loss)	144	(127)
Accumulated deficit	(144,004)	(105,873)
Total stockholders' equity	248,504	194,037
Total liabilities and stockholders' equity	$ 308,720	$ 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 June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	13,989	10,772	29,126	16,525
General and administrative	4,403	4,100	8,211	12,752
Total operating expenses	18,392	14,872	37,337	29,277
Loss from operations	(18,392)	(14,872)	(37,337)	(29,277)
Research and development incentives	-	-	250	186
Interest expense	(1,544)	(1,363)	(3,148)	(2,834)
Interest and other income, net	1,000	468	1,601	815
Accretion of discount on investments	256	-	503	-
Net loss	$ (18,680)	$ (15,767)	$ (38,131)	$ (31,110)
Net loss per share attributable to common stockholders - basic and diluted	$ (0.38)	$ (0.40)	$ (0.81)	$ (0.82)
Weighted-average common shares outstanding - basic and diluted	49,267,247	39,483,006	47,206,480	37,954,972

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 June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Net loss	$ (18,680)	$ (15,767)	$ (38,131)	$ (31,110)
Other comprehensive loss				
Net unrealized gain (loss) on investments	233	(89)	271	(79)
Total comprehensive loss	$ (18,447)	$ (15,856)	$ (37,860)	$ (31,189)

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

Rocket Pharmaceuticals, Inc.
Consolidated Statements of Stockholders' Equity
For the Three and Six Months Ended June 30, 2019 and 2018

(in thousands except share amounts)
(unaudited)

	Common Stock		Treasury	Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Stock	Capital	Income (Loss)	Deficit	Equity
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	-	-	-	-	-	-
Stock repurchase	-	-	(725)	(2)	-	-	(727)
Retirement of treasury stock	(100,000)	(1)	1,393	(1,392)	-	-	-
Unrealized comprehensive gain on marketable securities	-	-	-	-	38	-	38
Stock-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
Issuance of common stock, net of issuance costs	5,175,000	52	-	86,028	-	-	86,080
Issuance of common stock pursuant to exercise of stock options	42,998	-	-	-	-	-	-
Net exercise of options	-	-	(344)	-	-	-	(344)
Unrealized comprehensive gain on marketable securities	-	-	-	-	233	-	233
Stock-based compensation	-	-	-	4,138	-	-	4,138
Net loss	-	-	-	-	-	(18,680)	(18,680)
Balance at June 30, 2019	50,332,435	$ 503	$ (344)	$ 392,205	$ 144	$ (144,004)	$ 248,504

	Series A Convertible Preferred Shares		Series B Convertible Preferred Shares		Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Shares	Amount	Shares	Amount	Capital	Income (Loss)	Deficit	Equity
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
Stock-based compensation	-	-	-	-	-	-	5,382	-	-	5,382
Net loss	-	-	-	-	-	-	-	-	(15,343)	(15,343)
Balance at March 31, 2018	-	-	-	-	39,403,898	394	216,440	10	(46,698)	170,146
Issuance of	-	-	-	-	41,093	-	-	-	-	-

common stock pursuant to settlement of restricted stock units										
Issuance of common stock pursuant to settlement of stock option exercises	-	-	-	-	61,536	1	(1)	-	-	-
Unrealized comprehensive loss on marketable securities	-	-	-	-	-	-	-	(89)	-	(89)
Stock-based compensation	-	-	-	-	-	-	2,786	-	-	2,786
Net loss	-	-	-	-	-	-	-	-	(15,767)	(15,767)
Balance at June 30, 2018	-	$ -	-	$ -	39,506,527	$ 395	$ 219,225	$ (79)	$ (62,465)	$ 157,076

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

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

	Six Months Ended June 30,	
	2019	2018
Operating Activities:		
Net loss	$ (38,131)	$ (31,110)
Adjustments to reconcile net loss to net cash used in operating activities:		
Accretion of discount on convertible notes	1,733	1,454
Increase in lease liability	-	(109)
Depreciation expense	204	157
Stock-based compensation expense	7,319	8,168
Loss on disposal of property and equipment	-	205
Accretion of discount on investments	(503)	(118)
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(2,398)	(651)
Accounts payable and accrued expenses	(2,265)	665
Operating lease liabilities	(40)	-
Net cash used in operating activities	(34,081)	(21,339)
Investing activities:		
Cash acquired in connection with the Reverse Merger	-	76,348
Purchases of investments	(140,336)	(118,819)
Proceeds from maturities of investments	49,936	21,232
Proceeds from sale of property and equipment	-	20
Purchases of property and equipment	(7,305)	(117)
Net cash used in investing activities	(97,705)	(21,336)
Financing activities:		
Proceeds from issuance of common stock, net of issuance costs	86,080	78,518
Common stock repurchase	(727)	-
Net cash provided by financing activities	85,353	78,518
Net change in cash, cash equivalents and restricted cash	(46,433)	35,843
Cash, cash equivalents and restricted cash at beginning of period	112,791	18,349
Cash, cash equivalents and restricted cash at end of period	$ 66,358	$ 54,192
Supplemental disclosure of non-cash financing and investing activities:		
Accrued purchases of property and equipment	$ 716	$ -
Retirement of treasury stock	$ 1,395	$ -
Net exercise of options	$ 344	$ -
Unrealized gain (loss) on investments	$ 271	$ (79)
Conversion of convertible preferred stock into common stock	$ -	$ 41,466
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 began 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 $144.0 million as of June 30, 2019. As of June 30, 2019, the Company has $257.8 million of cash, cash equivalents and investments. During the six months ended June 30, 2019, the Company received net proceeds of $86.1 million from the completion on April 18, 2019 of a public offering of 5,175,000 shares of common stock. 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 June 30, 2019 and the results of its operations and its cash flows for the three and six months ended June 30, 2019 and 2018. The financial data and other information disclosed in these consolidated notes related to the three and six months ended June 30, 2019 and 2018 are unaudited. The results for the three and six months ended June 30, 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 United States ("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 stock-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 and six months ended June 30, 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 "Commitments and Contingencies" for additional disclosures) 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:

	June 30, 2019	December 31, 2018
Cash and cash equivalents	$ 64,833	$ 111,355
Restricted cash	1,525	1,436
	$ 66,358	$ 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.

Government Grants

Research and development expense is presented net of reimbursements from the California Institute for Regenerative Medicine ("CIRM"), which are recognized over the period necessary to match the reimbursement with the related costs when it is probable that the Company has complied with the CIRM conditions and will receive the reimbursement. During the three months ended June 30, 2019, we offset $0.8 million of grant funds against research and development expenses (see Note 14).

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 June 30, 2019 Using:			
	Level 1	**Level 2**	**Level 3**	**Total**
Assets:				
Money market mutual funds (included in cash and cash equivalents)	$ 30,905	$ -	$ -	$ 30,905
United States Treasury securities (included in cash and cash equivalents)	9,985	-	-	9,985
	40,890	-	-	40,890
Current:				
United States Treasury securities	121,946	-	-	121,946
Government Bonds	-	35,754	-	35,754
Corporate Bonds	-	26,250	-	26,250
Municipal Bonds	-	9,000	-	9,000
Investments	121,946	71,004	-	192,950
	$ 162,836	$ 71,004	$ -	$ 233,840

	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. The company classifies its Government, Corporate and Municipal Bonds as Level 2 assets as these assets are not traded in an active market and have been valued through a third-party pricing service. The fair value of the 2021 Convertible Notes as of June 30, 2019 was $46.9 million.

5. Property and Equipment

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

	June 30, 2019	**December 31, 2018**
Laboratory equipment	$ 1,850	$ 1,556
Leasehold improvements	29	29
Furniture and fixtures	273	273
Computer equipment	179	179
Construction in progress	8,196	469
	10,527	2,506
Less: accumulated depreciation	(683)	(479)
	$ 9,844	$ 2,027

Construction in progress comprises costs associated with the build out of the Company's research facilities under an operating lease in Cranbury, NJ. See Note 11 "Commitments and Contingencies" for additional disclosures. During the three and six months ended June 30, 2019, the Company recognized $0.1 million and $0.2 million of depreciation expense, respectively. During the three and six months ended June 30, 2018, the Company recognized $74,000 and $0.1 million of depreciation expense, respectively.

6. Accounts Payable and Accrued Expenses

At June 30, 2019 and December 31, 2018, the Company's accounts payable and accrued expenses consisted of the following:

	June 30, 2019	December 31, 2018
Research and development	$ 9,439	$ 10,414
Construction in progress	788	-
Government grant payable	548	534
Professional fees	665	690
Accrued interest	1,241	1,241
Bonus	1,021	1,774
Other	234	589
Accrued vacation	232	130
	$ 14,168	$ 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 and 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 June 30, 2019 and December 31, 2018.As of June 30, 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 June 30, 2019:

Principal amount	$ 52,000
Discount	(8,820)
Carrying value as of June 30, 2019	$ 43,180

Accretion of the 2021 Convertible Notes discount was $0.8 million and $1.7 million for the three and six months ended June 30, 2019, respectively. Accretion of the 2021 Convertible Notes discount was $0.7 million and $1.5 million for the three and six months ended June 30, 2018, respectively.

8. Stock Based Compensation

Stock Option Valuation

Effective July 1, 2018, the Company adopted ASU No. 2018-07, *Compensation—Stock Compensation (Topic 718): Improvements to Nonemployee Share-Based Payment Accounting* ("ASU 2018-07"). The table below for the six months ended June 30, 2019 is post adoption of ASU 2018-07. The weighted average assumptions that the Company used in the Black-Scholes pricing model to determine the fair value of the stock options granted to employees, non-employees and directors for the six months ended June 30, 2019 and the stock options granted to employees and directors for the six months ended June 30, 2018 were as follows:

	Six Months Ended June 30, 2019	2018
Risk-free interest rate	2.57%	2.60%
Expected term (in years)	5.79	5.78
Expected volatility	74.99%	88.60%
Expected dividend yield	0.00%	0.00%
Exercise price	$ 15.01	$ 17.63
Fair value of common stock	$ 15.01	$ 17.63

The following table for the six months ended June 30, 2018 is before the adoption of ASU 2018-07. The weighted average assumptions that the Company used in the Black-Scholes pricing model to determine the fair value of the stock options granted to non-employees and directors for the six months ended June 30, 2018 were as follows:

	Six Months Ended June 30, 2018
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 six months ended June 30, 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	1,175,866	15.01	9.79	
Exercised	(62,699)	1.81		1,389
Forfeited	(296,589)	6.50		
Outstanding as of June 30, 2019	9,432,575	$ 5.75	7.55	$ 92,783
Options vested and exercisable as of June 30, 2019	6,949,270	$ 2.55	6.92	$ 88,373
Options unvested as of June 30, 2019	2,483,305	$ 14.69	9.20	

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 June 30, 2019 and 2018 was $88,373 and $119,000 respectively.

The weighted average grant-date fair value per share of stock options granted during the six months ended June 30, 2019 and 2018 was $9.90 and $12.93, respectively.

The total fair value of options vested during the six months ended June 30, 2019 and 2018 was $35.7 million and $41.8 million, respectively.

Stock-Based Compensation

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Research and development	$ 2,252	$ 1,403	$ 3,818	$ 3,610
General and administrative	1,886	1,382	3,501	4,558
Total stock based compensation expense	$ 4,138	$ 2,785	$ 7,319	$ 8,168

As of June 30, 2019, the Company had an aggregate of $22.3 million of unrecognized stock-based compensation cost, which is expected to be recognized over the weighted average period of 1.92 years.

9. Stockholders' 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 June 30, 2019, convertible into 14,102 shares of common stock at an exercise price of $24.82 per share, and expiring on June 28, 2023.

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 an additional 675,000 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 $86.1 million after deducting $4.5 million of offering costs, commissions, legal and other expenses for net proceeds from the gross proceeds from the offering of $90.6 million.

On June 25, 2019, the Company recorded treasury stock of $0.3 million for the payroll tax liability of an option exercise. The related payroll tax liability is included in the balance sheet within accounts payable and accrued expenses.

10. Net Loss Per Share

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Numerator:				
Net loss	$ (18,680)	$ (15,767)	$ (38,131)	$ (31,110)
Denominator:				
Weighted-average common shares outstanding - basic and diluted	49,267,247	39,483,006	47,206,480	37,954,972
Net loss per share- basic and diluted	$ (0.38)	$ (0.40)	$ (0.81)	$ (0.82)

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Shares issuable upon conversion of the 2021 Convertible Notes	1,620,948	1,620,948	1,620,948	1,620,948
Warrants exercisable for common shares	14,102	14,102	14,102	14,102
Options to purchase common shares	9,432,575	8,870,004	9,432,575	8,870,004
	11,067,625	10,505,054	11,067,625	10,505,054

11. Commitments and Contingencies

Operating Leases

On August 14, 2018, Rocket entered into a lease for office space, process development, research activities and manufacturing to support the Company's pipeline in Cranbury, NJ. The NJ Lease Agreement was subsequently amended on June 26, 2019. The amendment to the NJ Lease increased by 10,000 square feet the space rental by the Company to 102,000 square feet and adjusted the lease payment date of rent for the full building ("Lease Payment Date") and the beginning of the term of the lease to September 1, 2019. The NJ Lease Agreement has a term of 15 years from the Lease Payment 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 Lease Payment 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 June 30, 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	**June 30, 2019**
Operating lease cost	$ 501
Total lease cost	$ 501

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 June 30, 2019:

Maturity of Lease Liabilities	**June 30, 2019**
2019 (remaining six months)	$ 546
2020	1,103
2021	894
2022	572
2023	73
Total future minimum lease payments	$ 3,188
Less: Imputed interest	(342)
Present value of future minimum lease payments	$ 2,846

The following disclosure is provided for periods prior to adoption of ASU 2016-02. Future annual minimum lease payment commitments as of June 30, 2019 were as follows:

2019 (remaining six months)	$ 987
2020	1,970
2021	1,907
2022	1,757
2023	1,618
Thereafter	20,144
Total	$ 28,383

Leases	**June 30, 2019**
Operating right-of-use assets	$ 2,452
Operating current lease liabilities	913
Operating noncurrent lease liabilities	1,933
Total operating lease liabilities	$ 2,846

Other information

Cash paid for amounts included in the measurement of lease liabilities:	
Operating cash flows from operating leases	$ 542
Weighted-average remaining lease term - operating leases	3.1 years
Weighted-average discount rate - operating leases	7.77%

Rent expense was $0.5 million and $0.4 million for the six months ended June 30, 2019 and 2018, respectively. Rent expense was $0.3 million and $0.2 million for the three months ended June 30, 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 six months ended June 30, 2019, none of the remaining milestones were met with regard to the LCGM.

14. CIRM Grant

On April 30, 2019, the Company announced the California Institute for Regenerative Medicine ("CIRM") awarded Rocket up to a $6.5 million CLIN2 grant award to support the clinical development of gene therapy for LAD-I. Proceeds from the grant will help fund clinical trial costs as well as manufactured drug product for Phase I/II patients enrolled at the U.S. clinical site, University of California, Los Angeles ("UCLA") Mattel Children's Hospital, led by principal investigator 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. On July 1, 2019, the Company received the first cash advance from CIRM of $0.8 million. The CIRM grant receipts will be treated as an offset against R&D expenses as reimbursable expenses are incurred. As of June 30, 2019, the Company recorded a receivable and reduction of R&D expenses of $0.8 million for the reimbursable expenses incurred prior to June 30, 2019.

15. 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 $3,600 and $149,293 during the six months ended June 30, 2019 and 2018, respectively and expenses of $3,600 and $35,000 during the three months ended June 30, 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 $27,500 per quarter, and the Company may terminate the agreement with 14 days' notice. The Company incurred expenses of $55,000 for the six months ended June 30, 2019 and 2018, respectively and $27,500 for the three months ended June 30, 2019 and 2018, respectively, relating to services provided under this consulting agreement.

16. 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 six months ended June 30, 2019 and 2018 was $125,204 and $57,995, respectively and $43,580 and $35,130 for the three months ended June 30, 2019 and 2018, respectively.

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

You should read the following discussion and analysis of our financial condition and results of operations together with the condensed consolidated financial statements and related notes that are included elsewhere in this Quarterly Report on Form 10-Q and our Annual Report on Form 10-K for the fiscal year ended December 31, 2018 filed with the U.S. Securities and Exchange Commission, or the SEC, on March 8, 2019, or our 2018 Form 10-K. This discussion contains forward-looking statements based upon current plans, expectations and beliefs that involve risks and uncertainties. Our actual results may differ materially from those anticipated in these forward-looking statements as a result of various factors, including, but not limited to, those discussed in the section entitled "Risk Factors" and elsewhere in this Quarterly Report on Form 10-Q. In preparing this MD&A, we presume that readers have access to and have read the MD&A in our Annual Report on Form 10-K, pursuant to Instruction 2 to paragraph (b) of Item 303 of Regulation S-K.

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 began 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 completed a public offering of 5,175,000 shares of common stock, which includes the full exercise of the underwriters' option to purchase an additional 675,000 shares of our common stock, at a public offering price of $17.50 per share. The net proceeds to Rocket from the public offering were approximately $86.1 million, after deducting $4.5 million of offering costs, commissions, legal and other expenses from the gross proceeds from the offering of $90.6 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 stockholders 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 California Institute for Regenerative Medicine ("CIRM") awarded Rocket up to $6.5 million CLIN2 grant award to support the clinical development of gene therapy for LAD-I. Proceeds from the grant will help fund clinical trial costs as well as manufactured drug product for Phase I/II patients enrolled at the U.S. clinical site, University of California, Los Angeles ("UCLA") Mattel Children's Hospital, led by principal investigator 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. On July 1, 2019, we received the first grant from CIRM of $0.8 million.

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).

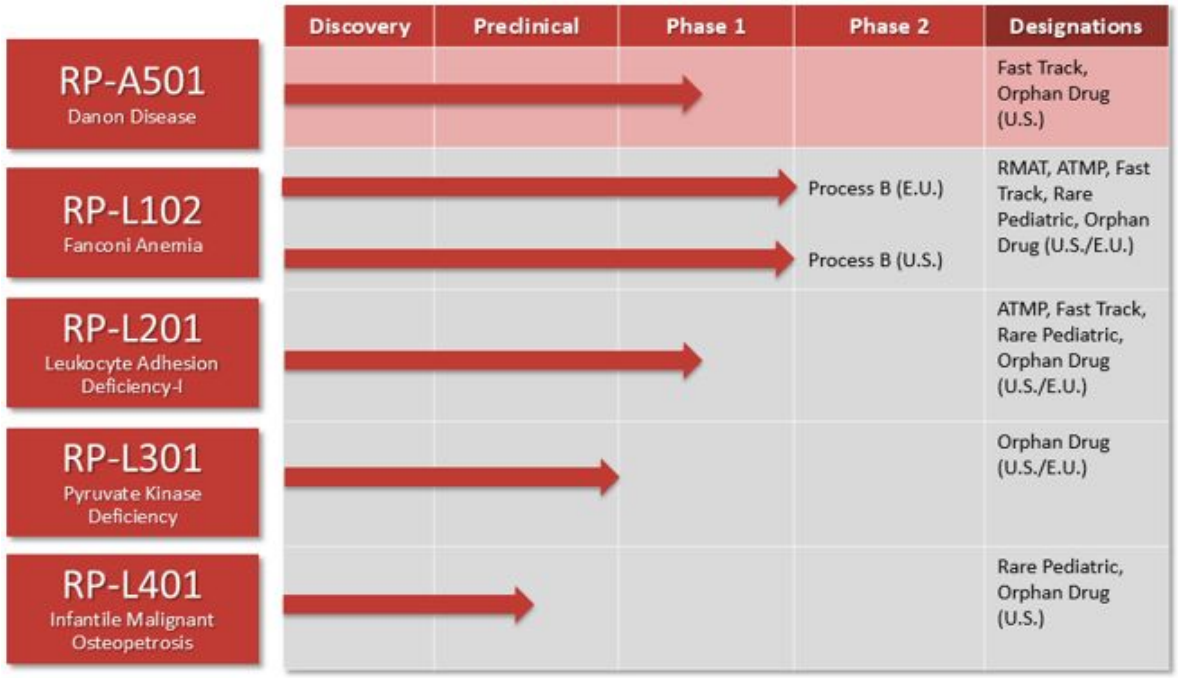
The gene therapies can be delivered either (1) *ex vivo* (outside the body), in which case the patient's cells are extracted and the vector is delivered to these cells in a controlled, safe laboratory setting, with the modified cells then being reinserted into the patient, or (2) *in vivo* (inside the 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' hematopoietic stem cell ("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. The initial two FA pediatric patients have received treatment on the Phase 1 clinical trial of "Process B" RP-L102 at Stanford University. 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 fourth quarter of 2019.

On April 29, 2019, long-term updated 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.

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. and will assess the safety and tolerability of RP-L201. The Phase 1 portion of the registrational trial is underway with the first patient's cells harvested in the second quarter. Preliminary data will be presented in the fourth quarter.

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 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.

This program has been granted EMA orphan drug disease designation and FDA orphan drug disease designation ("ODD"). Initiation of the Phase 1 clinical trial of RP-L301 is anticipated in the fourth quarter 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 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 January 2019, we announced the clearance of our IND application by the FDA for RP-A501. We dosed the first patient initiating our Phase 1 clinical trial of RP-A501 for Danon disease in the second quarter of 2019. University of California San Diego Health is the initial and lead center for the Phase 1 clinical trial. In February 2019, we were notified by the FDA that we were granted Fast Track designation for RP-A501.

On May 2, 2019, we presented 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 June 30, 2019, Rocket raised net cash proceeds of approximately $294.5 million from investors through both equity and convertible debt financing to fund operating activities. As of June 30, 2019, we had cash, cash equivalents and investments of $257.8 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 $38.1 million for the six months ended June 30, 2019. As of June 30, 2019, we had an accumulated deficit of $144.0 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 stock-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 stock-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 our 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 stock-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.

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 11 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 June 30, 2019 and 2018

The following table summarizes the results of operations for the three months ended June 30, 2019 and 2018 ($ in thousands):

	Three Months Ended June 30,		
	2019	2018	Change
Operating expenses:			
Research and development	$ 13,989	$ 10,772	$ 3,217
General and administrative	4,403	4,100	303
Total operating expenses	18,392	14,872	3,520
Loss from operations	(18,392)	(14,872)	(3,520)
Research and development incentives	-	-	-
Interest expense	(1,544)	(1,363)	(181)
Interest and other income net	1,000	468	532
Accretion of discount on investments	256	-	256
Total other expense net	(288)	(895)	607
Net loss	$ (18,680)	$ (15,767)	$ (2,913)

Research and Development Expenses

R&D expenses increased $3.2 million to $14.0 million for the three months ended June 30, 2019 compared to the three months ended June 30, 2018. The increase was primarily a result of an increase in clinical trial expenses of $2.1 million as the Phase 1 clinical trials commenced for FA and Danon disease; an increase in license expenses of $1.1 million; an increase in stock compensation expense of $0.8 million; offset by a decrease in preclinical research of $1.3 million.

General and Administrative Expenses

G&A expenses increased $0.3 million to $4.4 million for the three months ended June 30, 2019 compared to the three months ended June 30, 2018. The increase in G&A was primarily driven by an increase in stock compensation expense of $0.5 million and an increase in compensation and benefits expense of $0.4 million; offset by decreases in consulting, legal and other G&A expenses of $0.6 million.

Other Expense

Other expense decreased $0.6 million to $0.3 million for the three months ended June 30, 2019 compared to the three months ended June 30, 2018, which was primarily due to an increase in interest and accretion income of $0.8 million, offset by an increase in interest expense of $0.2 million.

Comparison of the Six Months Ended June 30, 2019 and 2018

The following table summarizes the results of operations for the six months ended June 30, 2019 and 2018 ($ in thousands):

	Six Months Ended June 30,		
	2019	2018	Change
Operating expenses:			
Research and development	$ 29,126	$ 16,525	$ 12,601
General and administrative	8,211	12,752	(4,541)
Total operating expenses	37,337	29,277	8,060
Loss from operations	(37,337)	(29,277)	(8,060)
Research and development incentives	250	186	64
Interest expense	(3,148)	(2,834)	(314)
Interest and other income net	1,601	815	786
Accretion of discount on investments	503	-	503
Total other expense net	(794)	(1,833)	1,039
Net loss	$ (38,131)	$ (31,110)	$ (7,021)

Research and Development Expenses

R&D expenses increased $12.6 million to $29.1 million for the six months ended June 30, 2019 compared to the six months ended June 30, 2018. The increase was primarily a result of increase in manufacturing and process development expenses of $4.7 million; increase in clinical trials expense of $2.5 million as the Phase I clinical trials commenced for FA and Danon disease; increase in compensation expense of $1.7 million as R&D headcount increased during the six months ended June 30, 2019 as compared to the six months ended June 30, 2018; an increase in license expense of $1.0 million and an increase in stock compensation expense of $1.0 million.

General and Administrative Expenses

G&A expenses decreased $4.5 million to $8.2 million for the six months ended June 30, 2019 compared to the six months ended June 30, 2018. Included in G&A for the six months ended June 30, 2018 were merger-related expenses of $5.3 million which were incurred for the six months ended June 30, 2018, including stock-based compensation expenses and post-Reverse Merger transition expenses including payroll and severance payments for remaining Inotek employees retained for the post-Reverse Merger transition which were not recurring for the six months ended June 30, 2019. The decrease in G&A for the six months ended June 30, 2019 was primarily driven by a decrease in stock compensation expense of $1.8 million; a decrease in compensation and benefits of $1.6 million and a decrease in consulting and legal expenses of $0.6 million.

Other Expense

Other expense decreased by $1.0 million to $0.8 million for the six months ended June 30, 2019 compared to the six months ended June 30, 2018, which was primarily due to an increase in interest and accretion income of $1.3 million, offset by an increase in interest expense of $0.3 million.

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:

	Six Months Ended June 30,	
	2019	**2018**
Cash used in operating activities	$ (34,081)	$ (21,339)
Cash used in investing activities	(97,705)	(21,336)
Cash provided by financing activities	85,353	78,518
Net change in cash, cash equivalents and restricted cash	$ (46,433)	$ 35,843

Operating Activities

During the six months ended June 30, 2019, operating activities used $34.1 million of cash, primarily resulting from our net loss of $38.1 million offset by net changes in our operating assets and liabilities of $4.7 million and net non-cash charges of $8.8 million, including stock-based compensation expense of $7.3 million and accretion of discount on convertible notes of $1.7 million. Changes in Rocket's operating assets and liabilities for the six months ended June 30, 2019 consisted of increases in prepaid expenses and other assets of $2.4 million, and accounts payable and accrued expenses of $2.3 million.

During the six months ended June 30, 2018, operating activities used $21.3 million of cash, primarily resulting from our net loss of $31.1 million, partially offset by net non-cash charges of $9.8 million, including stock-based compensation expense of $8.2 million. There was no change in our operating assets and liabilities for the six months ended June 30, 2018 as the increase in accounts payable and accrued expenses of $1.3 million was offset by a decrease in prepaid expenses and other current assets of $0.7 million and a decrease in accrued research and development of $0.6 million.

Investing Activities

During the six months ended June 30, 2019, net cash used in investing activities was $97.7 million, consisting of purchases of investments of $140.3 million and purchases of property and equipment of $7.3 million, offset by proceeds of $49.9 million from the maturities of investments.

During the six months ended June 30, 2018, net cash outflow on investing activities was $21.3 million, consisting of purchases of investments of $118.8 million offset by $76.3 million of cash acquired in connection with the Reverse Merger, and $21.2 million from the maturities of investments.

Financing Activities

During the six months ended June 30, 2019, net cash provided by financing activities was $85.4 million, consisting of proceeds from the issuance of common stock of $86.1 million, offset by share repurchase of $0.7 million.

During the six months ended June 30, 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 June 30, 2019, we had cash, cash equivalents and investments of $257.8 million. We expect 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 $257.8 million at June 30, 2019, consisting primarily of funds in a money market account, corporate and municipal bonds 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 June 30, 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 six months ended June 30, 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 $18.7 million and $38.1 million for the three and six months ended June 30, 2019, respectively, and $74.5 million and $19.6 million for the years ended December 31, 2018 and 2017, respectively. As of June 30, 2019, we had an accumulated deficit of $144.0 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 June 30, 2019, our cash, cash equivalents and investments was $257.8 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 narrower 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 August 6, 2019, we had 41 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 34.0% 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 (incorporated by reference to Exhibit 2.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on September 13, 2017)
3.1	Seventh Amended and Restated Certificate of Incorporation of Rocket Pharmaceuticals, Inc., effective as of February 23, 2015 (incorporated by reference to Exhibit 3.1 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 31, 2015)
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 (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on January 5, 2018)
3.3	Certificate of Amendment (Name Change) to the Seventh Amended and Restated Certificate of Incorporation of the Registrant, effective January 4, 2018 (incorporated by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on January 5, 2018)
3.4	Certificate of Amendment to the Seventh Amended and Restated Certificate of Incorporation of the Registrant, effective as of June 25, 2018 (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on June 25, 2018)
3.5	Amended and Restated By-Laws of Rocket Pharmaceuticals, Inc., effective as of March 29, 2018 (incorporated by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K, (001-36829), filed with the SEC on April 4, 2018)
10.1*†	Amended and Restated Lease Agreement, dated as of June 26, 2019, by and between Rocket Pharmaceuticals, Inc. and Cedar Brook 12 Corporate Center, L.P.
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.

† Certain portions of this exhibit have been excluded because they are both not material and would likely cause competitive harm to the Company if publicly disclosed

SIGNATURES

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

ROCKET PHARMACEUTICALS, INC.

August 8, 2019 By: /s/ Gaurav Shah, MD
Gaurav Shah, MD
President, Chief Executive Officer and Director
(Principal Executive Officer)

August 8, 2019 By: /s/ John Militello
John Militello
Controller
(Principal Financial and Accounting Officer)

Exhibit 10.1

Pursuant to 17 CFR 229.601, certain identified information marked "[***]" has been excluded from this exhibit because it is both (i) not material and (ii) would be competitively harmful if publicly disclosed.

Amended and Restated Lease Agreement

9 Cedar Brook, Cranbury NJ 05812

Between Cedar Brook 12 Corporate Center, L.P., Landlord

and

Rocket Pharmaceuticals, Inc., Tenant

June 26, 2019

Pursuant to 17 CFR 229.601, certain identified information marked "[***]" has been excluded from this exhibit because it is both (i) not material and (ii) would be competitively harmful if publicly disclosed.

Contents

Pursuant to 17 CFR 229.601, certain identified information marked "[***]" has been excluded from this exhibit because it is both (i) not material and (ii) would be competitively harmful if publicly disclosed.

This AMENDED AND RESTATED LEASE, made as of June 26, 2019 ("Effective Date"), between Cedar Brook 12 Corporate Center, L.P., 4A Cedar Brook Drive., Cranbury, New Jersey 08512 ("Landlord"), and Rocket Pharmaceuticals, Inc., 350 Fifth Avenue, Suite 7530, New York, NY 10118 ("Tenant").

RECITALS:

WHEREAS, Landlord and Tenant have entered into that certain Lease (the "Original Lease") dated as of August 14, 2018 whereby Landlord leased to Tenant the Leased Premises, as described in Section 1.1. below, located in the building located at 9 Cedar Brook Drive, Cranbury, New Jersey, 08512 ("Building") [***] as shown on the site plan attached hereto as Exhibit A (the "Property), and constituting a portion of the office/industrial park known as Cedar Brook Corporate Center ("Office Park"); and

WHEREAS, the parties hereto wish to amend and restate the Original Lease to mutually define their rights, duties and obligations as set forth herein.

NOW THEREFORE, in consideration of the promises set forth herein, Landlord leases unto Tenant and Tenant leases from Landlord the leased premises described in Paragraph 1, and Landlord and Tenant do hereby mutually covenant and agree as follows:

1. LEASED PREMISES

1.1 The leased premises shall consist of the entire 82,520 rentable square feet of [***] space in the Building, and 21,200 rentable square feet of basement space (collectively referred to herein as the "Leased Premises") as shown on Exhibit B attached hereto which identifies that portion of the Leased Premises referred to herein as the "Initial Premises" and that portion of the Leased Premises referred to herein as the "Additional Premises". The Leased Premises is measured from outside of exterior walls to outside of exterior walls or centerline of demising walls, if any, and shall include all fixtures and equipment that currently exist or are to be installed in and attached to the Leased Premises by the Landlord or the Tenant for the use of the Tenant. Tenant shall also have the exclusive use to all parking spaces on the Property, as shown on the attached Exhibit A, exclusive use to all areas of the Property on which equipment servicing the Leased Premises are currently or hereafter located, and the nonexclusive use of all of the other common areas within the Property. Tenant shall also have the right to use all common areas ("Common Areas") defined as those areas and facilities of the Office Park which are available for the use of tenants within the buildings in the Office Park, including parking areas, pedestrian walkways, sidewalks and landscaped areas within the Office Park. Tenant may use all Common Areas only for their intended purposes. Landlord shall have exclusive control of all Common Areas at all times and may make such changes to the Common Areas as Landlord deems appropriate, provided that Landlord shall provide advance notice to the Tenant of any planned changes and shall use commercially reasonable efforts to minimize disruption of Tenant's access to and use and occupancy of the Leased Premises and any material changes to the Property shall not be made without Tenant's prior, written consent.

2. TERM OF LEASE

2.1 The term of the Lease ("Term") shall be fifteen (15) years, to commence on the Commencement Date as hereinafter defined, and to end on the day before the fifteenth (15th) anniversary of the Commencement Date ("Expiration Date"). The term "Commencement Date" or "Lease Commencement Date" shall mean September 1, 2019.

3. CONSTRUCTION OF THE TENANT IMPROVEMENTS IN INITIAL PREMISES

3.1 (a) The Landlord shall provide all necessary labor and materials and perform any and all of the work required for construction of the Tenant's [***] facilities, including machinery, fixtures and equipment to be constructed and other improvements to be installed by Landlord in the Initial Premises in order to prepare the Initial Premises for Tenant's occupancy, including wiring for Tenant's data and telecommunications systems within the walls of the portion of the Leased Premises used for office and laboratory space, and all other improvements to be installed above the ceiling, within the walls and under the floor (the "Tenant Improvements"), all as shown on the construction drawings and specifications to be prepared by an architect and engineer selected by Tenant and approved by Landlord, which approval shall not be unreasonably withheld ("Plans"), subject to the terms and conditions of this Section 3. As of the Effective Date, Tenant's architect/engineer has delivered complete Plans for the Initial Premises to Landlord. Tenant anticipates delivering plans for the Additional Premises (the "Additional Premises Plans") by October 1, 2019. If Substantial Completion of Tenant Improvements in the Initial Premises does not occur by the two hundred eleventh (211th) day following the parties' approval of the Construction Budget for the Initial Premises (following the parties' mutually agreed value engineering (including any revisions to the Plans arising therefrom or relating thereto) and as determined in accordance with Section 3.2 and as evidenced by the parties signature thereon) (the "Initial Outside Completion Date") because of the action or inaction of Landlord or its employees, contractors and/or agents, then, following the Initial Outside Completion Date, Tenant shall be entitled to an abatement of rent equivalent to two times the daily Base Rent (determined by dividing the monthly Base Rent payable as of the first day of the Term by thirty (30)) for each day after the Initial Outside Completion Date, that Substantial Completion of the Tenant Improvements in the Initial Premises has not occurred. Landlord shall not be responsible for any delay in Substantial Completion of the Tenant Improvements in the Initial Premises due to the action or inaction of Tenant or its employees, contractors and/or agents, or for any delay arising as the result of any Change Orders or other modifications to the Plans requested by Tenant or required to comply with applicable law, rule or regulation. Tenant's designated representative for all work pertaining to the Tenant Improvements shall be [***] ("Representative"). Landlord shall supervise and direct the construction of Tenant Improvements using Landlord's best skill and attention, and Landlord shall be solely responsible for all construction means, methods, techniques, sequences, and procedures and for coordinating all portions of the work on the Tenant Improvements in accordance with the Plans. Landlord warrants to the Tenant that all materials and equipment incorporated into the existing Leased Premises will be new unless otherwise specified or approved by Tenant, and that all work on the Tenant Improvements will be of good quality, free from known faults and defects (provided that Landlord shall remain responsible to remedy any construction defects which are discovered after the Commencement Date as provided in Section 7.1), and in substantial conformity with the Plans, provided any change in the construction from that shown on the Plans which impacts Tenant's business operations or any substitution of materials from that shown on the Plans shall only be made with Tenant's prior written approval. Notwithstanding the foregoing, the Tenant Improvements to be constructed by Landlord are set forth on Schedule 3.1(b) attached hereto under the heading "Provided by Cedar Brook." In addition, Schedule 3.1(c) attached hereto shows the demarcation between the scope of work of Landlord and Tenant's contractor, AES Clean Technology, Inc. ("AES"), in the Leased Premises.

(b) Tenant shall be responsible for all items listed on the attached Schedule 3.1(b) under the heading "Provided by Rocket Pharma" and as shown on Schedule 3.1(c) attached hereto, which work shall not be included in "Tenant Improvements" ("Tenant Work"). With respect to Tenant Work, Tenant shall (i) coordinate the performance of Tenant Work so as to minimize interference with work being performed by Landlord; (ii) [***], and (iii) provide evidence that Tenant's contractors have appropriate insurance. Schedule 3.1(a) identifies those portions of the Tenant Improvements which must be completed by Landlord (the "Initial Landlord Work") prior to AES commencing its portion of the Tenant Work in the single-story area of the Initial Premises. Landlord will provide Tenant with written notice of the completion of the Initial Landlord Work. Subject to the provisions of Section 18 (other than Landlord's right to offer to perform the Alterations which Landlord hereby waives), Tenant may, at any point during the Term, engage AES (or a contractor having similar experience and reputation as AES (and being referred to herein as "AES")) to construct cleanroom and/or cleanroom support spaces within the area designated in the Leased Premises as the cleanroom space as shown on Schedule 3.1(c), at its option and expense at any point during the Term. Any construction or other work to be conducted by AES in the Leased Premises is referred to herein as the "AES Work."

(c) Tenant shall have the right to place mechanical and other equipment on the roof of the Building, provided the equipment is located within the roof screens. Tenant shall also have the right to request that Landlord install an emergency generator at Tenant's cost at a location mutually agreeable to the parties, which will be located on the Property, but may be located outside of the Leased Premises. Landlord will assist Tenant, if required, in obtaining any governmental approvals necessary for the installation of the generator.

(d) Tenant shall, at its expense, cause AES to (i) make commercially reasonable efforts to coordinate the performance of the AES Work with, and not unreasonably and materially interfere, delay, hinder or restrict, Landlord's construction of the Tenant Improvements, (ii) abide by and comply with all safety and construction rules, regulations, ordinances, codes, guidelines and procedures required under applicable law, rule, regulation or common construction industry practice, (iii) obtain, maintain and provide evidence of insurance in accordance with the insurance certificate attached hereto as Exhibit 3.1(d) attached hereto, (iv) obtain, maintain and provide evidence of its receipt of all governmental, local and municipal approvals, licenses, permits and authorizations necessary for the AES Work prior to the commencement thereof, (v) undertake all commercially reasonable efforts to only use contractors, employees and other personnel that will work in harmony with [***], (vi) not directly use or contract with any contractors generally used or designated by Landlord for any portion of the AES Work, other than pursuant to subcontracts with Landlord providing for Landlord pricing, and (vii) not cause or permit the filing of any liens, claims or charges upon the Building or Property in respect of its portion of the Tenant's Work and to immediately remove and discharge the same within thirty (30) days of notice thereof. Tenant shall be directly responsible to Landlord for any direct damages and shall indemnify and hold Landlord harmless for any loss, cost, expense, damage or claim arising solely from any breach by AES of any of the foregoing up to a maximum of One Million Dollars ($1,000,000.00) (except that such limitation shall not apply to any breach of clauses (ii), (iii), (iv) or (vii)). Notwithstanding anything in this Amendment or the Lease, under no circumstances shall Tenant be liable for any punitive or consequential damages.

3.2 (a) Landlord shall complete the construction of Tenant Improvements in the Initial Premises (and in the Additional Premises if Landlord performs the Tenant Improvements in the Additional Premises) in a good and workmanlike manner and in substantial accordance with the Plans, provided Landlord shall not make any modifications to the construction from that shown on the Plans which would impact Tenant's operations without Tenant's prior written consent. The Plans shall be in sufficient detail to permit Landlord to apply for a building permit for the Tenant Improvements (which Landlord shall promptly do), and to prepare a construction budget for the construction of the Tenant Improvements ("Initial Construction Budget(s)"). The Initial Construction Budget for the Initial Premises and/or Additional Premises, as applicable, shall set forth the lump sum amount payable by Tenant to Landlord for the construction of the Tenant Improvements ("Hard Construction Cost"). The Initial Construction Budgets for Tenant Improvements shall also include Landlord's standard mark-up of [***] of the Hard Construction Costs for general conditions, [***] of the Hard Construction Costs for overhead, and [***] of the Hard Construction Costs for profit. The only exclusion from the Initial Construction Budgets shall be the actual fees charged by the Township of Cranbury for construction permits and certificates of occupancy, which will not be determined by the municipality until after the Landlord applies for the construction permits and certificates of occupancy and shall be paid by Tenant as set forth hereafter.

Concurrent with the execution of this Lease, Landlord will provide the Initial Construction Budget to Tenant for the Tenant Improvements within the Initial Premises. The parties shall cooperate to value engineer the Tenant Improvements (which value engineering shall be approved by Tenant's engineers, architects and other professionals) and finalize the Initial Construction Budget in a manner and in an amount that is acceptable to the parties and, in any event, within 30 days of the Effective Date . Upon the approval of the Initial Construction Budget for the Initial Premises, the Plans and the budget will be signed by both parties and the budget shall thereafter be deemed the "Construction Budget."

Within thirty (30) days of receipt of Plans for the Additional Premises ("Additional Premises Plans"), Landlord shall prepare and submit the Initial Construction Budget for the Additional Premises to Tenant for its approval. Tenant, if so desires, and within this same thirty day period, may obtain additional bids for the construction of Tenant Improvements in the Additional Premises from contractors, construction estimators and/or construction managers experienced [***]the New York/New Jersey region. Bids or estimates from all parties shall be opened no later than the end of this fifteen (15) day period, at the same time in the presence of both the Tenant and Landlord, at Landlord's office and at a time mutually acceptable to the parties. All bids shall be revised, if necessary, to ensure that the bids include all items necessary to complete construction of Tenant Improvements according to the Additional Premises Plans. If the Landlord's Initial Construction Budget, including all markups for overhead, profit and general conditions, is not the lowest estimate, Landlord shall be provided with sufficient backup to determine whether the Tenant's bid includes all items contained in Landlord's Initial Construction Budget and Landlord shall have an opportunity to adjust its bid. In addition, if the bid submitted on behalf of Tenant contains items that are not included in Landlord's Initial Construction Budget, Landlord shall revise its proposed budget to include such items at Tenant's request. If, after finalizing the bids, the Landlord's Initial Construction Budget, compared to Tenant's bid is the low bid, Tenant shall accept Landlord's Initial Construction Budget after which Landlord shall immediately commence construction of Tenant Improvements in the Additional Premises. If, after review and any revisions to the bids, the Landlord's Initial Construction Budget is still higher than other bid submitted, then Landlord shall either cede the construction to the Tenant's general contractor or agree to perform the work at the lowest bid. Once finalized and approved by the parties, the budget shall be deemed the "Additional Premises Construction Budget". Landlord shall not be obligated to order any equipment or commence work until Tenant has approved the Additional Premises Construction Budget. A complete set of the agreed upon Additional Premises Plans, and the agreed upon Additional Premises Construction Budget, shall be initialed by, and distributed to Landlord and Tenant.

(b) Neither the Construction Budgets nor the Plans shall be changed or altered in any way except by change order approved in writing by Landlord and Tenant, which change order shall include any increased price as a result of the change in the Plans ("Change Order"). All Change Orders shall be valid and binding upon Landlord and Tenant only if authorized by written Change Order signed prior to commencement of the work on the portion of Tenant Improvements reflected in the Change Order. In the event a Change Order is submitted to Tenant and is not approved by Tenant within sufficient time for Landlord to implement the change to Tenant's Improvements, provided Tenant shall be given a minimum of five (5) business days to approve the Change Order, work on the Tenant Improvements shall continue as if the Change Order had never been requested unless if despite the fact that the Change Order will cause a delay, Tenant authorizes the Change Order, then Landlord will make the change provided Tenant agrees that any delay in reviewing and approving the Change Order shall not delay the Commencement Date and Tenant's obligation to pay Rent. The cost or credit to the Tenant due to any Change Order shall be determined per the terms of such Change Order. In the event the Change Order increases the cost set forth in the Construction Budget, then the amount shall be added to the Construction Budget and paid in accordance with payment by Tenant of the cost of Tenant's Improvements, as outlined below. The Landlord shall only have the right to substitute materials and equipment required by the Plans, provided said substitutions conform with applicable building codes, meet specifications and are the subject of a Change Order which is approved by Tenant. Each and every Change Order shall state whether the change will entail a delay in the date of Substantial Completion. Any Change Order requested by Tenant to the extent that it is the sole cause of a delay in the date of Substantial Completion shall delay the Outside Completion Date for the Initial Premises or Additional Premises, as applicable, on a day for day basis. However, any Change Order requested by Landlord, to the extent that it is the sole cause of a delay of the date of Substantial Completion, shall not delay the applicable Outside Completion Date.

3.3 (a) The Landlord may secure and advance payment for the construction permits necessary for the proper execution and completion of the Tenant Improvements. Tenant shall pay such amounts to Landlord not later than 30 days after receipt of an invoice therefor. Landlord shall obtain a temporary or permanent certificate of occupancy or certificate of acceptance (collectively referred to as the "CO") after the Tenant Improvements in the Initial Premises and Additional Premises, as applicable, have been Substantially Completed, as hereafter defined, which permits Tenant to occupy and operate its business within the Leased Premises. If a temporary CO is issued, Landlord shall perform any work necessary to obtain a permanent CO as soon as practicable, but no later than the date that any temporary CO would expire. Landlord shall not, however, be responsible for securing any environmental or operating permits or certifications that are required in order for Tenant to conduct its business. However, to the extent necessary and requested by Tenant, Landlord shall assist Tenant in securing any environmental or operating permits or certifications that are required in order for Tenant to actually conduct its business at no additional cost to the Landlord.

(b) After Substantial Completion of Tenant Improvements for the Initial Premises or Additional Premises, as applicable Tenant shall obtain "as built" Plans at its cost and shall provide Landlord with one reproducible set of the Plans. Landlord will also be provided with a current pdf containing the Plans at no cost to Landlord. Tenant hereby consents to Landlord's use of Tenant's Plans, solely in connection with the Leased Premises and subject to any rights retained by the Architect and Tenant. Tenant also agrees to make commercially reasonable efforts to contract with the Architect to provide Landlord with a CAD disk or disks containing the Plans, at no cost to the Landlord, upon Landlord's written request upon Substantial Completion of Tenant's and Landlord's Improvements and receipt of the CO, and shall further make commercially reasonable effort to obtain consent from the Architect for Landlord's use of the Plans, provided there is no additional cost to Tenant. Architect shall have no obligation to provide further services to Landlord unless and until an agreement mutually acceptable to Architect and Landlord with respect to compensation for such future services is executed by the parties, which Agreement shall not include any unpaid work performed on behalf of Tenant.

3.4 (a) Landlord shall provide Tenant with the following improvements to the Leased Premises and Common Areas, at Landlord's sole cost and expense no later than Substantial Completion of Tenant Improvements in the Initial Premises ("Landlord's Work"):

I. Finish parking lot with stripes, stenciled visitor & handicap parking with lighting as approved by local governing authorities and repair any defects in parking lot so that it is in new condition.;

II. Provide 4000 amp PSEG transformer capacity to Building;

III. Install Building main switchgear;

IV. Install fire service to Building with sufficient flow and pressure to support the facility design of ordinary hazard, group 2 occupancy;

V. Underground sewer main to the point of connection with the Building;

VI. Water service to Building to the point of connection with the Building;

VII. Natural gas capacity to the point of connection with the Building;

VIII. Exterior Building doors and windows in good working order and all portions of the Building, including windows, doors, roof, basement, and any other building penetration or system not associated with Tenant Improvements to be free of leaks;

IX. Install fire sprinkler flow monitoring valves;

X. Five inch concrete slab in single story area with fiber reinforcement. (Any additional work required to the slab beyond this scope shall be at an added cost to Tenant);

XI. Installation of generator pad at a location designated by Tenant based upon Building layout, and reasonably acceptable to Landlord;

XII. Installation of pad in parking lot for trash and recycling staging/pickup and relevant access thereto in the location approved by the Township of Cranbury and as shown on the site plan attached as Exhibit A;

XIII. Installation of an elevator in the two-story section of the Building, which is accessed in the lobby on the first floor and which also accesses the basement;

XIV. It is the understanding of the parties that any improvements required to be constructed outside of the Leased Premises except if serving Tenant's specific business operations rather than the general operation within the Building, shall be constructed by Landlord at its expense, regardless of whether specifically listed herein. The cost of any additional work in the Building required beyond this scope shall be the responsibility of the Tenant.

The entire cost of the construction of Tenant Improvements as contained in the Construction Budgets shall be Tenant's obligation ("Tenant's Cost Share"). Not later than thirty (30) days after approval of the Construction Budget for any Tenant Improvements to be performed by Landlord, Tenant shall promptly pay to Landlord a sum equal to 20% of Tenant's Cost Share. Thereafter, Tenant will be invoiced on a monthly basis for the work performed during the previous thirty-day period, which invoices shall be paid by Tenant no later than thirty (30) days of receipt. Upon Tenant's request, Landlord shall provide evidence to Tenant that all contractors and/or vendors have been paid for work performed to date and funded by Tenant. Upon Substantial Completion of Tenant Improvements and Landlord's Work, Tenant shall pay to Landlord a sum equal to the remaining balance of Tenant's Cost Share no later than thirty (30) days after Tenant's receipt of notice of the final amount of Tenant's Cost Share. In the event Tenant fails to pay to Landlord, upon approval of the Construction Budget, a sum equal to 20% of Tenant's Cost Share, Landlord shall not be obligated to commence work on the Tenant Improvements for the Leased Premises. In the event that Tenant fails to make subsequent payments in accordance with the terms of this Lease, Landlord shall not be obligated to continue the work. Such failure to pay shall constitute a default under this Lease, but shall not delay the Commencement Date of this Lease for any period the Tenant's Share remains unpaid. In the event that Tenant fails to pay to Landlord, upon Substantial Completion of the Tenant Improvements and Landlord's Work, a sum equal to the remaining Tenant's Cost Share, such failure shall constitute a default under this Lease; and Tenant shall not be permitted to occupy the Leased Premises; and Tenant shall continue to make payment of all Rent; and Landlord shall be entitled to all rights and remedies available hereunder, at law or in equity, which rights shall be cumulative. All sums so owing to Landlord shall constitute Additional Rent and shall be subject to the imposition of late charges as provided in this Lease. All payments of Tenant's Cost Share shall be made no later than thirty (30) days after receipt of notice from Landlord of the amount due.

(b) Except for extensions of time for delays, extensions of the Initial Outside Completion Date and Additional Premises Outside Completion Date(s) and payment of Rent and rent abatements as provided herein, no payment or allowance of any kind shall be claimed by Tenant, or made by Landlord as compensation for damages on account of any delay in the Substantial Completion of the Tenant Improvements and Landlord's Work, unless the delay is not a result of Tenant's delay and is a delay instead caused by Landlord, its contractors, employees and/or agents and is avoidable (not caused by force majeure).

3.5 During construction of Tenant Improvements, a representative of Tenant and Landlord shall inspect the site and progress of the work on a schedule to be mutually agreed upon by the parties.

3.6 The Tenant Improvements shall be commenced after approval of the Plans and applicable Construction Budget and receipt by Landlord from the governmental entities having jurisdiction therefor, all permits necessary to commence construction. Subject to the terms hereof, Substantial Completion of the Tenant Improvements in the Initial Premises shall be completed no later than the Initial Outside Completion Date. As used herein the term "Substantial Completion" shall mean that the Tenant Improvements and Landlord Work have been completed in substantial conformity with the Plans, provided any changes in construction which impact Tenant's business operations or any substitution of materials from those shown on the Plans shall be approved by Tenant, and a CO has been issued permitting Tenant to use and occupy the applicable portion of the Leased Premises (the Initial Premises or Additional Premises, as applicable), even though minor details, adjustments or punch list items that do not materially impair Tenant's use and enjoyment of the Leased Premises may not have been finally completed, but which work Landlord shall diligently pursue to final completion. Any delay in Landlord's ability to perform Landlord Work or Tenant Improvements, which delays Landlord's ability to achieve Substantial Completion and is caused solely by performance of Tenant Work shall not delay the Commencement Date by the duration of the delay caused by the performance of Tenant Work.

Tenant shall have the right to provide a punch list of incomplete items ("Punchlist") to Landlord within forty-five (45) days after issuance of the CO for any portion of Tenant Improvements constructed by Landlord, and Landlord shall complete all items on the Punchlist as soon as reasonably practicable thereafter. Tenant shall allow Landlord and its contractors to enter the Leased Premises during normal working hours and upon reasonable advance notice after issuance of the CO to complete remaining minor work and Punchlist items. Upon Tenant's request, Landlord or its agents shall be accompanied by a representative of Tenant. Notwithstanding anything contained herein, Landlord shall not be permitted to enter any portion(s) of the Leased Premises if Legal Requirements prohibit Landlord's access to such portion of the Leased Premises due to confidentiality restrictions. Landlord agrees that its employees, representatives or agents shall not enter any sterile areas within the Leased Premises without following the procedures outlined by Tenant for access to these areas. It is agreed that for the purpose of this Lease, wherever and whenever the term Substantial Completion is used, it shall not include items of maintenance or service or items on the Punchlist.

3.7. CONSTRUCTION IN ADDITIONAL PREMISES

The parties acknowledge that Tenant intends to construct improvements to the Additional Premises after the Commencement Date. Prior to commencing construction of improvements in the Additional Premises ("Additional Construction"), Tenant shall deliver plans and specifications to Landlord. The provisions of Section 3.2(a) and (b) and 3.3(a) and (b) shall be applicable to construction of the Additional Premises; provided, however, Landlord reserves the right to decline and waive its right to perform any phase of the Additional Construction. If Landlord constructs the Additional Premises, Landlord shall supervise and direct the construction of Tenant Improvements using Landlord's best skill and attention, and Landlord shall be solely responsible for all construction means, methods, techniques, sequences, and procedures and for coordinating all portions of the work on the Tenant Improvements in accordance with the plans for the Additional Premises. Landlord warrants to the Tenant that all materials and equipment incorporated by or on behalf of Landlord into the Leased Premises will be new unless otherwise specified or approved by Tenant, and that all work on the Tenant Improvements performed by Landlord will be of good quality, free from known faults and defects (provided that Landlord shall remain responsible to remedy any construction defects which are discovered after the Commencement Date as provided in Section 7.1), and in substantial conformity with the Plans, provided any change in the construction from that shown on the plans which impacts Tenant's business operations or any substitution of materials from those shown on the Plans shall only be made with Tenant's prior written approval. Tenant shall have the right to elect to construct Tenant Improvements in the Additional Premises in phases. If Substantial Completion of the Additional Construction performed by Landlord in any phase of the Additional Premises does not occur by the two hundred eleventh (211th) day following the parties' approval of the Construction Budget for such Additional Construction in the applicable phase (following the parties mutually agreed value engineering and as determined in accordance with Section 3.2 and as evidenced by the parties signature thereon) (the "Additional Outside Completion Date(s)") because of the action or inaction of Landlord or its employees, contractors and/or agents, then, following the Additional Outside Completion Date for such Additional Construction, Tenant shall be entitled to an abatement of rent equivalent to two times the daily Base Rent (determined by dividing the monthly Base Rent payable as of the first day of the Term by thirty (30)) for each day after such Additional Outside Completion Date, that Substantial Completion of such Additional Construction in the Additional Premises has not occurred. Landlord shall not be responsible for any delay in Substantial Completion of the Additional Construction in the Additional Premises due to the action or inaction of Tenant or its employees, contractors and/or agents, or for any delay arising as the result of any Change Orders or other modifications to the Additional Premises Plans requested by Tenant or required to comply with applicable law, rule or regulation or if the Additional Construction includes kitchen equipment or other items which require substantial lead times for procurement or requires permits, licenses or approvals from the Board of Health. Wherever in this Lease reference is made to "Tenant's contractors", it is not intended to include Landlord, if Landlord is performing the Tenant Improvements. If Landlord is not retained to complete the Additional Construction, then Tenant shall comply with the following: (i) not less than 10 business days prior to commencing the Additional Construction, Tenant shall deliver to Landlord final plans, specifications and necessary permits for the Additional Construction, together with certificates evidencing that Tenant's contractors and subcontractors have adequate insurance coverage naming Landlord, and any other associated or affiliated entity as their interests may appear as additional insureds, (ii) Tenant shall obtain Landlord's prior written approval of any contractor or subcontractor which consent shall not be unreasonably withheld, (iii) the Additional Construction shall be constructed with new materials, in a good and workmanlike manner, and in compliance with all Legal Requirements and the plans and specifications delivered to, and approved by Landlord. If Landlord is not the contractor, Tenant shall provide Landlord with as-built plans, in both CAD and PDF format, along with back-up disks, upon completion of the work. All Additional Construction attached to the Building shall become part of the realty immediately upon installation and, except for improvements which Landlord requires Tenant to remove pursuant to this Lease, shall be surrendered with the Leased Premises without payment by Landlord.

4. RENT

4.1 Tenant shall pay, as rent for the Leased Premises, the following:

(a) During the first year of the Term, an annual base rent for the Leased Premises, excluding the basement, of $13.00 per square foot, for an aggregate annual base rent of $1,072,760.00 (“Above-Ground Base Rent”), payable monthly in the sum of $89,396.66. In addition, during the first through the fifth year of the Term, Tenant shall pay an annual base rent of $6.00 per square foot for basement space for annual base rent of $127,200.00 (“Base Basement Rent”, and together with the Above-Ground Base Rent, the “Base Rent”) based upon an occupancy of 21,200 square feet, payable monthly in the sum of $10,600.

(b) Commencing on the first anniversary of the Lease Commencement date, and on every anniversary date of the Commencement Date thereafter, the Above-Ground Base Rent shall be increased by 3%. Commencing on the sixth anniversary of the Lease Commencement Date, and on every anniversary date of the Commencement Date thereafter, Tenant shall pay Base Basement Rent, based upon an escalation from the Commencement Date at the rate of 3% per year. For avoidance of doubt, annual Base Basement Rent during the sixth Lease year shall be $6.96 per square foot. Thereafter the Base Basement Rent shall continue to escalate by 3% on each anniversary of the Commencement Date.

4.2 Tenant shall also pay the following which shall be referred to herein as “Additional Rent”:

(a) Common Area Expenses as hereafter defined in paragraph 8.1.

(b) Any other charges as provided in this Lease.

The Base Rent and Additional Rent shall be referred to hereafter as “Rent”.

4.3 Tenant covenants to pay the Rent in lawful money of the United States which shall be legal tender for the payment of all debts, public and private, at the time of payment. Such Rent shall be paid to Landlord via wire transfer or other electronic transfer to an account provided by Landlord, or at such other place or means as Landlord may, from time to time, designate by notice to Tenant.

4.4 The Rent shall be payable by Tenant without any set-off or deduction of any kind or nature whatsoever and without notice or demand.

5. PARKING AND USE OF EXTERIOR AREA

The Tenant shall have the exclusive right to use all parking spaces located at the Building and designated on Exhibit A as “Tenant Parking”. The Landlord and Tenant mutually agree that they will not block, hinder, or otherwise obstruct the access driveways and parking areas so as to impede the free flow of vehicular traffic within the Office Park, including to the common areas adjacent to the Building. In connection with the use of the loading platforms, if any, Tenant agrees that it will not use the same so as to unreasonably interfere with the use of the access driveways and parking areas. Tenant shall not park or store trailers or other vehicles on any portion of the access driveways in a manner that would impede access to the parking areas, and shall not utilize any portion of the Office Park other than as provided in this Lease, without the prior written consent of Landlord. If at any time a café, restaurant or similar food establishment is open for business in the Office Park, that is of a quality and capacity to reasonably service Tenant’s employees working at the Leased Premises on a daily basis, Tenant shall not authorize any food truck or other vendor to sell food in the parking lot adjacent to the Leased Premises. This provision is not intended to prohibit Tenant from providing food to its employees within the Leased Premises. Tenant shall also have the right to utilize any portion of the Leased Premises to host private events related to its business, including events providing catering or other food service.

6. USE

The Tenant covenants and agrees to use and occupy the Leased Premises only for offices, cleanrooms, development space and laboratories for biotechnology, pharmaceutical or medical device research, production, manufacturing, and testing, and for customary related uses which use is expressly subject to all applicable zoning ordinances, rules and regulations of any governmental instrumentalities, boards or bureaus having jurisdiction thereof ("Zoning Laws"), or any other use permitted by the applicable Zoning Laws. Tenant's use of the Leased Premises shall not interfere with the peaceable and quiet use and enjoyment by other tenants in the Office Park. Tenant's use must comply with all present and future statutes, laws, codes, regulations, ordinances, orders, rules, bylaws, administrative guidelines, requirements, directives and actions of any federal, state or local governmental or quasi-governmental authority, and other legal requirements of whatever kind or nature ("Legal Requirements"). Tenant and Landlord shall not permit any conduct or condition which may endanger, disturb, or otherwise interfere with any other Building occupant's normal operations or with the management of the Building, provided that the management of the Building does not interfere with Tenant's normal business operations. Tenant and Landlord shall not commit any nuisance or excessive noise, and will dispose of all garbage and waste in compliance with Legal Requirements and in a manner that minimizes emissions of dirt, fumes, odors or debris.

7. REPAIRS AND MAINTENANCE

7.1 Tenant shall maintain, and repair the Leased Premises in a good and workmanlike manner, and shall, at the expiration of the Term, deliver the Leased Premises in good order and condition, damages by fire or casualty, the elements and ordinary wear and tear excepted. Tenant covenants and agrees that it shall not cause or permit any waste, damage or disfigurement to the Leased Premises, or any overloading of the floors. Tenant shall maintain, and make all repairs to the floor surface, HVAC, plumbing and electrical systems including all ballasts and fluorescent fixtures located within the Leased Premises. Notwithstanding the foregoing, the Tenant Improvements, including the electrical and mechanical portions of the Tenant Improvements, if constructed by Landlord shall have a Landlord's warranty of two (2) years from the date of Substantial Completion of the applicable Tenant Improvements, except that (a) any defect in construction of the Tenant Improvements discovered at any time during the Lease Term shall not be limited to the two year warranty, and (b) with respect to the operation of the HVAC system, the compressor which shall have a five-year manufacturer's warranty. All warranties shall run from Substantial Completion of that portion of the construction by Landlord of Tenant Improvements. Landlord shall be responsible for repairs to the roof, including the roof membrane, exterior load-bearing walls, and electric and plumbing systems to the point where they enter the Leased Premises and for any condition affecting such systems within the Leased Premises. Landlord shall also be responsible for maintenance, repair and replacement of all improvements constituting "Landlord's Work" pursuant to Section 3.4(a) of the Lease. Landlord shall not be required to make, and Tenant shall be responsible for, any repairs occasioned by the negligent acts or omissions of Tenant, its agents, employees, contractors, or subcontractors. Tenant shall promptly report in writing to Landlord any defective condition which Landlord is required to repair, and, in the event Tenant has actual knowledge thereof, Landlord's obligation to repair is conditioned upon receipt by Landlord of such written notice. Landlord's obligation to repair is also conditioned, at Landlord's option, upon Tenant not then being in default under this Lease after written notice and expiration of any applicable cure period. Landlord shall have no other maintenance or repair obligations whatsoever with respect to the Leased Premises except the foregoing unless caused by the gross negligence or willful act of Landlord. Except to the extent of Landlords' obligations, Tenant shall keep and maintain in good order, condition and repair the Leased Premises and every part thereof, including, without limitation, the interior surfaces of the exterior walls, interior doors, door frames, door checks, windows and window frames, all wall and floor coverings, all building systems and components thereof that exclusively service the Leased Premises, and alterations, additions or improvements ("Alterations") made by or on behalf of Tenant and shall make all other interior non-structural repairs, replacements, renewals and restorations, ordinary and extraordinary, foreseen and unforeseen, required to be made in and to the Leased Premises. The term "repair" as used in this Section shall include replacements when necessary. Tenant agrees to generally maintain the Leased Premises at a minimum temperature of 45 degrees to prevent the freezing of domestic water and sprinkler pipes and (with respect to the office area of the Leased Premises only) no higher than 78 degrees to prevent humidity, mold and mildew. In the event Tenant vacates the Leased Premises, Tenant shall be required to (i) continuously operate the HVAC system to maintain the temperatures set forth in the previous sentence, and (ii) inspect the Leased Premises and, report any defective conditions to Landlord immediately, and confirm upon request of the Landlord that such inspections have taken place.

7.2 From and after the earlier of (i) commencement by AES of the AES Work, or (ii) the Commencement Date, Tenant shall, at its own cost and expense, pay all electric, gas, water, sewer and other utility charges servicing the Leased Premises. Landlord shall install, a water meter at the Leased Premises at its cost and expense. Tenant shall not store any items outside the Leased Premises, and shall deliver its garbage and recyclables to the central receiving area as shown on Exhibit A. Tenant shall dispose of all hazardous/medical waste with an approved hauler at its own cost.

7.3 Landlord does not warrant that any services Landlord or any public utilities supply will not be interrupted. Services may be interrupted because of accidents, repairs, alterations, improvements, or any other reason beyond the reasonable control of Landlord and Landlord, except for in connection with the gross negligence or willful misconduct of Landlord or its agents or employees, shall not be subject to liability as a result thereof. Notwithstanding the above, if essential services (water, electric or gas) are interrupted for more than six (6) days (excluding days declared as a state of emergency by the State of New Jersey) except if such interruption is caused by Tenant's failure to maintain and repair the Leased Premises, and such interruption shall prevent Tenant from operating its business in the normal course, then Tenant shall be entitled to an abatement of Base Rent from and after the six (6) days until service is restored.

8. COMMON AREA EXPENSES, TAXES AND INSURANCE

8.1 Based upon a Building area of 103,720 square feet, the Tenant shall pay to the Landlord, monthly, as Additional Rent the cost of the following items, all of which shall be known as Common Area Expenses:

(a) The costs incurred by the Landlord for the operation, maintenance, and repair of the Common Areas in the Office Park, including Tenant's Parking ("Operating Costs"), which costs to Tenant ("Tenant's Share of OC") shall be $3.20 per square foot for calendar year 2018 and shall be adjusted each January 1st commencing on January 1, 2019 by three (3%) percent, including the following:

(1) lawns and landscaping

(2) exterior sewer lines;

(3) exterior utility lines which are not maintained by a public utility company;

(4) repair and maintenance of any signs furnished and installed by Landlord serving the Office Park;

(5) snow removal from all parking lots, driveways and walkways;

(6) standard trash disposal and recycling;

(7) ground maintenance and maintenance of the parking lot, driveways, and walkways;

(8) maintenance contracts for the roof;

(9) pest control;

(10) central station monitoring for fire sprinkler system; and

(11) other ordinary maintenance expenses normally incurred by Landlord relating to the Building (excluding any costs associated with the elevator, including maintenance and service of elevator) and common areas of the Office Park;

The $3.20/square foot, as increased annually, shall include the cost of the annual insurance premiums charged to the Landlord for insurance coverage which insure the buildings in the Office Park. The insurance shall be for the full replacement value of all insurable improvements with any customary extensions of coverage including, but not limited to, vandalism, malicious mischief, sprinkler damage and comprehensive liability, and insurance for one year's rent. The Landlord shall maintain said insurance in effect at all times hereunder. Any increase in the insurance premiums due to a change in rating of the Building to the extent attributable to Tenant's use, or due to special Tenant equipment, shall be paid entirely by the Tenant, except to the extent that the increase is due to construction of the Tenant Improvements and occupancy by Tenant of the Leased Premises which was otherwise a vacant building. Tenant expressly acknowledges that Landlord shall not maintain insurance on Tenant's furniture, laboratory fixtures, machinery, inventory, equipment or other personal property; and

(b) Tenant shall pay all real estate taxes assessed by governmental authorities against the Building and Property directly to Cranbury Township. Tenant shall provide evidence of payment of taxes upon request by Landlord after the date taxes are due. Nonpayment of these taxes prior to assessment of late fees shall be considered a default. In the event Landlord pays any delinquent taxes, Tenant shall be charged interest on the taxes and any penalties paid by Landlord, at the rate of 1.5% per month; and

(c) A management fee of 3% of the Tenant's Base Rent.

8.2 Tenant's Share of Operating Costs for any calendar year, part of which falls within the term of this Lease and part of which does not, shall be appropriately prorated.

8.3 If at any time during the term of this Lease the method or scope of taxation for real estate taxes prevailing at the commencement of the Lease Term shall be altered, Tenant's substituted tax or imposition shall be payable and discharged by the Tenant in the manner required pursuant to the law which shall authorize such change.

8.4 If at any time during the Term of the Lease any portion of the Building is leased by Landlord to another tenant, including any portion of the basement, then the Tenant's obligation to pay Real Estate Taxes shall be reduced to exclude the proportionate share of such Real Estate taxes attributable to the portion of the Building leased to another tenant. In addition, if at any time during the Lease term Landlord recaptures any portion of the Building, then Tenant's obligation to pay Operating Expenses shall be reduced to exclude the proportionate share of such Operating Expenses attributable to the portion of the Building recaptured by Landlord.

8.5 Tenant, at all times and at its expense, shall keep in effect commercial general liability insurance, including contractual liability insurance, covering Tenant's use of the Leased Premises, with such coverages and limits of liability as Landlord may reasonably require, but not less than a $2,000,000 combined single limit with a $5,000,000 general aggregate limit (which may be satisfied by an umbrella liability policy) for bodily injury or property damage and no less than $300,000.00 for property damage, with a deductible of no more than $20,000.00; however, such limits shall not limit Tenant's liability hereunder. The policy shall name Landlord, and at Landlord's written request, any mortgagee(s), as additional insureds, shall be written on an "occurrence" basis and not on a "claims made" basis and shall be endorsed to provide that it is primary to and not contributory to any policies carried by Landlord and to provide that it shall not be cancelable or reduced without 10 days prior notice to Landlord for nonpayment of premium, and at least 30 days prior notice to Landlord for all other reasons. The insurer shall be authorized to issue such insurance, licensed to do business and admitted in the state in which the Office Park is located and rated at least A VII in the most current edition of Best's Insurance Reports. Tenant shall deliver to Landlord on or before the Commencement Date or any earlier date on which Tenant accesses the Leased Premises, and at least 30 days prior to the date of each policy renewal, a certificate of insurance evidencing such coverage. Tenant shall at all times, at its own cost and expense, carry sufficient "All Risk" property insurance on a replacement cost basis to avoid any coinsurance penalties in applicable policies on all of Tenant's furniture, furnishings, fixtures, machinery, equipment and installations as well as on any Tenant Alterations. Such coverage is to include property undergoing additions and alterations, and shall cover the value of equipment and supplies awaiting installations.

(c) Landlord and Tenant each waive, and release each other from and against, all claims for recovery against the other for any loss or damage to the property of such party arising out of fire or other casualty coverable by the insurance required to be maintained under the Lease. This waiver and release is effective regardless of whether the releasing party actually maintains said insurance and is not limited to the amount of insurance actually carried, or to the actual proceeds received after a loss. Each party shall have its insurance company that issues its property coverage waive any rights of subrogation, and shall have the insurance company include an endorsement acknowledging this waiver, if necessary.

(d) Tenant shall have the right to file an appeal to reduce the real estate taxes for the Property at its sole cost and expense, and any reduction and reimbursement in taxes for the Property shall accrue solely for the benefit of Tenant.

9. SIGNS

Tenant shall not place any signs in the Office Park without the prior consent of Landlord, other than an identification sign with Tenant's name on the entry door to the Leased Premises, and signs that are located wholly within the interior of the Leased Premises. Tenant shall maintain all signs installed by Tenant in good condition. Tenant shall remove its signs at the termination of this Lease, shall repair any resulting damage. Landlord shall provide Tenant with a prominent listing, including Tenant's logo, on the two Building monument signs at the entrance to the Property. Tenant may also, at Tenant's expense, place a ground sign with Tenant's name on it at the entrance of the Building, subject to Landlord's approval of the size, design, and placement location of such sign.

10. ASSIGNMENT AND SUBLETTING

10.1 (a) Except as provided below, Tenant shall not enter into nor permit (i) any assignment, transfer, pledge or other encumbrance of all or a portion of Tenant's interest in this Lease, (ii) any sublease, license or concession of all or a portion of Tenant's interest in the Leased Premises, or (iii) any transfer of a controlling interest in Tenant voluntarily or by operation of law (collectively, "Transfer") without the prior written consent of Landlord. Landlord shall not unreasonably withhold or delay its consent if the following conditions are satisfied (i) the proposed transferee is not an existing tenant of Landlord or Landlord's affiliate in the Office Park, (ii) the business, business reputation or creditworthiness of the proposed transferee is acceptable to Landlord, and (iii) there is no Event of Default under the Lease at the time Tenant requests Landlord's consent. Consent to one Transfer shall not be deemed to be consent to any subsequent Transfer. In no event shall any Transfer relieve Tenant from any obligation under this Lease. Landlord's acceptance of Rent from any person shall not be deemed to be a waiver by Landlord of any provision of this Lease or to be consent to any Transfer except that any Rent accepted by Landlord shall offset any outstanding Rent owed by Tenant. Any Transfer not in conformity with this Section shall be void at the option of Landlord.

(b) Landlord's consent shall not be required in the event of any Transfer by Tenant to an Affiliate (defined as (i) any entity controlling, controlled by, or under common control of, Tenant, (ii) any successor to Tenant by merger, consolidation or reorganization, and (iii) any purchaser of all, substantially all of the assets of Tenant located in the Premises, as a going concern) provided that (i) the transferee has a tangible net worth at least equal to that of Tenant as of the date of this Lease, (ii) Tenant provides Landlord notice of the Transfer no later than 15 days after the effective date of the Transfer, (iii) upon written request by Landlord, Tenant provides copies of the current financial statements of the transferee certified by an executive officer of the transferee, and (iv) in the case of an assignment or sublease, Tenant delivers to Landlord an assumption or sublease agreement reasonably acceptable to Landlord executed by Tenant and the transferee.

(c) The provisions of subsection (a) above notwithstanding, if Tenant proposes to Transfer all of the Leased Premises (other than to an Affiliate), Landlord may terminate this Lease, and Landlord may condition the termination on execution of a new lease between Landlord and the proposed transferee. If Tenant proposes to enter into a Transfer of less than all of the Leased Premises (other than to an Affiliate), Landlord may amend this Lease to remove the portion of the Leased Premises to be transferred, and Landlord may condition the amendment on execution of a new lease between Landlord and the proposed transferee. If this Lease is not so terminated or amended, Tenant shall pay to Landlord monthly, 50% of the excess of (i) all compensation received by Tenant for the Transfer of the Lease over (ii) the Rent allocable to the Leased Premises transferred, less Tenant's reasonable expenses of marketing the space and paying brokerage commissions, which Landlord shall provide the Tenant with evidence of such expenditures.

(d) If Tenant requests Landlord's consent to a Transfer, Tenant shall upon written request by Landlord provide copies of the current financial statements of the transferee certified by an executive officer of the transferee, a complete copy of the proposed Transfer documents, and any other information Landlord reasonably requests. Landlord shall notify Tenant within 10 days after receipt of the foregoing, whether Landlord is granting or withholding consent, or, if (c) applies, whether Landlord elects to terminate the Lease. Immediately following any approved assignment or sublease, Tenant shall deliver to Landlord an assumption agreement reasonably acceptable to Landlord executed by Tenant and the transferee, together with a certificate of insurance evidencing the transferee's compliance with the insurance requirements of Tenant under this Lease. Tenant agrees to reimburse Landlord for reasonable administrative and attorneys' fees incurred by Landlord in connection with the processing and documentation of any Transfer for which Landlord's consent is requested, not to exceed $3,000.

10.4 In the event of any assignment or subletting permitted by the Landlord, the Tenant shall remain and be directly and primarily responsible for payment and performance of the within Lease obligations, except if Landlord elects to terminate the Lease with respect to any portion or all of the Leased Premises in accordance with this Section 10, and the Landlord reserves the right, at all times, to require and demand that the Tenant pay and perform the terms and conditions of this Lease. In the case of a complete recapture of all or a portion of the Leased Premises, Tenant shall be released from all further liability with respect to the recaptured space. No such assignment or subletting shall be made to any Tenant who shall occupy the Leased Premises for any use other than that which is permitted to the Tenant, except with Landlord's consent, which shall not be unreasonably withheld, or for any use which may be deemed inappropriate for the Building or extra hazardous, or which would in any way violate applicable Legal Requirements.

11. FIRE AND CASUALTY

11.1 In case of any damage to or destruction of any portion of the Building of which the Leased Premises is a part by fire or other casualty occurring during the term of this Lease (or prior thereto), which shall render at least 1/3 of the floor area of the Leased Premises or the building untenantable or unfit for occupancy ("Total Destruction"), which damage cannot be repaired within 180 days from the happening of such casualty, using reasonable diligence, as determined in a report prepared by an independent engineer, then the term hereby created shall, at the option of the Landlord, upon written notice to the Tenant within 15 days of such fire or casualty, cease and become null and void from the date of such Total Destruction unless within fifteen (15) days of Landlord's notice of Total Destruction Tenant sends notice to Landlord that it elects to continue the Lease notwithstanding the fact that the Leased Premises cannot be repaired within 180 days. In the event of the termination, the Tenant shall immediately surrender the Leased Premises to the Landlord and this Lease shall terminate. The Tenant shall only pay Rent to the time of such Total Destruction. However, in the event of Total Destruction if the Landlord shall elect not to cancel this Lease within the 15 day period the Landlord shall repair and restore the Building to substantially the same condition as it was prior to the damage or destruction, with reasonable speed and dispatch, and in all events within 180 days, or if Tenant sends notice to Landlord that it elects to continue the Lease, Landlord shall repair and restore the Building to substantially the same condition as it was prior to the damage or destruction, with reasonable speed and dispatch, and in all events within the timeframe stated in the independent engineer's report. The Rent shall not be accrued after said damage or while the repairs and restorations are being made, but shall recommence upon 30 days notice from Landlord that the Leased Premises are substantially restored as evidenced by the issuance of a CO by municipal authorities. In any case where Landlord must restore, consideration shall be given for delays under the Force Majeure paragraph in this Lease. Whether or not this Lease has been terminated as a result of a casualty, in every instance, all insurance proceeds payable under policies of insurance carried by Landlord as a result of damage or destruction to the Building shall be paid to Landlord as its sole and exclusive property.

11.2 In the event of any other casualty which shall not be tantamount to Total Destruction the Landlord shall repair and restore the Building and the Leased Premises to substantially the same condition as they were prior to the damage or destruction, but not Tenant's personal property, furnishings, inventory, fixtures or equipment, with reasonable speed and dispatch. Such repairs will not exceed 180 days from the date of the casualty. The Rent shall abate or shall be equitably apportioned as to any portion of the Leased Premises which shall be unfit for occupancy by the Tenant, or which cannot be used by the Tenant to conduct its business in the ordinary course. The Rent shall recommence 30 days after notice from Landlord that the Leased Premises has been substantially restored, as evidenced by the issuance of a CO by municipal authorities.

11.3 In the event of any casualty caused by an event which is not covered by Landlord's insurance policy; the Landlord may elect to treat the casualty as though it had insurance or it may terminate the Lease. If it treats the casualty as though it had insurance then the provisions of this paragraph shall apply. The Landlord shall serve a written notice upon the Tenant within 15 days of the casualty specifying the election which it chooses to make.

11.4 In the event the Landlord rebuilds, the Tenant agrees, at its cost and expense, to forthwith remove any and all of its equipment, fixtures, stock and personal property to the extent necessary to permit Landlord to expedite the construction unless such costs would be covered by Landlord's insurance. The Tenant shall assume at its sole risk the responsibility for damage to or security of such fixtures and equipment in the event that any portion of the Building area has been damaged and is not secure.

12. COMPLIANCE WITH LAWS, RULES AND REGULATIONS

12.1. Compliance with Legal Requirements

(a) Tenant covenants and agrees that it will, at its own cost, promptly comply with and carry out all Legal Requirements, including, but not limited to Environmental Laws, as defined below, to the extent that same apply to the manner of Tenant's occupation or use of the Leased Premises, the conduct of Tenant's business therein, the construction of any Alterations to the Leased Premises by or on behalf of Tenant, any termination of this Lease and surrender of possession by Tenant, or any acts, omissions or other activities of Tenant in or on the Office Park. Subject to the foregoing, to the extent that any Legal Requirements require modifications to the Leased Premises or the Building, in order to bring same into compliance with Legal Requirements and such Legal Requirements were in effect prior to the Commencement Date and are not Tenant's responsibility under this Section, Landlord shall be responsible for the compliance of such items with such Legal Requirements at Landlord's cost.

(b) The Tenant agrees, at its own cost and expense, to comply with such regulations or requests as may be required by the fire or liability insurance carriers providing insurance for the Leased Premises, and the Board of Fire Underwriters, in connection with Tenant's use and occupancy of the Leased Premises.

(c) In case the Tenant shall fail to comply with Legal Requirements, then Landlord may, after 10 days' written notice (except for emergency repairs, which may be made immediately), enter the Leased Premises and take any reasonable actions to comply with them, at the cost and expense of the Tenant if Tenant has not otherwise commenced and then diligently pursued such actions as are necessary to comply with Legal Requirements. In addition to Landlord's rights and remedies by reason of default by Tenant, the cost thereof shall be added to the next month's Rent and shall be due and payable as such.

12.2. Compliance with Environmental Laws.

(a) "Environmental Laws" are defined herein as all present or future federal, state or local laws, ordinances, rules, executive orders or regulations (including the rules and regulations of the federal Environmental Protection Agency and comparable state agency) relating to the protection of human health or the environment including, but not limited to the Comprehensive Environmental Response Compensation and Liability Act of 1980, 42 U.S.C. 9601 et seq.. ("CERCLA"); the Industrial Site Recovery Act, N.J.S.A. 13:lK-6 et seq., ("ISRA"); the New Jersey Spill Compensation and Control Act, N.J.S.A. 58:10-23.11 et seq., ("Spill Act"): the Solid Waste Management Act, N.J.S.A. 13:1E-1 et seq.. ("SWMA"); the Resource Conservation and Recovery Act, 42 U.S.C. 6901 et seq..("RCRA"); the New Jersey Underground Storage of Hazardous Substances Act, NJ.S.A. 58:10A-21 et seq., ("USTA"); the Clean Air Act, 42 U.S.C. Section 7401 et seq., ("CAA"); the Air Pollution Control Act, NJ.S.A. 26:2C-1 et seq. ("APCA"); the New Jersey Water Pollution Control Act, N.J.S.A. 58:10A-1 et seq., ("WPCA"); and any rules or regulations promulgated thereunder or in any other applicable federal, state or local law, rule or regulation dealing with environmental protection.

(b) For purposes of Environmental Laws, to the extent authorized by law, Tenant is and shall be deemed to be the responsible party, including without limitation, the "owner" and "operator" of Tenant's "facility" (but not the "owner" of the Property) and the "owner" of all Hazardous Materials brought on the Leased Premises and/or Property by Tenant, its agents, employees, contractors or invitees, and the wastes, by-products, or residues generated, resulting, or produced therefrom.

(c) Tenant agrees that: (i) no activity will be conducted on the Leased Premises that will use or produce any pollutants, contaminants, toxic or hazardous wastes or other materials the removal of which is required or the use of which is regulated, restricted, or prohibited by any Environmental Law ("Hazardous Materials,") except for activities which are part of the ordinary course of Tenant's business and are conducted in accordance with all Environmental Laws, ("Permitted Activities"); "Hazardous Materials" includes any pollutant, dangerous substance, toxic substances, any hazardous chemical, hazardous substance, hazardous pollutant, hazardous waste or any similar term as defined in or pursuant to the (i) CERCLA; (ii) RCRA; (iii) ISRA; (iv) Spill Act; (v) USTA; (vi) WPCA; (vii) APCA; (viii) SWMA; (ix) CAA; and (x) USTA and any rules or regulations promulgated thereunder or in any other applicable federal, state or local law, rule or regulation dealing with environmental protection (it is understood and agreed that the provisions contained in this Lease shall be applicable notwithstanding whether any substance shall not have been deemed to be a Hazardous Material at the time of its use or release); (ii) the Leased Premises will not be used for storage of any Hazardous Materials, except for materials used in the Permitted Activities which are properly stored in a manner and location complying with all Environmental Laws; (iii) no portion of the Leased Premises or real property on which the Leased Premises is located (the "Property") will be used by Tenant or Tenant's Agents for disposal of Hazardous Materials except in accordance with Environmental Laws; (iv) Tenant will deliver to Landlord copies of all Material Safety Data Sheets and other written information prepared by manufacturers, importers or suppliers of any chemical on compact disks or electronic format acceptable to Landlord; and (v) Tenant will immediately notify Landlord of any violation by Tenant or Tenant's Agents of any Environmental Laws or the release or suspected release of Hazardous Materials in, under or about the Leased Premises, and Tenant shall immediately deliver to Landlord a copy of any notice, filing or permit sent or received by Tenant with respect to the foregoing. "Release" shall mean the spilling, leaking, disposing, pumping, pouring, discharging, emitting emptying, ejecting, depositing, injecting, leaching, escaping or dumping however defined, and whether intentional or unintentional, of any Hazardous Material.

(d) Tenant shall take immediate steps to halt, remedy or cure any release of a Hazardous Material in under or about the Leased Premises to the extent caused by the Tenant or by its use of the Leased Premises. If at any time during or after the Term, any portion of the Property is found to be contaminated by Tenant or Tenant's Agents or subject to conditions prohibited in this Lease caused by Tenant or Tenant's Agents or Tenant's invitees, Tenant will indemnify, defend and hold Landlord harmless from all claims, demands, actions, liabilities, costs, expenses, attorneys' fees, damages and obligations of any nature arising from or as a result thereof, and Landlord shall have the right to direct remediation activities, all of which shall be performed at Tenant's cost and in a manner in compliance with Environmental Laws. Such remediation shall be completed without the use of Engineering Controls or Institutional Controls (as those terms are defined at N.J.A.C. 7:26E-1.8)("Controls") except to the extent such Controls are in place or required to address conditions that are not the responsibility of Tenant hereunder. Tenant shall perform such work at any time during the period of the Lease upon written request by Landlord or, in the absence of a specific request by Landlord, before Tenant's right to possession of the Leased Premises and/or Property terminates or expires to the extent practicable. Tenant's obligations pursuant to this subsection shall survive the expiration or termination of this Lease. If Tenant fails to perform such work within the reasonable time period specified by Landlord or before Tenant's right to possession terminates or expires (whichever is earlier), Landlord may at its discretion, and without waiving any other remedy available under this Lease or at law or equity (including without limitation an action to compel Tenant to perform such work), perform such work at Tenant's cost. Tenant shall pay all costs reasonably incurred by Landlord in performing such work within twenty (20) days after Landlord's request therefor. Such work performed by Landlord is on behalf of Tenant and Tenant remains the owner, generator, operator, transporter, and/or arranger of the Hazardous Materials for purposes of Environmental Laws. Tenant agrees not to enter into any agreement with any person, including without limitation any governmental authority, regarding the removal of Hazardous Materials that have been released onto or from the Leased Premises without the written approval of the Landlord, which approval shall not be unreasonably withheld, conditioned or delayed.

(e) Tenant hereby represents and warrants that its North American Industrial Classification System ("NAICS") classification, as defined by the most recent edition of the NAICS United States Manual is 541710 . Tenant hereby agrees that it shall promptly inform Landlord of any change in its NAICS number and obtain Landlord's consent for any change in the nature of the business to be conducted in the Leased Premises. If Tenant's operations on the Premises constitute an "Industrial Establishment" (as that term is defined by ISRA) Tenant shall comply with ISRA, the regulations promulgated thereunder and any amending and successor legislation and regulations (including, without limitation, the New Jersey Site Remediation Reform Act, N.J.S.A. 58:10C-1 et seq., referred to herein as "SRRA") by obtaining one of the following: (i) a de minimis quantity exemption; (ii) a Response Action Outcome with respect to the Leased Premises; or (iii) such confirmation that indicates that the New Jersey Department of Environmental Protection has confirmed that ISRA compliance has been achieved ("ISRA Clearance"). Tenant shall make all submissions to, provide all information to, and comply with all requirements of, the New Jersey Department of Environmental Protection ("NJDEP") and a Licensed Site Remediation Professional (as this term is defined under SRRA, herein referred to as an "LSRP") as selected by Tenant as necessary to accomplish ISRA Clearance. Without limitation of the foregoing, Tenant's obligations shall include (i) the proper filing, with the NJDEP, of an initial notice under NJ.S.A. 13:lK-9(a) and (ii) the performance of all remediation and other requirements of ISRA, including without limitation all requirements of N.J.S.A. 13:lK-9(b) through and including (l). However, if the timing of compliance with ISRA is triggered by an act of Landlord (such as by Landlord's sale of the Property) the Landlord shall be responsible for all costs (including reasonable consultant and legal fees and filing fees) associated with Initial Notice submissions needed to achieve ISRA compliance.

(f) In the event that ISRA Clearance, if required, is not delivered to the Landlord prior to surrender of the Leased Premises by the Tenant to the Landlord, to the extent the failure to obtain ISRA clearance precludes Landlord from leasing the Leased Premises to another party at fair market rents, it is understood and agreed that the Tenant shall be liable to pay to the Landlord an amount equal to 200% of the Base Rent then in effect, together with all applicable Additional Rent from the date of such surrender until such ISRA Clearance is delivered to the Landlord, and together with any costs and expenses reasonably incurred by Landlord in enforcing Tenant's obligations under this paragraph.

(g) In addition to the above, Tenant agrees that it shall cooperate with Landlord in the event ISRA is applicable to any portion of the Property. In such case, Tenant agrees that it shall fully cooperate with Landlord in connection with any information or documentation which may be requested by the NJDEP or the relevant LSRP. In the event that any remediation of the Property is required in connection with the conduct by Tenant of its business at the Leased Premises, Tenant expressly covenants and agrees that it shall be responsible for the remediation attributable to the Tenant's operation and Tenant shall, at Tenant's own expense, prepare and submit the required plans and financial assurances, and carry out the approved remediation plans.

(h) Tenant shall indemnify, defend and hold Landlord harmless from and against any and all losses (including, without limitation, diminution in value of the Premises or the Property), claims, demands, actions, suits, damages (excluding punitive damages from the indemnification to the extent that such damages result from acts or omissions of Landlord), reasonable expenses (including, without limitation, remediation, removal, repair, corrective action, or clean up expenses), and reasonable costs (including, without limitation, actual attorneys' fees, consultant fees or expert fees) which are brought or are recoverable against, or suffered or incurred by Landlord to the extent resulting from any breach of the requirements under this Section 12 by Tenant, its agents, employees, contractors, subtenants, assignees or invitees, regardless of whether Tenant had knowledge of such non-compliance.

(i) Notwithstanding anything in this Lease to the contrary, the liability of the Tenant, and any indemnities provided by the Tenant hereunder, shall not extend to Hazardous Materials that were placed on the Leased Premises, in the Building, or on the Office Park by Landlord, by any of Landlord's Agents, or by any current or former tenant of the Office Park other than Tenant. In addition, Landlord shall not include in Additional Rent or Operating Costs, or pass on to Tenant directly or indirectly, the cost incurred by Landlord in monitoring, reporting, testing, abating and/or removing Hazardous Materials that were contained in the Leased Premises, in the Building and/or on the Office Park unless caused by Tenant or Tenant's Agents.

(j) Landlord's Indemnity. Landlord hereby represents that, to the best of its knowledge, as of the date of this Lease, there are no Hazardous Substances located in the Office Park which violate any Environmental Laws. Landlord shall comply with all applicable Environmental Laws, and shall indemnify, defend, and hold harmless Tenant from and against any and all liabilities, damages, claims, losses, judgments, causes of action, and reasonable costs and expenses (including the reasonable fees and expenses of counsel) that may be incurred by Tenant or threatened against Tenant, relating to or arising out of Hazardous Substances located on, in or under the Office Park as of the Commencement Date, or were introduced onto the Office Park after the Commencement Date that are not Tenant's responsibility hereunder.

12.3 The covenants of this section 12 shall survive the expiration or earlier termination of the Lease term.

13. INSPECTION BY LANDLORD

Tenant agrees that Landlord shall have the right to enter into the Leased Premises during business hours for the purpose of examining the same upon reasonable advance written notice of not less than 24 hours (except in the event of emergency), or to make such repairs as are necessary, to exhibit the Leased Premises to mortgagees or prospective mortgagees or purchasers, and during the last 12 months of the Term, to prospective tenants. Upon Tenant's request, Landlord or its agents shall be accompanied by a representative of Tenant. Notwithstanding anything contained herein, Landlord shall not be permitted to enter any portion(s) of the Leased Premises if Legal Requirements prohibit Landlord's access to such portion of the Premises due to confidentiality restrictions. Landlord agrees that its employees, representatives or agents shall not enter any sterile areas within the Leased Premises without following the procedures outlined by Tenant for access to these areas. Any entry or repair shall not materially interfere with Tenant's use of or access to the Leased Premises. Tenant agrees that if Tenant has ceased business operations in the Leased Premises and vacated the Leased Premises, Landlord shall have the right to enter into the Leased Premises at all hours for any reason without notice. If Tenant vacates the Leased Premises, Tenant shall immediately give Landlord a copy of all keys and swipe cards and Landlord shall have the right to enter the Leased Premises at any time.

14. DEFAULT BY TENANT

14.1 Each of the following shall be deemed a default ("Event of Default") by Tenant and a breach of this Lease:

(a) (1) filing of a petition by the Tenant for adjudication as a bankrupt entity, or for reorganization, or for an arrangement under any federal or state statute, except in a Chapter 11 Bankruptcy where the Rent stipulated herein is being paid and the terms of the Lease are being complied with;

(2) dissolution or liquidation of the Tenant;

(3) appointment of a permanent receiver or a permanent trustee of all or substantially all of the property of the Tenant, if such appointment shall not be vacated within 60 days, provided the Rent stipulated herein is being paid and the terms of the Lease are being complied with, during said 60-day period;

(4) taking possession of the property of the Tenant by a governmental officer or agency pursuant to statutory authority for dissolution, rehabilitation, reorganization or liquidation of the Tenant if such taking of possession shall not be vacated within 60 days, provided the Rent stipulated herein is being paid and the terms of the Lease are being complied with, during said 60-day period;

(5) making by the Tenant of an assignment for the benefit of creditors; and

(6) abandonment, desertion or vacation of the Leased Premises by the Tenant, unless Tenant employs at least one individual in the Leased Premises on a full-time basis for the purpose of maintaining the HVAC system and observing the Leased Premises.

(b) if Tenant defaults in the payment of Rent or any other sums due under the Lease when due and such default continues for five business days after written notice thereof from Landlord, provided however, that if Landlord has delivered two such written notices of default to Tenant in any 12-month period, then any subsequent default in the payment of Rent or any other sums due under the Lease which is not paid within five business days after the date it is due shall constitute an Event of Default without requirement of any written notice of nonpayment.

(c) if Tenant shall, whether by action or inaction, be in default of any other obligations under this Lease for 30 business days after written notice thereof from Landlord. The foregoing notwithstanding, if (i) such default cannot reasonably be cured within such 30-day period despite Tenant's due diligence, (ii) the continuance of the cure period beyond 30 business days after Landlord's default written notice will not subject Landlord or any mortgagee of Landlord to prosecution for a crime or any other civil or criminal fine or charge, or otherwise violate applicable Laws, subject the Office Park, or any part thereof, to being condemned or vacated, subject the Office Park, or any part thereof, to any lien or encumbrance, or result in the foreclosure of any mortgage or deed of trust on the Office Park, (iii) no emergency exists, and (iv) Tenant advises Landlord in writing within the initial 30 business day period of Tenant's intention to take all steps necessary to cure such default and duly commences and thereafter diligently and continuously prosecutes to completion all steps necessary to cure such default, then such 30-day cure period shall be extended for a reasonable period of time as necessary under the circumstances for Tenant to cure such default (but in no event shall the cure period be extended beyond 75 days after the date of Landlord's default written notice to Tenant).

(d) if Tenant shall assign this Lease or sublet the Leased Premises or any portion thereof in violation of the requirements of the Lease.

14.2 Upon the occurrence of an Event of Default, Landlord shall have the following remedies, in addition to any and all other rights and remedies provided by law or otherwise provided in this Lease, any one or more of which Landlord may resort to cumulatively, consecutively, or in the alternative:

(a) Landlord may continue this Lease in full force and effect, and collect Rent when due.

(b) Landlord may terminate this Lease upon written notice to Tenant to such effect, in which event this Lease (and all of Tenant's rights hereunder) shall immediately terminate, but such termination shall not affect those obligations of Tenant which are intended by their terms to survive the expiration or termination of this Lease, nor Tenant's obligation to pay damages as set forth below. This Lease may also be terminated by a judgment specifically providing for termination.

(c) Landlord may terminate Tenant's right of possession without terminating this Lease, in which event Tenant's right of possession of the Leased Premises shall immediately terminate, but this Lease shall continue subject to the effect of this Section. Landlord may, but shall not be obligated to, perform any defaulted obligation of Tenant, and to recover from Tenant, as Additional Rent, the reasonable and actual costs incurred by Landlord in performing such obligation. Landlord may only exercise its rights under this Section with such prior written notice as may be reasonable under the circumstances in the event of any one or more of the following circumstances is present: (i) there exists a reasonable risk of prosecution of Landlord unless such obligation is performed sooner than the stated cure period; (ii) there exists an emergency arising out of the defaulted obligation; or (iii) the Tenant has failed to obtain insurance required by this Lease, or such insurance has been canceled by the insurer without being timely replaced by Tenant, as required herein.

(d) Landlord shall have the right to recover damages from Tenant, as set forth in the following Section. Upon any termination of this Lease or of Tenant's right of possession, Landlord, at its sole election, may (i) re-enter and take possession of the Leased Premises and all the remaining improvements or property, (ii) eject Tenant or any of the Tenant's subtenants, assignees or other person or persons claiming any right under or through Tenant, (iii) remove all property from the Leased Premises and store the same in a public warehouse or elsewhere at Tenant's expense, and/or (iv) deem such property to be abandoned, and, in such event, Landlord may dispose of such property at Tenant's expense, free from any claim by Tenant or anyone claiming by, through or under Tenant. Landlord shall use reasonable commercial efforts to relet the Leased Premises after recovering possession of the Leased Premises. It shall not constitute a constructive or other termination of this Lease or Tenant's right to possession if Landlord (A) exercises its right to repair or maintain the Leased Premises, (B) performs any unperformed obligations of Tenant, (C) stores or removes Tenant's property from the Leased Premises after Tenant's dispossession, (D) attempts to relet, or, in fact, does relet, the Leased Premises or (E) seeks the appointment of a receiver on Landlord's initiative to protect Landlord's interest under this Lease.

15. DAMAGES

(a) Upon any termination of this Lease or Tenant's right of possession, or any reentry by Landlord under Section 14 of the Lease, or under any summary dispossession or other proceeding or action or any provision of law by reason of any Event of Default by Tenant, then in addition to the aggregate amount of Rent which Tenant has failed to pay under this Lease through the date of termination or re-entry (as the case may be) and any other damages recoverable by Landlord under applicable state law or this Lease, Tenant shall pay to Landlord as damages, at Landlord's election, either:

(i) a lump sum which shall be immediately due and payable by Tenant and which, at the time of termination of this Lease or any such reentry by Landlord, as the case may be, represents the excess of (a) the aggregate amount of the Base Rent and Additional Rent which would have been payable by Tenant (conclusively presuming that the average monthly Additional Rent is the same as was payable for the 12 calendar months prior to such termination or reentry, or if less than 12 calendar months have elapsed since the Rent Commencement Date, then all of the calendar months preceding such termination or reentry) for the period commencing with such termination or reentry, as the case may be, and ending with the Expiration Date, over (b) the aggregate amount of Rent that Tenant proves should reasonably have been received by Landlord for the same period (taking into account an appropriate vacancy period to seek and obtain a replacement tenant and time to fit the Leased Premises out for such tenant's occupancy, during which Landlord cannot reasonably be expected to receive rent), which excess amount shall be discounted to present value using a discount rate equal to the lesser of (A) the prime rate of interest announced from time to time in the "Money Rates" column of The Wall Street Journal (or any successor column published by The Wall Street Journal, or if there be none, such index of the then prevailing "prime rate" of interest as designated by Landlord) plus 1%, or (B) 6% per annum; or

(ii) sums equal to the Base Rent and Additional Rent provided for in this Lease which would have been payable by Tenant had this Lease not been terminated, or Landlord had not so reentered, payable upon the due dates specified herein for such payments following such termination or reentry until the Expiration Date.

(b) In addition, Tenant shall immediately become liable to Landlord for all damages proximately caused by Tenant's breach of its obligations under this Lease, including all costs Landlord realizes and incurs through the use of a third party in reletting (or attempting to relet) the Leased Premises or any part thereof, including, without limitation, third party brokers' commissions, expenses of a vendor for cleaning the Leased Premises for new tenants, reasonable outside legal fees and all other like third party expenses properly chargeable against the Leased Premises and the rental received therefrom and like costs. If Landlord relets the Leased Premises (or any portion thereof), such reletting may be for a period shorter or longer than the remaining Term, and upon such terms and conditions as Landlord deems appropriate, in its reasonable discretion, and Tenant shall have no interest in any sums collected by Landlord in connection with such reletting (except as a credit against any damages payable by Tenant) except to the extent expressly set forth herein. Landlord shall use commercially reasonable efforts to mitigate its damages hereunder, provided that Landlord (i) shall not be obligated to show preference for reletting the Leased Premises over any other vacant space in the Building; (ii) may divide the Leased Premises, as Landlord deems appropriate, (iii) may relet the whole or any portion of the Leased Premises upon such terms as it deems appropriate, and may grant any rental or other lease concessions as it reasonably deems advisable under prevailing market conditions, including rent abatements for a portion of the term; and (iv) Landlord's obligation to mitigate damages shall be deemed satisfied by its providing adequate information to a commercial third party broker as to the availability of such space (based on a customary brokerage fee being earned by such broker), having the Leased Premises available for inspection by prospective tenants during reasonable business hours, and by acceptance of a commercially reasonable offer for the Leased Premises from a creditworthy person or entity based on a form of lease agreement which is substantially the same as the form utilized for other space tenants in the Building. If Landlord shall succeed in reletting the Leased Premises during the period in which Tenant is paying monthly rent damages, Landlord shall credit Tenant with the net rents collected by Landlord from such reletting, after first deducting from the gross rents, as and when collected by Landlord, (A) all third party expenses incurred or paid by Landlord in collecting such rents, and (B) any theretofore unrecovered costs associated with the termination of this Lease or Landlord's reentry into the Leased Premises, including any theretofore unrecovered expenses of reletting and other damages payable hereunder. If the Leased Premises or any portion thereof be relet by Landlord for the unexpired portion of the Term before presentation of proof of such damages to any court, commission or tribunal, the amount of rent reserved upon such reletting shall, prima facie, constitute the fair and reasonable rental value for the Leased Premises, or part thereof, so relet for the term of the reletting. Provided in all cases that Landlord has acted in a commercially reasonable manner and in conformance with this Section 15. Landlord shall not be liable in any way whatsoever for its failure or refusal to relet the Leased Premises, or if the Leased Premises or any part are relet, for its failure to collect the rent under such reletting, and no such refusal or failure to relet or failure to collect rent shall release or affect Tenant's liability for damages or otherwise under this Lease.

(c) Notwithstanding anything to the contrary contained in this Lease, Landlord shall not make any claim against Tenant for (i) any damage to, or loss of, any property of Landlord or any other person, or (ii) special, consequential, indirect or punitive damages. Landlord hereby waives all claims against Tenant with respect to the foregoing. The provisions of this Section 15(c) shall survive the expiration or earlier termination of the Lease.

16. NOTICES

Any notice, consent or other communication under this Lease shall be in writing and addressed to Landlord or Tenant as follows (or to such other address as either may designate by written notice to the other) with a copy to any mortgagee or other party designated in writing by Landlord:

(a) If to Landlord, one copy to each of the named parties: Cedar Brook 12 Corporate Center, L.P.
4A Cedar Brook Drive
Cranbury, NJ 08512
Attention: Bruce Simon and Aaron Drillick
Email: bsimon@easternproperties.net
adrillick@easternproperties.net

or such other address as Landlord may designate by notice to Tenant;

(b) If to Tenant: Rocket Pharmaceuticals, Inc.
350 Fifth Avenue, Suite 7530
New York, NY 10118
Attention: Sara M. Turken
Email: st@rocketpharma.com

and a copy under separate cover to: Sills, Cummis & Gross, P.C.
1 Riverfront Plaza
Newark, NJ 07102
Attention: Debbie Kramer Gregg, Esq.
Email: dgregg@sillscummis.com

Each written notice shall be deemed given if sent by prepaid overnight delivery service or by certified mail, return receipt requested, postage prepaid or by electronic mail, provided delivery is confirmed and is followed by notice sent by overnight delivery service, with delivery in any case evidenced by a receipt, and shall be deemed to have been given on the day of actual delivery to the intended recipient or on the business day delivery is refused. The giving of written notice by Landlord's or Tenant's attorneys, representatives and agents under this Section shall be deemed to be the acts of Landlord or Tenant, as applicable.

17. NON-WAIVER BY LANDLORD

The failure of Landlord to insist upon the strict performance of any of the terms of this Lease, or to exercise any option contained herein, shall not be construed as a waiver of any such term. Acceptance by Landlord of performance of anything required by this Lease to be performed, with the knowledge of the breach of any term of this Lease, shall not be deemed a waiver of such breach, nor shall acceptance of Rent in a lesser amount than is due (regardless of any endorsement on any check, or any statement in any letter accompanying any payment of Rent) be construed either as an accord and satisfaction or in any manner other than as payment on account of the earliest Rent then unpaid by Tenant. No waiver by Landlord of any term of this Lease shall be deemed to have been made unless expressed in writing and signed by Landlord.

18. ALTERATIONS

Tenant shall have the right to make non-structural Alterations to the Leased Premises without Landlord's consent provided the cost does not exceed a total of $200,000 in any calendar year. Any other Alterations shall require Landlord's consent which shall not be unreasonably withheld or delayed. Any construction performed in the Additional Premises shall not be considered an alteration and shall be governed by Section 3 of this Lease. At the time Tenant requests Landlord's consent for any Alterations that require Landlord's consent, Tenant shall deliver plans and specifications to Landlord. Landlord shall notify Tenant, within ten (10) business days after receipt of Tenant's plans and specifications, whether Landlord offers to perform the Alterations, along with a draft construction budget. Tenant shall notify Landlord within 10 business days whether Tenant wishes to proceed with the Alterations and whether it elects to retain Landlord to perform the Alterations in accordance with the construction budget provided by Landlord. In the event Landlord consents to the Alterations but does not to perform the work, Tenant shall comply with the following: (i) not less than 10 business days prior to commencing any Alteration, Tenant shall deliver to Landlord final plans, specifications and necessary permits for the Alteration, together with certificates evidencing that Tenant's contractors and subcontractors have adequate insurance coverage naming Landlord, and any other associated or affiliated entity as their interests may appear as additional insureds, (ii) Tenant shall obtain Landlord's prior written approval of any contractor or subcontractor which consent shall not be unreasonably withheld, (iii) the Alteration shall be constructed with new materials, in a good and workmanlike manner, and in compliance with all Legal Requirements and the plans and specifications delivered to, and approved by Landlord. If Landlord is not the contractor, Tenant shall provide Landlord with as-built plans, in both CAD and PDF format, along with back-up disks, upon completion of the work. All Alterations attached to the Building shall become part of the realty immediately upon installation and, except for Alterations which Landlord requires Tenant to remove pursuant to this Lease, shall be surrendered with the Leased Premises without payment by Landlord. If Landlord's consent to the Alterations is conditioned upon Tenant's removal of such Alterations at the expiration or termination of the Lease Term, then Tenant will remove the Alterations and will repair any resulting damage and will restore the Leased Premises to the condition existing prior to the Alteration. If any contractor performing work on behalf of Tenant files a mechanics lien against the Property, then Tenant, within 15 days after receipt of notice that a lien has been filed shall either discharge the lien or post sufficient security in the amount of the lien to guaranty the removal of the lien.

19. NON-LIABILITY OF LANDLORD

Tenant agrees to assume all risk of damage to its property, equipment and fixtures occurring in or about the Leased Premises, whatever the cause of such damage or casualty except if caused by the gross negligence or willful misconduct of Landlord. Landlord shall not be liable for any damage or injury to property or person caused by or resulting from steam, electricity, gas, water, rain, ice or snow, or any leak or flow from or into any part of the Building, or from any damage or injury resulting or arising from any other cause or happening whatsoever (refer to Paragraph 8.4 (c) of this Lease) unless caused by the gross negligence or willful misconduct of Landlord. The Landlord shall not be released from liability if Tenant, its employees, agents, or visitors is injured outside the Leased Premises but within the Office Park through the gross negligence or willful misconduct of the Landlord.

20. RESERVATION OF EASEMENT

Landlord reserves the right, easement and privilege to enter on the Leased Premises in order to install, at its own cost and expense and upon reasonable written notice to Tenant (other than in an emergency) any utility lines and services in connection therewith as may be required by the Landlord provided such installation is performed by Landlord during business hours and does not interfere with Tenant's business operations. Landlord shall indemnify and hold Tenant harmless from and against all damages incurred by Tenant as a result of Landlord's exercise of its rights under this Section. It is understood and agreed that if such work as may be required by Landlord requires any interior installation, or displaces any exterior paving or landscaping, the Landlord shall at its own cost and expense, restore such items, to substantially the same condition as they were before such work.

21. STATEMENT OF ACCEPTANCE

Upon the delivery of the Leased Premises to the Tenant the Tenant covenants and agrees that it will furnish to Landlord a statement which shall set forth the Date of Commencement and the Date of Expiration of the Lease Term.

22. FORCE MAJEURE

Except for the obligation of the Tenant to pay Rent, including Additional Rent, the period of time during which the Landlord or Tenant is prevented from performing any act required to be performed under this Lease by reason of fire, catastrophe, strikes, lockouts, civil commotion, weather conditions, acts of God, government prohibitions or preemptions or embargoes, inability to obtain material or labor by reason of governmental regulations, the act or default of the other party, or other events beyond the reasonable control of Landlord or Tenant, as the case may be, shall be added to the time for performance of such act.

23. STATEMENT BY TENANT

Tenant and Landlord shall at any time and from time to time upon not less than 10 days' prior notice from the other execute, acknowledge and deliver to the party requesting same, a statement in writing, certifying that this Lease is unmodified and in full force and effect (or if there have been modifications, that the same is in full force and effect as modified and stating the modifications), that it is not in default (or if claimed to be in default, stating the amount and nature of the default) and specifying the dates to which the Rent and other charges have been paid in advance.

24. CONDEMNATION

24.1 If (a) all of the Leased Premises are taken by a public authority having the power of eminent domain by condemnation or conveyance in lieu of condemnation, or (b) so much of the Leased Premises or Common Areas is so taken and the remainder is insufficient in Landlord's or Tenant's opinion for the reasonable operation of Tenant's business, then this Lease shall terminate as of the date the condemning authority takes possession. If this Lease is not terminated, Landlord shall restore the Building and/or the Common Areas to a condition as near as reasonably possible to the condition prior to the taking, the Rent shall be abated for the period of time all or a part of the Leased Premises is untenantable in proportion to the square foot area untenantable, and this Lease shall be amended appropriately. The compensation awarded for a taking shall belong to Landlord. Except for any relocation benefits or any other benefits to which Tenant may be entitled, and which do not diminish Landlord's claim, Tenant hereby assigns all claims against the condemning authority to Landlord, including, but not limited to, any claim relating to Tenant's leasehold estate.

25. LANDLORD'S RIGHTS

25.1 The rights and remedies given to the Landlord in this Lease are distinct, separate and cumulative remedies, and no one of them, whether or not exercised by the Landlord, shall be deemed to be in exclusion of any of the others.

25.2 In addition to any other legal remedies for violation or breach of this Lease by the Tenant or by anyone holding or claiming under the Tenant such violation or breach shall be restrainable by injunction at the suit of the Landlord.

25.3 No receipt of money by the Landlord from any receiver, trustee or custodian or debtors in possession shall reinstate, or extend the term of this Lease or affect any notice theretofore given to the Tenant, or to any such receiver, trustee, custodian or debtor in possession, or operate as a waiver or estoppel of the right of the Landlord to recover possession of the Leased Premises for any of the causes therein enumerated by any lawful remedy; and the failure of the Landlord to enforce any covenant or condition by reason of its breach by the Tenant shall not be deemed to void or affect the right of the Landlord to enforce the same covenant or condition on the occasion of any subsequent default or breach.

26. QUIET ENJOYMENT

The Landlord covenants that the Tenant, on paying the Rent and performing the covenants and conditions contained in this Lease, may peaceably and quietly have, hold and enjoy the Leased Premises for the Lease term.

27. SURRENDER OF PREMISES; HOLDOVER

On the last day, or earlier permitted termination of the Lease, Tenant shall quit and surrender the Leased Premises in good and orderly condition and repair (reasonable wear and tear, and damage by fire or other casualty excepted) and shall deliver and surrender the Leased Premises to the Landlord peaceably, together with all Tenant Improvements. All data and communication wiring located within the walls or ceiling of the Leased Premises, whether installed by Tenant or Landlord, shall be surrendered and Tenant shall take no action to impair the then-existing condition thereof. Landlord reserves the right, however, to require the Tenant at its cost and expense to remove any Alterations installed by the Tenant after the Commencement Date, and restore the Leased Premises to its original state, normal wear and tear excepted, subject to the other provisions of this Lease relating to Tenant Improvements and Alterations. If items are to be removed during the Term of the Lease or at the expiration of the Lease, Tenant shall remove them in a manner reasonably acceptable to Landlord, and must repair any damage caused by such removal. Prior to the expiration of the Lease term the Tenant shall have the right to remove Tenant's property identified on Schedule 27 if so desired, from the Leased Premises and shall repair all damage caused by such removal. Notwithstanding the foregoing, Tenant shall not remove any electrical, mechanical, plumbing, HVAC systems or components, or equipment that support any systems or improvements built into the Leased Premises, including casework (cabinets installed to the floors and/or walls), chemistry hoods ducted to exhaust and biological safety cabinets that are ducted to exhaust and shall leave any such systems or improvements in good working order less wear and tear. Tenant shall take no action to impair the then-existing condition thereof. Tenant shall have the right to remove all of its fixtures and equipment, provided any damages caused by such removal shall be repaired by Tenant. Prior to Tenant's occupancy of the Leased Premises, Landlord and Tenant will execute a mutually agreed-upon amendment to this agreement setting forth a list of equipment servicing the Building which is not related to the operation of Tenant's business which Tenant shall not remove in the Leased Premises after the end of the lease term and which will become Landlord's property. Since systems and equipment will change over the Term, Landlord and Tenant, no later than three months prior to the termination of the Lease shall acting in good faith mutually agree upon the equipment and systems servicing the Building that will remain with the Leased Premises or must be removed by Tenant. All property not removed by Tenant shall be deemed abandoned by Tenant, and Landlord reserves the right to remove and dispose such property and charge the reasonable cost of such removal and disposal to the Tenant. If the Leased Premises are not surrendered at the end of the Lease term, it shall constitute a default under the Lease by Tenant, and in addition to any other remedy available to Landlord, the Tenant shall be liable for 125% of the then current Rent for the first two months or any portion thereof that Tenant remains in the Leased Premises and for 200% for any month or portion of any month Tenant remains in the Leased Premises thereafter. These covenants shall survive the termination of the Lease. The parties agree that, so long as no Event of Default then exists and is continuing, Tenant may, at any time, remove the clean room laboratory pods and other portions of Tenant's Work related thereto (collectively, the "Pods"), provided, that, prior to the expiration of the Term (however arising), Tenant shall, at its expense, restore the approximately 25,000 square foot area designated on Schedule 3.1(c) as the Phase 2/3 Cleanrooms and the AES Area (the "Pods Area") to a vanilla box state (which, for the avoidance of doubt, shall include the removal by Tenant of all structural lattices, ductwork, and HVAC and other piping, and remove all epoxy and restore an undamaged concrete slab floor). Upon the occurrence of any Event of Default which is not cured within any applicable cure period, Landlord may, in addition to any other right or remedy herein, either (i) require Tenant to remove the Pods and restore the Pods Area to a vanilla box state as aforesaid, or (ii) require Tenant to transfer, assign and convey the Pods to Landlord (or its designee), free and clear of all liens, claims and encumbrances, in which event the Pods shall remain in the Leased Premises and shall be deemed the sole property of the Landlord (or its designee) (and Tenant shall cause the removal of any and all liens, claims and encumbrances thereon). Upon Tenant's written request delivered no later than nine months prior to the then stated expiration date of the Term, Landlord may waive Tenant's obligation to remove the Pods and restore the Pods Area, provided that any such waiver shall be in writing.

28. INDEMNITY

Anything in this Lease to the contrary notwithstanding, and without limiting the Tenant's obligation to provide insurance hereunder, the Tenant covenants and agrees that it will indemnify, defend and save harmless the Landlord against and from all liabilities, obligations, damages, penalties, claims, costs, charges and expenses, including without limitation reasonable attorneys' fees, which may be imposed upon or incurred by Landlord by reason of any of the following occurring during the term of this Lease:

(a) Any matter, cause or thing arising out of Tenant's use, occupancy, control or management of the Leased Premises and any part thereof.

(b) Any gross negligence on the part of the Tenant or any of its agents, employees, licensees or invitees, arising in or about the Leased Premises.

(c) Any failure on the part of Tenant to perform or comply with any of its covenants, agreements, terms or conditions contained in this Lease.

The foregoing indemnity shall survive termination or expiration of the Lease. Subject to the provisions of paragraph 19, the foregoing shall not require indemnity by Tenant in the event of damage or injury occasioned by the negligence or acts of commission or omission of the Landlord, its agents, servants, or employees or to the extent of any damages covered by insurance carried by Landlord.

Landlord shall promptly notify Tenant of any such claim asserted against it and shall promptly send to Tenant copies of all papers or legal process served upon it in connection with any action or proceeding brought against Landlord.

29. BIND AND CONSTRUE CLAUSE

The terms, covenants and conditions of this Lease shall be binding upon, and inure to the benefit of, each of the parties hereto and their respective heirs, successors, and assigns. If any one of the provisions of this Lease shall be held to be invalid by a court of competent jurisdiction, such adjudication shall not affect the validity or enforceability of the remaining portions of this Lease. The parties each acknowledge to the other that this Lease has been drafted by both parties, after consultation with their respective attorneys, and in the event of any dispute, the provisions are not to be interpreted against either party as the drafter of the Lease.

30. INCLUSIONS

The neuter gender when used herein, shall include all persons and corporations, and words used in the singular shall include words in the plural where the text of the instrument so requires.

31. DEFINITION OF TERM “LANDLORD”

When the term “Landlord” is used in this Lease it shall be construed to mean and include only the entity which is the owner of title to the building. Upon the transfer by the Landlord of the title, the Landlord shall advise the Tenant in writing by certified mail, return receipt requested, of the name of the Landlord’s transferee. In such event, the Landlord shall be automatically freed and relieved from and after the date of such transfer of title of all personal liability with respect to the performance of any of the covenants and obligations on the part of the Landlord herein contained to be performed, provided any such transfer and conveyance by the Landlord is expressly subject to the assumption by the transferee of the obligations of the Landlord hereunder.

32. COVENANTS OF FURTHER ASSURANCES

If, in connection with obtaining financing for the improvements on the Leased Premises, the mortgage lender shall request reasonable modifications in this Lease as a condition to such financing, Tenant will not unreasonably withhold, delay or refuse its consent thereto, provided that such modifications do not in Tenant’s reasonable judgment increase the obligations of Tenant hereunder or materially adversely affect the leasehold interest hereby created or Tenant’s use and enjoyment of the Leased Premises.

33. COVENANT AGAINST LIENS; WAIVER OF LANDLORD LIEN

Tenant agrees that it shall not encumber, or permit to be encumbered; the Leased Premises or the fee thereof by any lien, charge or encumbrance, and Tenant shall have no authority to mortgage or hypothecate this Lease in any way whatsoever. Any violation of this Paragraph shall be considered a breach of this Lease. Tenant promptly shall pay for any labor, services, materials, supplies or equipment furnished to Tenant in or about the Leased Premises. Tenant shall keep the Leased Premises and the Office Park free from any liens arising out of any labor, services, materials, supplies or equipment furnished or alleged to have been furnished to Tenant. Tenant shall take all steps permitted by law in order to avoid the imposition of any such lien. Should any such lien or notice of such lien be filed against the Leased Premises or the Office Park, Tenant shall discharge the same by bonding or otherwise, within 15 business days after Tenant has notice that the lien or claim is filed regardless of the validity of such lien or claim. Landlord hereby waives the right to any Landlord’s lien, statutory or otherwise against any equipment, furniture and personal property owned by Tenant (“Tenant’s Property”). Upon request by Tenant, unless there is an existing Event of Default, Landlord agrees to execute a separate agreement acknowledging the waiver of its right to a Landlord’s lien against Tenant’s Property.

34. SUBORDINATION

This Lease shall be subject and subordinate at all times to the lien of any mortgages or ground leases or other encumbrances now or hereafter placed on the land, Building and Leased Premises without the necessity of any further instrument or act on the part of Tenant to effectuate such subordination. However, Tenant agrees to execute such further documents evidencing the subordination of the Lease to the lien of any mortgage or ground lease reasonably acceptable to Tenant, as shall be desired by Landlord within 5 business days. However, any mortgagee may at any time subordinate its mortgage to this Lease, without Tenant's consent, by giving written notice to Tenant, and this Lease shall then be deemed prior to such mortgage without regard to their respective dates of execution and delivery; provided that such subordination shall not affect any mortgagee's rights with respect to condemnation awards, casualty insurance proceeds, intervening liens or any right which shall arise between the recording of such mortgage and the execution of this Lease. Landlord shall use reasonable efforts to cause any existing or future Lender with a lien against the Leased Premises to enter into a written subordination, non-disturbance and attornment agreement with Tenant on such lender's standard form, whereby such lender agrees that, for so long as Tenant shall not be in default of its obligations hereunder, after the giving of required written notice and the expiration of applicable cure periods, such lender shall not disturb Tenant's rights hereunder in the event of a foreclosure of its security interest in the Building, land or Leased Premises on such lender's standard form.

35. EXCULPATION OF LANDLORD

The word "Landlord" in this Lease includes the Landlord executing this Lease as well as its successors and assigns, each of which shall have the same rights, remedies, powers, authorities and privileges as it would have had it originally signed this Lease as Landlord. Any such person or entity, whether or not named in this Lease, shall have no liability under this Lease after it ceases to hold title to the Leased Premises except for obligations already accrued (and, as to any unapplied portion of Tenant's Security, Landlord shall be relieved of all liability upon transfer of such portion to its successor in interest). Tenant shall look solely to Landlord's successor in interest for the performance of the covenants and obligations of the Landlord hereunder which subsequently accrue. Landlord shall not be deemed to be in default under this Lease unless Tenant gives Landlord written notice specifying the default and Landlord fails to cure the default within a reasonable period following Tenant's notice. In no event shall Landlord be liable to Tenant for any loss of business or profits of Tenant or for consequential, punitive or special damages of any kind. Neither Landlord nor any principal of Landlord nor any owner of the Office Park, whether disclosed or undisclosed, shall have any personal liability with respect to any of the provisions of this Lease or the Leased Premises; Tenant shall look solely to the equity of Landlord in the Office Park for the satisfaction of any claim by Tenant against Landlord and no deficiency judgment or other judgment for money damages shall be entered by Tenant against Landlord.

36. NET RENT

It is the intent of the Landlord and Tenant that this Lease shall yield, net to Landlord, the Base Rent specified and all Additional Rent and charges in each month during the term of the Lease, and that all costs, expenses and obligations of every kind relating to the Leased Premises shall be paid by the Tenant, unless expressly assumed by the Landlord. Nothing in this Section is intended to increase Tenant's obligations as provided in the remainder of this Lease.

37. SECURITY

Concurrent with its execution of this Lease, Tenant is depositing with Landlord the sum of $287,000.00 by check for the Initial Premises, subject to collection, as the security deposit under this Lease (the "Security"). Landlord shall retain such amount as security for the faithful performance of all of the terms, covenants, and conditions of this Lease. Landlord shall in no event be obligated to apply the Security to Rent in arrears or damages for Tenant's default, although Landlord may so apply the Security, at its option. Landlord's right to bring a special proceeding to recover or otherwise obtain possession of the Leased Premises for non-payment of Rent or for any other reason shall not in any event be affected by reason of the fact that Landlord holds the Security. The Security, if not applied toward the payment of Rent in arrears or toward the payment of damages suffered by Landlord by reason of Tenant's default, shall be returned to Tenant without interest within thirty (30) days of the expiration of the Lease, or when this Lease is terminated, but in no event shall the Security be returned until Tenant has vacated the Leased Premises and delivered possession thereof to Landlord in accordance with the terms and provisions of this Lease, which shall be verified by a walk-through by Landlord within ten (10) days after the Leased Premises has been vacated to confirm that the Leased Premises are in the condition required to be at the expiration or termination of the Term. If Landlord repossesses the Leased Premises, because of Tenant's default, Landlord may apply the Security to damages suffered to the date of such repossession and may apply the Security to such damages as may be suffered or shall accrue thereafter by reason of Tenant's default. Except as otherwise required by the Laws, Landlord shall not be obligated to keep the Security as a separate fund and may commingle the Security with its own funds. If Landlord applies the Security in whole or in part against damages incurred by reason of Tenant's default, Tenant shall, upon demand by Landlord, deposit sufficient funds to replenish the Security to the original amount required hereunder. Failure of Tenant to deposit such additional security within 30 days of Landlord's demand therefore shall entitle Landlord to avail itself of the remedies provided in this Lease for nonpayment of Rent by Tenant.

38. BROKERAGE

The parties mutually represent to each other that Cushman and Wakefield of New Jersey LLC (the "Broker") was the only broker involved in the introduction of Tenant to the Landlord and the Leased Premises, negotiation of the Lease Agreement, or consummation of the within transaction, that neither party dealt with any other broker in connection with the Lease, and that neither party will deal with any other broker in connection with this Lease in the future. Landlord shall pay all commissions or other fees due to the Broker in connection with this Lease. In the event that either party violates or is claimed by a third party to have violated this representation, it shall indemnify, defend, and hold the other party harmless from all claims and damages.

39. LATE CHARGES

In addition to any other remedy, a late charge of 1 1/2% per month, retroactive to the date Rent was due, shall be due and payable, without notice from Landlord, on any portion of Rent or other charges not paid within 5 business days of the due date.

40. PRESS RELEASES

Landlord shall have the right to announce the execution of this Lease, and the real estate brokers involved in such press releases as Landlord shall deem advisable, provided that no press release shall identify the name of the Tenant. All press releases are subject to Tenant's prior review and written consent.

41. WAIVER OF JURY TRIAL

Landlord and Tenant both irrevocably waive a trial by jury in any action or proceeding between them or their successors or assigns arising out of this Lease or any of its provisions, or Tenant's use or occupancy of the Leased Premises.

42. LAWS OF NEW JERSEY

Without regard to principles of conflicts of laws, the validity, interpretation, performance and enforcement of this Lease shall be governed by and construed in accordance with the laws of the State of New Jersey. The sole and exclusive venue for any dispute between the parties shall be in Middlesex County, New Jersey.

43. RENEWAL

Provided the Tenant is not in default hereunder, it has the right to renew the Lease two, five-year periods, to commence at the end of the initial or renewed term of this Lease. The renewal shall be upon the same terms and conditions as contained in this Lease, including the Rent Escalation. The option of the Tenant to renew this Lease is expressly conditioned upon the Tenant delivering to the Landlord a notice, in writing, by overnight delivery or certified mail, return receipt requested at least nine months prior to the date fixed for termination of the original Lease term or renewal term, as appropriate.

44. DELETED.

45. TENANT REPRESENTATION

Tenant represents, warrants and covenants that neither Tenant nor any of its officers or directors (i) is listed on the Specially Designated Nationals and Blocked Persons List maintained by the Office of Foreign Asset Control, Department of the Treasury ("OFAC") and all applicable provisions of Title III of the USA Patriot Act or any other publicly available list of terrorists, terrorist organizations or narcotics traffickers maintained by the United States Department of State, the United States Department of Commerce or any other governmental authority; (ii) is listed on the List of Terrorists and List of Disbarred parties maintained by the United State Department of State; or (iii) has been convicted, indicted, arraigned, pleaded no contest or been custodially detained on charges involving money laundering or predicate crimes to money laundering, drug trafficking, terrorist-related activities or other crimes or in connection with the Bank Secrecy Act.

46. LANDLORD INDEMNIFICATION.

Landlord hereby indemnifies, and shall pay, protect and hold Tenant harmless from and against all liabilities, losses, claims, demands, costs, expenses (including attorneys' fees and expenses) and judgments of any nature, (except to the extent Tenant is compensated by insurance maintained by Tenant or Landlord under this Lease and except for such of the foregoing as arising from the negligence or willful misconduct of Tenant, its agents, servants or employees), arising, or alleged to arise, from or in connection with (i) any violation of any Legal Requirement or requirements of any insurance company insuring the Leased Premises, (ii) performance of any labor or services by Landlord or the furnishing of any materials or other property in respect of the Building by Landlord, (iii) any breach or default in the performance of any obligation on Landlord's part to be performed under the terms of this Lease, and (iv) any act or omission of Landlord, or any officer, agent or employee. Landlord shall, at its sole cost and expense, defend any action, suit or proceeding brought against Tenant by reason of any such occurrence with independent counsel selected by Landlord and reasonably acceptable to Tenant. The obligations of Landlord under this Section 46 will survive the expiration or earlier termination of this Lease.

IN WITNESS WHEREOF, the parties hereto have executed this document on the date first above written.

LANDLORD:
CEDAR BROOK 12 CORPORATE CENTER, L.P.

Date: _____

By: _____
Name:
Title:

TENANT:
ROCKET PHARMACEUTICALS, INC.

Date: _____

By: _____
Name:
Title:

EXHIBIT A
SITE PLAN OF PROPERTY
[***]

EXHIBIT B
FLOOR PLAN OF LEASED PREMISES
[***]

Schedule 3.1(b)
[***]

Schedule 3.1(c)
[***]

Schedule 27
[***]

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 June 30, 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: August 8, 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 June 30, 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: August 8, 2019

/s/ John Militello
John Militello
Controller
(Principal Financial and Accounting 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 June 30, 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: August 8, 2019

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

Date: August 8, 2019

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