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 September 30, 2022

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.)**
9 Cedarbrook Drive, Cranbury, NJ	**08512**
(Address of principal executive office)	(Zip Code)

(609) 659-8001
(Registrant's telephone number, including area code)

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

Title of each class	**Trading Symbol(s)**	**Name of each exchange on which registered**
Common Stock, $0.01 par value per share	**RCKT**	**Nasdaq Global Market**

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities 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 ☒

As of November 1, 2022, there were 75,684,423 shares of common stock, $0.01 par value per share, outstanding.

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PART I - FINANCIAL INFORMATION

Cautionary Statement Regarding Forward-Looking Statements

This Quarterly Report on Form 10-Q for the quarter ended September 30, 2022 contains forward-looking statements that involve risks and uncertainties, as well as assumptions that, if they never materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q are forward-looking statements. In some cases, you can identify forward-looking statements by words such as "anticipate," "believe," "contemplate," "continue," "could," "estimate," "expect," "intend," "may," "can," "plan," "potential," "possible" "predict," "project," "seek," "should," "target," "will," "would," or the negative of these words or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- 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 with the FDA and to obtain and maintain FDA or other regulatory authority approval of, or other action with respect to, our product candidates;
- our competitors' activities, including decisions as to the timing of competing product launches, 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, enroll a sufficient number of patients 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 acquire additional businesses, form strategic alliances or create joint ventures and our ability to realize the benefit of such acquisitions, alliances, or joint ventures;
- 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, including the development of our direct manufacturing capabilities for our AAV programs, and any 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 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;
- natural and manmade disasters, including pandemics such as COVID-19, including additional strains of COVID-19, or any other health epidemic, and other force majeures, which could impact our operations, and those of our partners and other participants in the health care industry, and which could adversely impact our clinical studies, preclinical research activities, drug supply and the global economy as a whole;
- the impact of global economic and political developments on our business, including rising inflation and capital market disruptions, the current conflict in Ukraine, economic sanctions and economic slowdowns or recessions that may result from such developments which could harm our research and development efforts as well as the value of our common stock and our ability to access capital markets;
- our ability to close the Renovacor acquisition and realize the anticipated benefits from the transaction;
- 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.

We caution you that the foregoing list may not contain all of the forward-looking statements made in this Quarterly Report on Form 10-Q.

Any forward-looking statements in this Quarterly Report on Form 10-Q reflect our current views with respect to future events or to our future financial performance and involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance, or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. We have included important factors in the cautionary statements included in this Quarterly Report on Form 10-Q, particularly in the "Risk Factors" section incorporated by reference from our Annual Report for the year ended December 31, 2021, on Form 10-K, that could cause actual results or events to differ materially from the forward-looking statements that we make. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures, or investments we may make or enter into.

You should read this Quarterly Report on Form 10-Q and the documents that we have filed as exhibits to this Quarterly Report on Form 10-Q completely and with the understanding that our actual future results, performance, or achievements may be materially different from what we expect. Except as required by law, we assume no obligation to update or revise these forward-looking statements for any reason, even if new information becomes available in the future.

This Quarterly Report on Form 10-Q also contains estimates, projections and other information concerning our industry, our business, and the markets for certain diseases, including data regarding the estimated size of those markets, and the incidence and prevalence of certain medical conditions. Information that is based on estimates, forecasts, projections, market research or similar methodologies is inherently subject to uncertainties and actual events, or circumstances may differ materially from events and circumstances reflected in this information. Unless otherwise expressly stated, we obtained this industry, business, market and other data from reports, research surveys, studies and similar data prepared by market research firms and other third parties, industry, medical and general publications, government data and similar sources. This Quarterly Report contains summaries of certain provisions contained in some of the documents described herein, but reference is made to the actual documents for complete information. All of the summaries are qualified in their entirety by the actual documents. Unless stated otherwise, references in this Quarterly Report to "us," "we," "our," or our "Company" and similar terms refer to Rocket Pharmaceuticals, Inc.

PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

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

	September 30, 2022 (unaudited)	December 31, 2021
Assets		
Current assets:		
Cash and cash equivalents	$ 196,669	$ 232,694
Investments	109,865	156,046
Prepaid expenses and other current assets	4,597	3,319
Total current assets	311,131	392,059
Property and equipment, net	25,613	22,299
Goodwill	30,815	30,815
Restricted cash	1,354	1,343
Deposits	455	455
Operating lease right-of-use assets	1,022	1,569
Finance lease right-of-use asset	46,875	48,480
Total assets	$ 417,265	$ 497,020
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 27,823	$ 19,615
Operating lease liabilities, current	634	863
Finance lease liability, current	1,724	1,689
Total current liabilities	30,181	22,167
Operating lease liabilities, non-current	492	905
Finance lease liability, non-current	19,242	19,144
Other liabilities	37	80
Total liabilities	49,952	42,296
Commitments and contingencies (Note 10)		
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	-	-
Series B convertible preferred stock; 300,000 shares designated as Series B; 0 shares issued and outstanding	-	-
Common stock, $0.01 par value, 120,000,000 shares authorized; 67,838,803 and 64,505,889 shares issued and outstanding at September 30, 2022 and December 31, 2021, respectively	678	645
Additional paid-in capital	1,014,283	946,152
Accumulated other comprehensive loss	(596)	(161)
Accumulated deficit	(647,052)	(491,912)
Total stockholders' equity	367,313	454,724
Total liabilities and stockholders' equity	$ 417,265	$ 497,020

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	43,383	39,621	115,533	92,459
General and administrative	15,105	10,025	39,728	30,456
Total operating expenses	58,488	49,646	155,261	122,915
Loss from operations	(58,488)	(49,646)	(155,261)	(122,915)
Research and development incentives	-	-	-	500
Interest expense	(465)	(534)	(1,395)	(2,514)
Interest and other income, net	1,353	806	2,644	2,218
Amortization of premium on investments - net	(156)	(744)	(1,128)	(2,111)
Net loss	$ (57,756)	$ (50,118)	$ (155,140)	$ (124,822)
Net loss per share - basic and diluted	$ (0.87)	$ (0.79)	$ (2.37)	$ (1.99)
Weighted-average common shares outstanding - basic and diluted	66,215,535	63,825,429	65,406,844	62,828,601

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 September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Net loss	$ (57,756)	$ (50,118)	$ (155,140)	$ (124,822)
Other comprehensive loss				
Net unrealized gain (loss) on investments	169	(16)	(435)	(55)
Total comprehensive loss	$ (57,587)	$ (50,134)	$ (155,575)	$ (124,877)

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

Rocket Pharmaceuticals, Inc.
Consolidated Statements of Stockholders' Equity
For the Three and Nine Months Ended September 30, 2022 and 2021
(in thousands except share amounts)
(unaudited)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income/(Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2021	64,505,889	$ 645	$ 946,152	$ (161)	$ (491,912)	$ 454,724
Issuance of common stock pursuant to exercise of stock options and restricted stock units	16,168	-	76	-	-	76
Unrealized comprehensive loss on investments	-	-	-	(468)	-	(468)
Stock-based compensation	-	-	6,270	-	-	6,270
Net loss	-	-	-	-	(42,982)	(42,982)
Balance at March 31, 2022	64,522,057	645	952,498	(629)	(534,894)	417,620
Issuance of common stock pursuant to exercise of stock options	2,387	-	3	-	-	3
Issuance of common stock pursuant to the at-the-market offering program, net of issuance costs	1,313,450	13	17,229	-	-	17,242
Unrealized comprehensive loss on marketable securities	-	-	-	(136)	-	(136)
Stock-based compensation	-	-	7,369	-	-	7,369
Net loss	-	-	-	-	(54,402)	(54,402)
Balance at June 30, 2022	65,837,894	658	977,099	(765)	(589,296)	387,696
Issuance of common stock pursuant to exercise of stock options	22,437	-	229	-	-	229
Issuance of common stock pursuant to the at-the-market offering program, net of issuance costs	1,978,472	20	29,278	-	-	29,298
Unrealized comprehensive gain on marketable securities	-	-	-	169	-	169
Stock-based compensation	-	-	7,677	-	-	7,677
Net loss	-	-	-	-	(57,756)	(57,756)
Balance at September 30, 2022	67,838,803	$ 678	$ 1,014,283	$ (596)	$ (647,052)	$ 367,313

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income/(Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2020	60,996,367	$ 610	$ 825,794	$ (42)	$ (322,843)	$ 503,519
Issuance of common stock pursuant to exercise of stock options	991,432	9	8,783	-	-	8,792
Unrealized comprehensive loss on investments	-	-	-	(33)	-	(33)
Stock-based compensation	-	-	7,900	-	-	7,900
Net loss	-	-	-	-	(40,179)	(40,179)
Balance at March 31, 2021	61,987,799	619	842,477	(75)	(363,022)	479,999
Issuance of common stock pursuant to exercise of stock options	133,838	2	1,113	-	-	1,115
Issuance of common stock pursuant to conversion of notes	1,326,432	13	35,530	-	-	35,543
Unrealized comprehensive loss on investments	-	-	-	(6)	-	(6)
Stock-based compensation	-	-	7,311	-	-	7,311
Net loss	-	-	-	-	(34,525)	(34,525)
Balance at June 30, 2021	63,448,069	634	886,431	(81)	(397,547)	489,437
Issuance of common stock pursuant to exercise of stock options	21,402	-	284	-	-	284
Issuance of common stock pursuant to conversion of notes	160,614	2	5,148	-	-	5,150
Issuance of common stock, net of issuance costs	812,516	8	26,346	-	-	26,354
Warrant Issuance	-	-	7,578	-	-	7,578
Unrealized comprehensive loss on investments	-	-	-	(16)	-	(16)
Stock-based compensation	-	-	6,989	-	-	6,989
Net loss	-	-	-	-	(50,118)	(50,118)
Balance at September 30, 2021	64,442,601	$ 644	$ 932,776	$ (97)	$ (447,665)	$ 485,658

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

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

	Nine Months Ended September 30, 2022	2021
Operating Activities:		
Net loss	$ (155,140)	$ (124,822)
Adjustments to reconcile net loss to net cash used in operating activities:		
Accretion of discount on convertible notes	-	752
Depreciation and amortization of property and equipment	2,883	2,188
Write down of property and equipment, net	177	-
Right of use asset	1,605	-
Stock-based compensation	21,316	22,200
Accretion of discount on investments, net	1,132	2,089
Expense in connection with warrant issuance	-	7,578
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(1,278)	1,016
Accounts payable and accrued expenses	7,189	(2,842)
Operating lease liabilities	(96)	17
Finance lease liability	134	1,644
Other liabilities	(43)	(42)
Net cash used in operating activities	(122,121)	(90,222)
Investing activities:		
Purchases of investments	(177,460)	(226,484)
Proceeds from maturities of investments	222,074	234,146
Payments made to acquire right of use asset	-	(18)
Purchases of property and equipment	(5,355)	(5,655)
Net cash provided by investing activities	39,259	1,989
Financing activities:		
Issuance of common stock, pursuant to exercise of stock options	308	10,191
Issuance of common stock, net of issuance costs	-	26,354
Issuance of common stock pursuant to the at-the-market offering program, net of issuance costs	46,540	-
Net cash provided by financing activities	46,848	36,545
Net change in cash, cash equivalents and restricted cash	(36,014)	(51,688)
Cash, cash equivalents and restricted cash at beginning of period	234,037	298,666
Cash, cash equivalents and restricted cash at end of period	$ 198,023	$ 246,978
Supplemental disclosure of non-cash financing and investing activities:		
Accrued purchases of property and equipment	$ 1,747	$ 1,132
Unrealized loss on investments	$ (435)	$ (55)
Conversion of convertible notes into common stock	$ -	$ (40,693)
Reclassification of construction in process from finance right of use asset	$ -	$ 39
Supplemental cash flow information:		
Cash paid for interest	$ -	$ 148

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

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

1. Nature of Business

Rocket Pharmaceuticals, Inc. ("Rocket" or the "Company") is a clinical-stage, multi-platform biotechnology company focused on the development of gene therapies, with direct on-target mechanism of action and clear clinical endpoints, for rare and devastating diseases. Rocket has three clinical-stage *ex vivo* lentiviral vector ("LVV") programs. These include programs 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, Leukocyte Adhesion Deficiency-I ("LAD-I"), a genetic disorder that causes the immune system to malfunction and Pyruvate Kinase Deficiency ("PKD"), a rare red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia. Of these, both the Phase 2 FA program and the Phase 1/2 LAD-I program are in registration-enabling studies in the United States ("U.S.") and Europe ("EU"). In addition, in the U.S., Rocket has a clinical stage *in vivo* adeno-associated virus ("AAV") program for Danon disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. The Danon program is currently in an ongoing Phase 1 trial. Additional work on a gene therapy program for the less common FA subtypes C and G is ongoing. The Company has global commercialization and development rights to all of these product candidates under royalty-bearing license agreements.

Effective December 2021, the Company made a decision to no longer pursue Rocket-sponsored clinical evaluation of RP-L401; this program was returned to academic innovators. The Company has opted to focus available resources towards advancement of RP-A501, RP-L102, RP-L201 and RP-L301, based on the clinical data to date and potential for therapeutic advancement in these severe disorders of childhood and young adulthood.

2. Risks and Liquidity

The Company has not generated any revenue and has incurred losses since inception. Operations of the Company are subject to certain risks and uncertainties, including, among others, uncertainty of drug candidate development, technological uncertainty, uncertainty regarding patents and proprietary rights, having no commercial manufacturing experience, 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 product 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 $647.1 million as of September 30, 2022.

On February 28, 2022, the Company entered into a sales agreement (the "Sales Agreement"), with Cowen and Company, LLC ("Cowen"), with respect to an at-the-market offering program pursuant to which the Company may offer and sell, from time to time at its sole discretion, shares of its common stock, par value $0.01 per share, having an aggregate offering price of up to $200,000,000 (the "Shares") through Cowen as its sales agent. As of September 30, 2022, the Company sold 3.3 million shares of common stock for net proceeds of $46.6 million pursuant to the at-the-market offering program (see Note 7). As of September 30, 2022, the Company had $306.5 million of cash, cash equivalents and investments.

On October 6, 2022, the Company completed a follow-on offering (the "Offering") pursuant to which it sold 7,820,000 shares of common stock for net proceeds of $108.2 million. With the proceeds from the Offering and the at-the-market offering program, the Company expects such resources will be sufficient to fund its operating expenses and capital expenditure requirements into the second half of 2024.

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, 2021 included in the Annual Report on Form 10-K filed with the Securities and Exchange Commission ("SEC") on February 28, 2022 ("2021 Form 10-K"). 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 consolidated financial position as of September 30, 2022 and the results of its operations and its cash flows for the three and nine months ended September 30, 2022. The financial data and other information disclosed in these consolidated notes related to the three and nine months ended September 30, 2022 and 2021 are unaudited. The results for the three and nine months ended September 30, 2022 are not necessarily indicative of results to be expected for the year ending December 31, 2022 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 ("U.S. GAAP"). All intercompany accounts have been eliminated in consolidation.

Use of Estimates

The preparation of the consolidated financial statements in conformity with U.S. 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 ("R&D") 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.

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 10 "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:

	September 30, 2022	December 31, 2021
Cash and cash equivalents	$ 196,669	$ 232,694
Restricted cash	1,354	1,343
	$ 198,023	$ 234,037

Income Taxes

In May 2022, the Company received a notice from the New York City Department of Finance regarding an audit of the NYC Biotechnology Credit for the tax periods ended December 31, 2018 through December 31, 2020, which is ongoing as of September 30, 2022.

Reclassifications

Certain reclassifications have been made to the prior year financial statements in order to conform to the current year's presentation.

Significant Accounting Policies

The significant accounting policies used in the preparation of these consolidated financial statements for the three and nine months ended September 30, 2022 are consistent with those disclosed in Note 3 to the consolidated financial statements in the 2021 Form 10-K.

Recent Accounting Pronouncements

There were no recent accounting pronouncements that impacted the Company, or which had a significant effect on the consolidated financial statements.

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 September 30, 2022 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market mutual funds	$ 152,398	$ -	$ -	$ 152,398
	152,398	-	-	152,398
Investments:				
United States Treasury securities	75,329	-	-	75,329
Corporate Bonds	-	34,536	-	34,536
	75,329	34,536	-	109,865
	$ 227,727	$ 34,536	$ -	$ 262,263

	Fair Value Measurements as of December 31, 2021 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market mutual funds	$ 179,900	$ -	$ -	$ 179,900
	179,900	-	-	179,900
Investments:				
United States Treasury securities	44,045	-	-	44,045
Corporate Bonds	-	96,696	-	96,696
Municipal Bonds	-	6,000	-	6,000
Agency Bonds	-	9,305	-	9,305
	44,045	112,001	-	156,046
	$ 223,945	$ 112,001	$ -	$ 335,946

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 corporate, municipal and agency 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 based on quoted prices for similar assets.

5. Property and Equipment, Net

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

	September 30, 2022	December 31, 2021
Laboratory equipment	$ 17,968	$ 12,600
Machinery and equipment	10,807	10,432
Computer equipment	244	218
Furniture and fixtures	2,125	1,963
Leasehold improvements	568	407
Internal use software	1,903	1,902
	33,615	27,522
Less: accumulated depreciation and amortization	(8,002)	(5,223)
	$ 25,613	$ 22,299

During the three and nine months ended September 30, 2022 the Company recognized $1.1 million and $2.9 million of depreciation and amortization expense, respectively. During the three and nine months ended September 30, 2021 the Company recognized $0.8 million and $2.2 million of depreciation and amortization expense, respectively.

6. Accounts Payable and Accrued Expenses

As of September 30, 2022 and December 31, 2021, the Company's accounts payable and accrued expenses consisted of the following:

	September 30, 2022	December 31, 2021
Research and development	$ 17,027	$ 12,082
Property and equipment	1,747	728
Employee compensation	4,793	4,533
Government grant payable	597	597
Professional fees	1,779	1,196
Other	1,880	479
	$ 27,823	$ 19,615

7. Stockholders' Equity

At-the-Market Offering Program

On February 28, 2022, the Company entered into the Sales Agreement with Cowen with respect to an at-the-market offering program pursuant to which the Company may offer and sell, from time to time at its sole discretion, shares through Cowen as its sales agent. The shares to be offered and sold under the Sales Agreement, if any, will be offered and sold pursuant to the Company's shelf registration statement on Form S-3. The Company filed a prospectus supplement with the SEC on February 28, 2022 in connection with the offer and sale of the shares pursuant to the Sales Agreement.

The Company will pay Cowen a cash commission of 3.0% of gross proceeds from the sale of the shares pursuant to the Sales Agreement. The Company has provided Cowen with customary indemnification and contribution rights. The Company reimbursed Cowen for certain expenses incurred in connection with the Sales Agreement. Through September 30, 2022, the Company sold 3.3 million shares under the at-the-market offering program for gross proceeds of $48.0 million, less commissions of $1.4 million for net proceeds of $46.6 million.

8. Stock Based Compensation

Stock Option Valuation

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 were as follows:

	Nine Months Ended September 30, 2022	2021
Risk-free interest rate	2.40%	0.78%
Expected term (in years)	5.82	5.84
Expected volatility	73.21%	69.31%
Expected dividend yield	0.00%	0.00%
Exercise price	$ 15.79	$ 53.98
Fair value of common stock	$ 15.79	$ 53.98

The following table summarizes stock option activity for the nine months ended September 30, 2022, 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, 2021	11,143,761	$ 14.51	5.95	$ 128,817
Granted	2,192,166	15.79	6.30	
Exercised	(30,824)	9.99		167
Cancelled	(574,109)	33.07		
Outstanding as of September 30, 2022	12,730,994	$ 13.91	5.68	$ 88,457
Options vested and exercisable as of September 30, 2022	9,715,600	$ 10.92	4.60	$ 85,081
Options unvested as of September 30, 2022	3,015,394	$ 23.48	9.15	$ 3,376

The weighted average grant-date fair value per share of stock options granted during the nine months ended September 30, 2022, and 2021 was $15.79 and $53.98, respectively.

The total fair value of options vested during the nine months ended September 30, 2022 and 2021 was $26.9 million and $18.2 million, respectively.

Restricted Stock Units ("RSU")

The following table summarizes the Company's RSU activity for the nine months ended September 30, 2022:

	Number of Shares	Weighted Average Grant Date Fair Value
Unvested as of December 31, 2021	23,500	$ 30.61
Granted	939,122	16.10
Vested	(10,168)	62.32
Forfeited	(56,585)	16.10
Unvested as of September 30, 2022	895,869	$ 16.23

Stock-based Compensation

Stock-based compensation expense recognized by award type was as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Stock options	$ 6,694	$ 6,831	$ 19,332	$ 21,872
Restricted stock units	983	158	1,984	328
Total share based compensation expense	$ 7,677	$ 6,989	$ 21,316	$ 22,200

Stock-based compensation expense by classification included within the consolidated statements of operations and comprehensive loss was as follows:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Research and development	$ 3,040	$ 3,084	$ 8,247	$ 9,148
General and administrative	4,637	3,905	13,069	13,052
Total share based compensation expense	$ 7,677	$ 6,989	$ 21,316	$ 22,200

As of September 30, 2022, the Company had an aggregate of $50.1 million of unrecognized stock-based compensation expense related to both stock options and RSU grants, which is expected to be recognized over the weighted average period of 2.05 years.

Warrants

A summary of the warrants outstanding as of September 30, 2022 is as follows:

Exercise Price	Outstanding	Grant Date	Expiration Date
24.42	7,051	June 28, 2013	June 28, 2023
57.11	603,386	December 21, 2020	December 21, 2030
33.63	301,291	August 9, 2021	August 9, 2031
22.51	153,155	December 17, 2021	December 17, 2031
22.51	153,155	December 17, 2021	December 17, 2031
Total	1,218,038		

The following table below is a summary of changes in warrants to purchase common stock for the nine months ended September 30, 2022:

	Number of Warrant Shares Outstanding and Exercisable	Exercise Price per Share
Balance as of December 31, 2021	1,218,038	
Granted	-	$ -
Exercised	-	$ -
Balance as of September 30, 2022	1,218,038	

9. Net Loss Per Share

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

	Three Months Ended September 30, 2022	Three Months Ended September 30, 2021	Nine Months Ended September 30, 2022	Nine Months Ended September 30, 2021
Numerator:				
Net loss attributable to common stockholders	$ (57,756)	$ (50,118)	$ (155,140)	$ (124,822)
Denominator:				
Weighted-average common shares outstanding - basic and diluted	66,215,535	63,825,429	65,406,844	62,828,601
Net loss per share attributable to common stockholders - basic and diluted	$ (0.87)	$ (0.79)	$ (2.37)	$ (1.99)

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 and Nine Months Ended September 30, 2022	2021
Shares issuable upon conversion of the 2021 Convertible Notes	-	160,536
Warrants exercisable for common shares	1,218,038	610,437
Restricted stock units convertible for common shares	895,869	23,500
Options to purchase common shares	12,730,994	11,040,697
	14,844,901	11,835,170

10. Commitments and Contingencies

The Company determines if an arrangement is a lease at inception. Operating and finance leases are presented in the Company's consolidated balance sheet as right-of-use assets from leases, current lease liabilities and long-term 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 using an estimate of the Company's collateralized borrowing rate for debt with a similar term. 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). For operating leases, the Company recognizes the minimum rental expense on a straight-line basis based on the fixed components of a lease arrangement. The Company will amortize this expense over the term of the lease beginning with the lease commencement date. 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.

Finance Lease

The Company has a lease for a facility in Cranbury, New Jersey, consisting of 103,720 square feet of space including areas for offices, process development, research, and development laboratories and 50,000 square feet dedicated to AAV Current Good Manufacturing Practice ("cGMP") manufacturing facilities to support the Company's pipeline (such lease, as amended, the "NJ Lease Agreement"). The NJ Lease Agreement has a 15-year term from September 1, 2019, with an option to renew for two consecutive five-year renewal terms.

Estimated rent payments for the NJ Lease Agreement 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 $29.3 million over the 15-year term of the lease. The Company paid 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 as of September 30, 2022 and December 31, 2021.

Operating Leases

On June 7, 2018, the Company entered into a three-year lease agreement for office space in the Empire State Building in New York, NY (the "ESB Lease Agreement"). In connection with the ESB Lease Agreement, the Company established an irrevocable standby letter of credit (the "Empire LOC") for $0.9 million. On March 26, 2021, the Company entered in Amendment No. 1 to the ESB Lease Agreement ("ESB Lease Amendment") that extended the term of the lease agreement to June 30, 2024, reduced the rent payments going forward, and reduced the Empire LOC to $0.8 million. The Empire LOC serves as the Company's security deposit on the lease in which the landlord is the beneficiary and expires August 29, 2024. The Company has accounted for the ESB Lease Amendment as a modification to the ESB Lease Agreement and remeasured the lease liability and adjusted the operating lease right of use asset by $1.1 million. The Company has a certificate of deposit of $0.8 million with a bank as collateral for the Empire LOC which is classified as part of restricted cash in the consolidated balance sheets as of September 30, 2022 and December 31, 2021.

On January 4, 2018, in connection with the reverse merger with Inotek Pharmaceuticals Corporation ("Inotek"), the Company assumed an operating lease for Inotek's former headquarters in Lexington, Massachusetts, with a term ending in February 2023. In July 2018, the Company signed an agreement to sublease a portion of the Lexington, Massachusetts space and in September 2018, the Company signed an agreement to sublease the remaining portion of the Lexington, Massachusetts space. Rental income received under the sublease agreements totaled $0.1 million and $0.3 million for the three and nine months ended September 30, 2022, respectively. Rental income received under the sublease agreement totaled $0.1 million and $0.3 million for the three and nine months ended September 30, 2021, respectively. Rental income is netted against rent expense in the consolidated statements of operations.

Rent expense was $0.3 million and $0.8 million for the three and nine months ended September 30, 2022, respectively. Rent expense was $0.2 million and $0.8 million for the three and nine months ended September 30, 2021, respectively.

The total restricted cash balance for the Company's operating and finance leases as of each of September 30, 2022 and December 31, 2021 was $0.8 million.

Lease cost	**September 30, 2022**
Operating lease cost	$ 592
Finance lease cost	
Amortization of right of use assets	1,605
Interest on lease liablities	1,395
Total lease cost	$ 3,592

The following table summarizes the maturity of the Company's operating and finance lease liabilities on an undiscounted cash flow basis and a reconciliation to the operating and finance lease liabilities as of September 30, 2022:

Maturity of operating lease liabilities	**September 30, 2022**
2022	230
2023	548
2024	269
2025	64
2026	54
Total lease payments	$ 1,165
Less: interest	(39)
Total operating lease liabilities	$ 1,126

Maturity of finance lease liability	**September 30, 2022**
2022	428
2023	1,736
2024	1,791
2025	1,856
2026	1,912
Thereafter	45,000
Total lease payments	$ 52,723
Less: interest	(31,757)
Total finance lease liability	$ 20,966

Leases	**September 30, 2022**
Operating right-of-use assets	$ 1,022
Operating current lease liabilities	634
Operating noncurrent lease liabilities	492
Total operating lease liabilities	$ 1,126
Finance right-of-use assets	$ 46,875
Finance current lease liability	1,724
Finance noncurrent lease liability	19,242
Total finance lease liability	$ 20,966

Other information	
Cash paid for amounts included in the measurement of lease liabilities:	
Operating cash flows from operating leases	$ 687
Cash flows from finance lease	$ 1,261
Weighted-average remaining lease term - operating leases	2.0 years
Weighted-average remaining lease term - finance lease	21.9 years
Weighted-average discount rate - operating leases	3.94%
Weighted-average discount rate - finance lease	8.96%

Litigation

From time to time, the Company may be subject to 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.

11. Agreements Related to Intellectual Property

The Company, directly and through its subsidiary Spacecraft Seven, LLC, 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.

12. CIRM Grants

LAD-1 CIRM Grant

On April 30, 2019, the California Institute for Regenerative Medicine ("CIRM") awarded the Company up to $7.5 million under a CLIN2 grant award to support the clinical development of its LVV-based gene therapy for RP-L201. Proceeds from the grant will help fund clinical trial costs as well as manufactured drug product for Phase 1/2 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. In 2019, the Company received the first two grants from CIRM in the aggregate of $1.2 million which were included as an offset against R&D expenses. In 2020, the Company met additional CIRM milestones and received an additional $1.1 million milestone which was recorded as a reduction of R&D expenses in 2020. The Company received the additional milestone payments of $1.1 million and $1.0 million in January and April of 2021, respectively. In March 2022, the Company met the next CIRM milestone and recorded a receivable of $0.9 million, included in prepaid and other current assets in the consolidated balance sheet and a reduction of research and development expenses. The Company received the $0.9 million milestone payment on April 5, 2022. No additional milestones were achieved as of September 30, 2022.

13. Related Party Transactions

During April 2018, the Company entered into an agreement with a member of the Board of Directors for business development consulting services. Payments for the services under the agreement are $28 per quarter, and the Company may terminate the agreement with 14 days' notice. This agreement was terminated on February 15, 2022. The Company incurred expenses of $0 for the three and nine months ended September 30, 2022 and $27.5 and $82.5 during the three and nine months ended September 30, 2021, respectively, relating to services provided under this agreement.

In October 2020, the Company entered into a consulting agreement with the spouse of one of the Company's executive officers for information technology advisory services. In exchange for the services provided under the agreement, the Company granted 10,000 restricted stock units which vest over a three-year period.

On August 27, 2021, the Company entered into a Securities Purchase Agreement (the "Purchase Agreement") with a fund affiliated with RTW Investments, LP, the Company's largest shareholder (the "Purchaser"), pursuant to which it agreed to sell and issue to the Purchaser, in a private placement (the "Private Placement"), 812,516 shares of the Company's common stock at a purchase price of $32.48 per share for aggregate net proceeds of approximately $26.4 million to the Company before deducting estimated offering expenses payable by the Company. The Private Placement closed on August 31, 2021. In addition, concurrently with the execution of the Purchase Agreement, the Company entered into a registration rights agreement with the Purchaser, pursuant to which the Company agreed, following demand by the Purchaser, to file with the Securities and Exchange Commission a Registration Statement on Form S-3 covering the resale of shares of common stock held by the Purchaser as promptly as reasonably practicable following such demand, and in any event within 60 days of such demand.

On August 9, 2021, the Company issued a warrant exercisable for 301,291 shares of common stock to a related party for business development and asset identification consulting services ("August 2021 Warrant"). The Company recorded a non-cash R&D expense of $7.6 million during year ended December 31, 2021, related to the issuance of the August 2021 warrant. On December 17, 2021, the Company issued warrants exercisable for 153,155 and 153,155 shares of common stock, respectively to the same related party for business development and asset identification consulting services ("December 2021 Warrants"). The Company recorded a non-cash R&D expense of $5.2 million during year ended December 31, 2021, related to the issuance of the December 2021 warrant. Total non-cash R&D expense of $12.8 million during the year ended December 31, 2021, related to the issuance of the August 2021 and December 2021 warrants. There was no expense related to this item for the three and nine months ended September 30, 2022.

In September 2021, the Company entered into a consulting agreement with a member of the Board of Directors for pipeline development, new asset evaluation, and corporate strategy. In lieu of cash for services to be provided under the consulting agreement during its one-year term, the Company granted the board member options to purchase 20,000 shares of the Company's common stock with a fair value of $0.4 million. The Company incurred expense of $0 and $0.3 million for the three and nine months ended September 30, 2022 and 2021.

14. Renovacor Merger Agreement

On September 19, 2022, the Company entered into an Agreement and Plan of Merger (the "Merger Agreement") with Renovacor, Inc., a Delaware corporation ("Renovacor") pursuant to which, among other matters, and subject to the satisfaction or waiver of the conditions set forth in the Merger Agreement, the Company will acquire Renovacor. The acquisition is intended to qualify for federal income tax purposes as a tax-free reorganization under the provisions of Section 368(a) of the Internal Revenue Code of 1986, as amended. Subject to the terms and conditions of the Merger Agreement, each share of Renovacor's common stock, par value $0.0001 per share ("Renovacor Shares") outstanding immediately prior to the effective time of the merger (including Company Earnout Shares (as defined in the Merger Agreement)) will be canceled and converted into the right to receive a number of fully paid and non-assessable shares of the Company determined on the basis of an exchange formula set forth in the Merger Agreement (the "Exchange Ratio"). The Exchange Ratio will initially be equal to 0.1676 for each Renovacor Share (subject to adjustment as described in the Merger Agreement). Under certain circumstances further described in the Merger Agreement, the Exchange Ratio may be adjusted upward or downward based on the level of Renovacor's net cash at the closing of the merger and certain other adjustments, as determined in accordance with the Merger Agreement. The acquisition is expected to close by the first quarter of 2023. The Company incurred approximately $1.3 million of acquisition related costs during the three months ended September 30, 2022.

15. 401(k) Savings Plan

The Company has a defined contribution savings plan (the "Plan") under Section 401(k) of the Internal Revenue Code of 1986. This Plan covers substantially all employees who meet minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre-tax basis. Company contributions to the Plan may be made at the discretion of the Company's Board of Directors. The Company has elected the safe harbor match of 4% of employee contributions to the Plan, subject to certain limitations. The Company's matching contribution for the three and nine months ended September 30, 2022, was $0.2 million and $0.7 million, respectively. The Company's matching contribution for the three and nine months ended September 30, 2021, was $0.1 million and $0.4 million, respectively.

16. Subsequent Events

On October 6, 2022, the Company completed a follow-on public offering pursuant to which it sold 7,820,000 shares of common stock, which included the full exercise of the underwriters' option to purchase an additional 1,020,000 shares of common stock, at a public offering price of $14.75 per share. The gross proceeds to Rocket from the public offering were approximately $115.3 million, net of $7.1 million of offering costs, commissions, legal and other expenses for net proceeds of $108.2 million.

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 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, filed on February 28, 2022 with the SEC (the "2021 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 2021 Form 10-K, pursuant to Instruction 2 to paragraph of Item 303 of Regulation S-K. 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.

We are a clinical-stage, multi-platform biotechnology company focused on the development of first, only and best-in-class gene therapies, with direct on-target mechanism of action and clear clinical endpoints, for rare and devastating diseases. We have three clinical-stage *ex vivo* lentiviral vector ("LVV") programs. These include programs 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, Leukocyte Adhesion Deficiency-I ("LAD-I"), a genetic disorder that causes the immune system to malfunction and Pyruvate Kinase Deficiency ("PKD"), a rare red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia. Of these, both the Phase 2 FA program and the Phase 1/2 LAD-I program are in potentially registration-enabling studies in the United States ("U.S.") and Europe ("EU"). In addition, in the U.S., we have a clinical stage *in vivo* adeno-associated virus ("AAV") program for Danon disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. The Danon program is currently in an ongoing Phase 1 trial. Additional work on a gene therapy program for the less common FA subtypes C and G is ongoing. We have global commercialization and development rights to all of these product candidates under royalty-bearing license agreements.

Effective December 2021, a decision was made to no longer pursue Rocket-sponsored clinical evaluation of RP-L401; this program was returned to academic innovators. Although we believe that gene therapy may be beneficial to patients afflicted with this disorder, we have opted to focus available resources towards advancement of RP-A501, RP-L102, RP-L201 and RP-L301, based on the compelling clinical data to date and potential for therapeutic advancement in these severe disorders of childhood and young adulthood.

Recent Developments

At-the-Market Offering Program

On February 28, 2022, we entered into the Sales Agreement with Cowen with respect to an at-the-market offering program pursuant to which we may offer and sell, from time to time at its sole discretion, shares through Cowen as our sales agent. The shares to be offered and sold under the Sales Agreement, if any, will be offered and sold pursuant to our shelf registration statement on Form S-3. We filed a prospectus supplement with the SEC on February 28, 2022 in connection with the offer and sale of the shares pursuant to the Sales Agreement. We will pay Cowen a cash commission of 3.0% of gross proceeds from the sale of the shares pursuant to the Sales Agreement. We also agreed to provide Cowen with customary indemnification and contribution rights. We reimbursed Cowen for certain expenses incurred in connection with the Sales Agreement. Through September 30, 2022, we sold 3.3 million shares under the at-the-market offering program for gross proceeds of $48.0 million, less commissions of $1.4 million for net proceeds of $46.6 million.

Renovacor Merger Agreement

On September 19, 2022, we entered into an Agreement and Plan of Merger (the "Merger Agreement") with Renovacor, Inc., a Delaware corporation ("Renovacor") pursuant to which, among other matters, and subject to the satisfaction or waiver of the conditions set forth in the Merger Agreement, we will acquire Renovacor. The acquisition is intended to qualify for federal income tax purposes as a tax-free reorganization under the provisions of Section 368(a) of the Internal Revenue Code of 1986, as amended. Subject to the terms and conditions of the Merger Agreement, each share of Renovacor's common stock, par value $0.0001 per share ("Renovacor Shares") outstanding immediately prior to the effective time of the merger (including Company Earnout Shares (as defined in the Merger Agreement)) will be canceled and converted into the right to receive a number of fully paid and non-assessable shares of the Company determined on the basis of an exchange formula set forth in the Merger Agreement (the "Exchange Ratio"). The Exchange Ratio will initially be equal to 0.1676 for each Renovacor Share (subject to adjustment as described in the Merger Agreement). Under certain circumstances further described in the Merger Agreement, the Exchange Ratio may be adjusted upward or downward based on the level of Renovacor's net cash at the closing of the merger and certain other adjustments, as determined in accordance with the Merger Agreement. The acquisition is expected to close by the first quarter of 2023. We incurred approximately $1.3 million of pre-acquisition related costs during the three months ended September 30, 2022.

Follow-on Public Offering

On October 6, 2022, we completed a public offering of 7,820,000 shares of our common stock, which included the full exercise of the underwriters' option to purchase an additional 1,020,000 shares of our common stock, at a public offering price of $14.75 per share. The gross proceeds to Rocket from the public offering were approximately $115.3 million, net of $7.1 million of offering costs, commissions, legal and other expenses for net proceeds from the offering of $108.2 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.

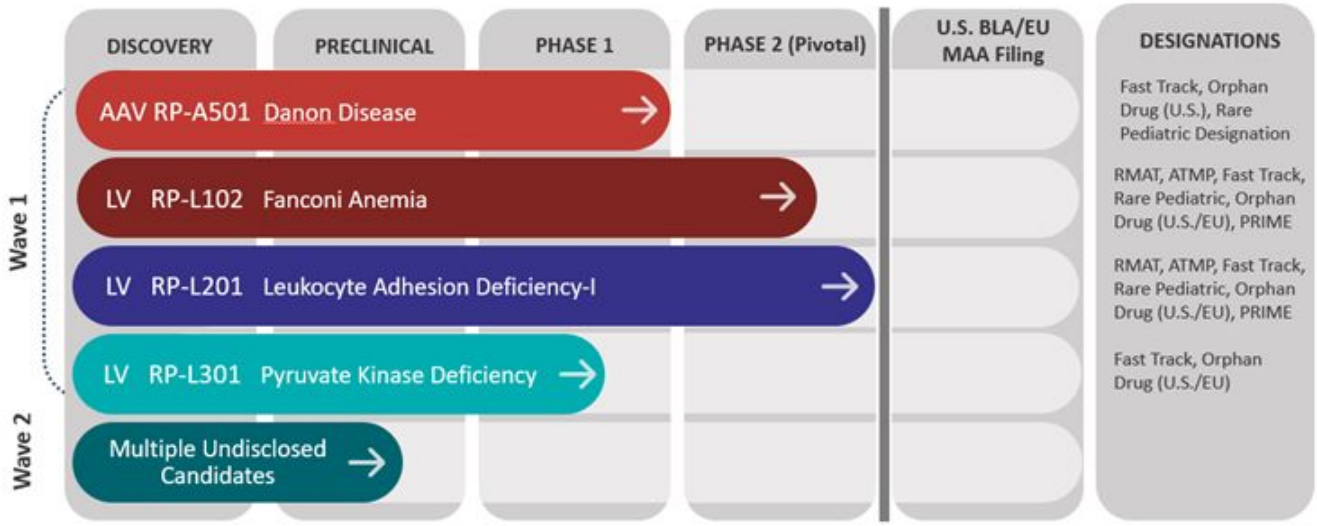
We are using modified non-pathogenic viruses for the development of our gene therapy treatments. Viruses are particularly well suited as delivery vehicles because they are adept at penetrating cells and delivering genetic material inside a cell. In creating our viral delivery vehicles, the viral (pathogenic) 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 FDA approval of several gene therapies in recent years indicates that there is a regulatory pathway forward for gene therapy products.

Pipeline Overview

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



AAV Program:

Danon Disease:

Danon disease ("DD") is a multi-organ lysosomal-associated disorder leading to early death due to heart failure. DD 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 DD-related manifestations can include skeletal muscle weakness and intellectual impairment. There are no specific therapies available for the treatment of DD and medications typically utilized for the treatment of congestive heart failure (CHF) are not believed to modify progression to end-stage CHF. Patients with end-stage CHF may undergo heart transplant, which currently is available to a minority of patients, is associated with significant short- and long-term complications and is not curative of the disorder in the long-term. RP-A501 is in clinical trials 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.

Danon disease is an X-linked dominant, monogenic rare inherited disorder characterized by progressive cardiomyopathy which is almost universally fatal in males even in settings where cardiac transplantation is available. Danon disease predominantly affects males early in life and is characterized by absence of *LAMP2B* expression in the heart and other tissues. Preclinical models of Danon disease have demonstrated that AAV-mediated transduction of the heart results in reconstitution of *LAMP2B* expression and improvement in cardiac function.

We currently have one adeno-associated viral vector program targeting DD, RP-A501. We have treated seven patients in the RP-A501 Phase 1 clinical trial, which enrolled young adult and pediatric male DD patients. This includes a first cohort evaluating a low-dose (6.7e13 genome copies (gc)/kilogram (kg)) in adult/older adolescent patients aged 15 or greater (n=3), a second cohort evaluating a higher dose (1.1e14 gc/kg) in adult/older adolescent patients aged 15 or greater (n=2), and we have initiated treatment in a pediatric cohort at a low dose level (6.7e13 gc/kg; n=2).

Data disclosed from our Phase 1 study of RP-A501 in May 2022 and September 2022 included safety and clinical activity results from the three patients treated with the low dose of 6.7e13 gc/kg and from two patients treated with the higher dose of 1.1e14 gc/kg, and initial safety information from the two pediatric patients (pediatric cohort is age 8-14 years) treated with the low dose of 6.7e13 gc/kg.

Based on the activity observed in the low dose cohort and to mitigate complement-mediated TMA (safety concerns observed in the high dose cohort) and in agreement with the FDA, we are focusing on the low dose (6.7e13 gc/kg) and we will no longer administer doses of 1.1e14 gc/kg or higher in this trial. Additional safety measures have been implemented and are reflected in the updated trial protocol. These measures include exclusion of patients with end-stage heart failure, and a refined immunomodulatory regimen involving transient B- and T-cell mediated inhibition, with emphasis on preventing complement activation, while also enabling lower steroid doses and earlier steroid taper, with all immunosuppressive therapy discontinued 2-3 months following administration of RP-A501. As presented in May 2022 at the 25th Annual Meeting of the American Society of Gene and Cell Therapy (“ASGCT”), two pediatric patients have received RP-A501 therapy (6.7e13 gc/kg dose level). The two pediatric patients were observed to have normal-range platelets, diminished complement activation relative to the adult cohorts, and no complement-related adverse events. Corticosteroid taper commenced at day 10 following therapy for both patients and there was no significant worsening of the patients’ baseline DD-related skeletal myopathy during the weeks following RP-A501. Of note, transaminases and parameters of liver inflammation (including blood levels of gamma-glutamyl transferase, bilirubin and coagulation parameters) were not significantly increased during the weeks following therapy.

In the adult (age ≥15 years) low-dose cohort, RP-A501 was generally well-tolerated. All 4 adult (age ≥15 years) patients with observed immunomodulatory regimen compliance and preserved (>40%) left ventricular ejection fraction at baseline demonstrated disease modification across molecular, echocardiographic, and functional parameters. These patients demonstrated evidence of cardiac LAMP2B expression by immunohistochemistry and reduced levels of autophagic vacuoles on histologic evaluation. Echocardiograms showed stabilized or decreased cardiac wall thickness with improved or stabilized ejection fraction in these patients. Patients in the adult cohorts demonstrated sustained improvement or stabilization in Brain Natriuretic Peptide (“BNP”) and New York Heart Association (“NYHA”) class, 6-minute walk test and reported increases in physical activity. Adverse events were manageable with transient immunomodulation. All treatment-related adverse events in pediatric and adult cohorts were reversible with no lasting renal, hepatic, or other sequelae.

Efficacy assessments include evaluation of NYHA Functional Classification, which is the most commonly used heart failure classification system. NYHA Class II is where a patient exhibits a slight limitation of physical activity, is comfortable at rest, and ordinary physical activity results in fatigue, palpitation and/or dyspnea. Class I is where a patient exhibits no limitation of physical activity and ordinary physical activity does not cause undue fatigue, palpitation and/or dyspnea. Brain natriuretic peptide (BNP) is a blood-based evaluation and a key marker of heart failure with prognostic significance in CHF and cardiomyopathies. High sensitivity troponin I (“hsTnI”) is a blood-based evaluation and a key marker of cardiac injury, one that is (like BNP) frequently elevated in Danon disease patients. Other efficacy parameters include echocardiographic measurements of heart thickness, most notably the thickness of the left ventricular posterior wall, and importantly, measurement of LAMP2B gene expression both via immunohistochemistry and Western blot, as obtained via endomyocardial biopsy. Biopsied heart tissue is also evaluated via hematoxylin and eosin (“H&E”) histology and electron microscopy for evidence of DD-associated tissue derangements, including the presence of autophagic vacuoles and disruption of myofibrillar architecture, each of which are characteristic of DD-related myocardial damage.

In September 2022, interim data for the ongoing Phase 1 trial of RP-A501 was presented at the Heart Failure Society of America ("HFSA") meeting, including updated safety and initial efficacy parameters for the pediatric cohort and longer-term efficacy parameters for the low and high dose adult cohort (patients aged 15 and older; n=5) (data cut-off September 27, 2022). In the pediatric cohort, an improvement in NYHA Class (from Class II to I) were reported in both patients after 6 and 9 months of follow-up post-RP-A501. In the adult cohorts, improvement in NYHA Class (from II to I) was observed in three patients (two low-dose and one high-dose) who had closely monitored immunomodulation and stabilization of NYHA Class was observed in one low-dose adult patient without a closely monitored immunomodulatory regimen. Substantial improvements (reductions) in BNP, a key marker of heart failure, were observed in both pediatric patients at 6 and 9 months of follow-up, with levels at these assessments less than 50% of baseline values. Improvements (reductions) in hsTnI, a key marker of myocardial injury, were observed in both pediatric patients at 6 and 9 months of follow-up, with levels at these assessments less than 20% of baseline values. In the adult cohorts, reductions in hsTnI were observed in three low-dose patients and one high-dose patient, with reductions greater than 50% of baseline levels identified in these four patients on at least one assessment, and reductions sustained through 24-36 months of follow-up. Reductions in BNP of at least 25% below baseline values were identified in three low-dose patients and one high-dose patient on at least one assessment. In two of the adult patients, BNP levels were modestly above baseline at the most recent assessment; however baseline BNP levels were either within normal limits or mildly elevated for these two patients. In adult cohort patients with closely monitored immunomodulation (two low-dose and one high-dose) left ventricular (LV) posterior wall thickness improved (approximately 15-25% decrease compared to pretreatment baseline) and reductions in left ventricular mass were identified in four patients, including the patient in the low-dose cohort for whom immunomodulation was not closely monitored. Severe and progressive wall thickening is a hallmark of the hypertrophic cardiomyopathy of Danon disease and is a major contributor to early mortality in male patients. Evidence of sustained cardiac LAMP2B gene expression by immunohistochemistry with qualitative improvement of vacuoles and cardiac tissue architecture on standard H&E and electron microscopy was observed at both dose levels in four of five patients in the adult cohorts and both patients in the pediatric cohort. Sustained cardiac LAMP2B gene expression by immunohistochemistry was observed in all three adult patients with a closely monitored immunomodulatory regimen through 24 months of follow-up. Importantly, genetic correction (as evidenced by myocardial vector copy numbers ("VCNs") and LAMP2 protein expression were accompanied by reductions in the relative area of autophagic vacuoles relative to overall myocardial area, with decreases in this ratio of at least 20% relative to baseline identified in four adult cohort patients (three of whom had reductions of at least 50%). Substantial reductions (>50% baseline) in vacuolar area were also identified in the one pediatric cohort patient for whom this parameter was evaluable at 6 months post-therapy. In addition to the improvements identified in NYHA Class, improvements in quality of life ("QOL") as reported via the Kansas City Cardiomyopathy Questionnaire ("KCCQ") were noted in three of the adult patients who had closely monitored immunomodulation, and both of the pediatric cohort patients; KCCQ score at baseline was 50 for the initial pediatric patient and was 93 at the most recent 9 month assessment; KCCQ score at baseline was 52 for the second pediatric patient and was 81 at a preliminary 3 month assessment.

RP-A501 was generally well tolerated at the 6.7e13 gc/kg dose level, or lower dose. All observed adverse effects were reversible with no lasting sequelae. Early transaminase and creatinine kinase elevations returned to baseline or decreased. No unexpected and serious drug product-related adverse events or severe adverse events were observed in this low dose cohort. The most common adverse events were predominantly mild, not associated with clinical symptoms and were related to elevated transaminases post-treatment. Elevation in transaminases and creatinine kinases was observed in all three low-dose adult cohort patients and returned to baseline levels within the first one to two months post-treatment. There was also a transient and reversible decline in platelets observed in two of three of these patients. These changes were largely responsive to corticosteroids and other immunosuppressive therapies. All patients were given oral steroids to prevent or minimize potential immune-related events. Corticosteroids were associated with transient exacerbation of DD-associated skeletal myopathy, which resolved upon discontinuation of steroid therapy. At the higher dose administered (1.1e14 gc/kg adult cohort), additional immunosuppressive therapies were stipulated and administered to mitigate the immune response associated with RP-A501. Interim data presented at HFSA in September 2022 included that RP-A501 was observed to be generally well tolerated at the low dose with a manageable safety profile across pediatric and adult cohorts. In the pediatric cohort, RP-A501 was well tolerated in both patients with six to eleven months follow-up. The patients were observed to have normal-range platelets, minimal complement activation and no complement-related adverse events. The two pediatric patients received a modified immunomodulatory regimen to mitigate adverse events. No significant immediate or delayed toxicities, significant skeletal myopathy, or late transaminase elevations have been observed.

Taken together, the totality of data from the six patients currently enrolled in the Phase 1 trial is expected to support advancement toward a Phase 2 pivotal study.

One of the patients receiving therapy on the high dose cohort had progressive heart failure and underwent a heart transplant at Month 5 following therapy. This patient had more advanced disease than the 4 other adult/older adolescent patients who received treatment in the low and high dose cohorts, as evidenced by diminished baseline LV ejection fraction (35%) on echocardiogram and markedly elevated LV filling pressure prior to treatment. His clinical course was characteristic of DD progression. Assessments regarding gene transduction from the explanted heart are summarized below:

Explanted Heart

- Analysis of the explanted heart revealed significant fibrosis consistent with advanced DD.
- Myocardial tissue from the explanted heart at 5 months post-treatment displayed 100% LAMP2B protein expression by immunohistochemistry throughout non-fibrotic cardiac regions including the ventricles and other essential targeted areas

As disclosed in December 2020, this same patient (one of the two patients receiving the 1.1e14 gc/kg dose) had more advanced heart failure than the others, and was the heaviest patient treated to-date (receiving the highest absolute AAV9 dose). This patient experienced a non-persistent, immune-related event that was classified as a drug product-related serious adverse event. This thrombotic microangiopathy ("TMA") event (which was later reclassified as a Sudden Unexpected Serious Adverse Reaction ("SUSAR") was believed to be likely due to immune-mediated complement activation, resulting in reversible thrombocytopenia and acute kidney injury requiring eculizumab and transient hemodialysis. This patient regained normal kidney function within three weeks. (This event occurred in the same patient in whom RP-A501 was not associated with clinical stabilization or improvement, and who required a heart transplant 5 months post-therapy).

Following transplant, this patient has been clinically stable and reports resolution of a baseline skeletal myopathy that was present prior to treatment. Analysis of the explanted heart is described above. Of note, this patient had more advanced heart failure at time of treatment; the clinical protocol has been modified to exclude enrollment of DD with end-stage CHF/cardiomyopathy. In May 2021, 5 months after details of this event were disclosed and after recognition of complement-mediated TMA in other systemic AAV programs, the FDA placed the study on clinical hold. In response to the FDA's clinical hold, we amended the trial protocol in order to enable more defined mechanisms for prevention, early recognition and management of complement-mediated adverse events. The FDA lifted the clinical hold on August 16, 2021 and investigational treatment in the pediatric cohort was initiated in the fourth quarter of 2021. Updated results were presented at HFSA as summarized above.

Anticipated Milestones

Since preliminary clinical activity and ongoing tolerability have been observed in pediatrics along with evidence of longer-term tolerability and clinical activity in adults, we expect these results to support FDA discussions on study design and endpoints for our planned Phase 2 pivotal trial. Phase 2 trial planning activities are expected to begin in the fourth quarter of 2022.

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 our 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. Our 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. We believe that the development of a broadly applicable autologous gene therapy can be transformative for these patients.

Each of our LV-based programs utilize third-generation, self-inactivating lentiviral vectors to correct defects in patients' HSCs, which are the cells found in bone marrow that are capable of generating blood cells over a patient's lifetime. Defects in the genetic coding of HSCs can result in severe, and potentially life-threatening anemia, which is when a patient's blood lacks enough properly functioning red blood cells to carry oxygen throughout the body. Stem cell defects can also result in severe and potentially life-threatening decreases in white blood cells resulting in susceptibility to infections, and in platelets responsible for blood clotting, which may result in severe and potentially life-threatening bleeding episodes. Patients with FA have a genetic defect that prevents the normal repair of genes and chromosomes within blood cells in the bone marrow, which frequently results in the development of acute myeloid leukemia ("AML"), a type of blood cancer, as well as bone marrow failure and congenital defects. The average lifespan of an FA patient is estimated to be 30 to 40 years. The prevalence of FA in the U.S. and EU is estimated to be approximately 4,000 patients in total. In light of the efficacy seen in non-conditioned patients, the addressable annual market opportunity is now believed to be 400 to 500 patients collectively in the U.S. and EU.

We currently have one ex-vivo LV-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 our Phase 2 registrational enabling clinical trials treating FA patients at the Center for Definitive and Curative Medicine at Stanford University School of Medicine ("Stanford"), the University of Minnesota, Great Ormond Street Hospital ("GOSH") in London and Hospital Infantil de Nino Jesus ("HNJ") in Spain. The trial has enrolled a total of ten patients from the U.S. and EU with the first patient in this Phase 2 trial treated in December 2019. Patients will receive a single intravenous infusion of RP-L102 that utilizes fresh cells and "Process B" which incorporates a modified stem cell enrichment process, transduction enhancers, as well as commercial-grade vector and final drug product.

Resistance to mitomycin-C, a DNA damaging agent, in bone marrow stem cells at a minimum time point of one year post treatment is the primary endpoint for our ongoing Phase 2 study. Per agreement with the FDA and EMA, engraftment leading to bone marrow restoration exceeding a 10% mitomycin-C resistance threshold could support a marketing application for approval.

In December 2020, we presented updated interim data from our FA program at the 62nd American Society of Hematology ("ASH") Annual Meeting. The FA data presented at the ASH Annual Meeting were from seven of the nine patients treated (out of twelve patients enrolled) as of October 2020 in both the U.S. Phase 1 and global Phase 2 studies of RP-L102 for FA. Patients in these studies received a single intravenous infusion of "Process B" RP-L102 which incorporates a modified stem cell enrichment process, transduction enhancers, as well as commercial-grade vector. Preliminary data from these studies support "Process B" as a consistent and reproducible improvement over "Process A" which was used in earlier academic FA studies.

Seven patients had follow-up data of at least two-months and three of the seven patients had been followed for twelve-months or longer. As patients are treated with gene therapy product without the use of a conditioning regimen, the data indicated that RP-L102 was generally well-tolerated with no significant safety issues reported with infusion or post-treatment. One drug related serious adverse event of Grade 2 transient infusion-related reaction was observed. In five out of the seven patients for whom there was follow-up data, evidence of preliminary engraftment was observed, with bone marrow ("BM") vector copy numbers ("VCNs") from 0.16 to 0.22 (long-term follow-up only) and peripheral VCNs ranging from 0.01 (2-month follow-up) to 0.11 (long-term follow-up). Further, two of the three patients with greater than 12-months follow-up showed evidence of increasing engraftment, mitomycin-C ("MMC") resistance and stable blood counts, which suggests a halt in the progression of bone marrow failure. The third patient with greater than 12-month follow-up contracted *Influenza B* nine months post-treatment resulting in progressive BM failure, for which, such patient received a successful bone marrow transplant at 18 months post-treatment.

In May 2021, we presented positive clinical data at the 24th Annual Meeting of the ASGCT. The preliminary data from the Phase 1/2 trials presented in a poster at ASGCT were from nine pediatric patients and showed increasing evidence of engraftment in at least six of the nine patients, including two patients with at least 15-months of follow-up and four patients with at least 6-months of follow-up. RP-L102 demonstrated a highly favorable tolerability profile with all subjects being treated without conditioning and with no sign of dysplasia. One patient experienced a Grade 2 transient infusion-related reaction.

In December 2021, we presented encouraging clinical data at the 63rd Annual Meeting of the American Society of Hematology (ASH). The preliminary results from the Phase 1/2 trials presented in a poster at ASH were from eleven pediatric patients and showed increasing evidence of engraftment in at least six of eight patients for whom there are at least 12 months of follow-up, including bone marrow progenitor cell resistance to mitomycin-C (MMC) ranging from 16-63% in six patients (bone marrow cells in FA patients are highly sensitive to DNA-damaging agents including MMC; this susceptibility to DNA damage is believed to mediate the FA-associated bone marrow failure and predisposition to malignancy. In addition to the development of MMC-resistance in BM hematopoietic cells, sustained peripheral VCN levels were seen in six of seven patients with at least 12-months of follow-up. One patient experienced an Influenza B infection approximately 9 months following treatment with concomitant progressive hematologic failure requiring allogeneic hematopoietic stem cell transplant, which was administered successfully; the remaining patients have not required transfusions. RP-L102 demonstrated a highly favorable tolerability profile with all subjects being treated without cytotoxic conditioning and no signs of dysplasia. The only RP-L102 related serious adverse event to-date has been a Grade 2 transient infusion-related reaction in one patient.

In May 2022, we presented updated data for RP-L102 at ASGCT's 25th Annual Meeting. Five of nine evaluable patients as of the April 4, 2022 cut-off date had increased resistance to MMC in bone marrow-derived colony forming cells, ranging from 21% to 42% at 12 to 18 months, increasing to 51% to 94% at 18 – 21 months. The primary endpoint has been achieved, based on a trial protocol in which statistical and clinical significance requires a minimum of five patients to attain increased MMC resistance at least 10% above baseline at two or more timepoints, and concomitant evidence of genetic correction and clinical stabilization. A sixth patient has displayed evidence of progressively increasing genetic correction as evidenced by peripheral VCN. Three additional patients were less than 12 months post-treatment at the time of presentation. One patient had progressive bone marrow failure following therapy and underwent successful allogeneic transplant as previously disclosed. The tolerability profile of RP-L102 appears favorable with no signs of dysplasia, clonal dominance or oncogenic integrations; as previously reported, one patient experienced a Grade 2 transient infusion-related reaction, which resolved.

In October 2022, we presented data for RP-L102 at the European Society for Cell and Gene Therapy 29th Annual Meeting, including the clinical activity results presented at the ASGCT 2022 meeting. We also disclosed at least one of the additional three patients in our Phase 2 trial of RP-L102 for FA for whom there is less than 12 months of follow-up has demonstrated initial evidence of engraftment (as demonstrated by bone marrow mitomycin-C resistance and VCN in blood and bone marrow) at levels comparable to those seen in the five patients for whom there is longer-term evidence of progressive engraftment and phenotypic correction. We also disclosed that one of the initial five patients in this trial who had evidence of engraftment developed a T-cell lymphoblastic lymphoma approximately 22 months after RP-L102 administration. A surgical biopsy of the lymphoma indicated negligible gene markings (VCN of 0.003) at a juncture when concomitant VCN in blood and bone marrow were 0.26 and 0.42 respectively. These findings conclusively indicate that the lymphoma did not result from a LV-mediated insertion, as there were essentially no gene markings in the tumor (the very low but detectable VCN is likely the result of blood cells in the tumor specimen). FA is a cancer-predisposition syndrome and cancers may develop in patients under the age of 10. Importantly, the patient tolerated induction chemotherapy for the lymphoma without significant complications and is currently in a complete response. The presence of gene-corrected hematopoietic cells may have contributed to this patient's overall tolerance of chemotherapy.

Anticipated Milestones

Based on achievement of the primary endpoint as defined in our pivotal Phase 2 study for FA, we have initiated FDA dialogue around biologics license application ("BLA") filing plans for RP-L102 for the treatment of FA and anticipate making such filing in the second half of 2023. Continued data readouts for the FA program are expected in the fourth quarter of 2022.

Leukocyte Adhesion Deficiency-I (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 tissues where these cells are needed to combat infections. As is the case with many rare diseases, accurate estimates of incidence are difficult to confirm; however, several hundred cases 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.

We currently have one *ex*-vivo program targeting LAD-I, RP-L201. RP-L201 is a clinical program that we in-licensed from CIEMAT. We have partnered with UCLA to lead U.S. clinical development efforts for the LAD-I 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, and HNJ and GOSH serving as the lead clinical sites in Spain and London, respectively. This study has received a $7.5 million CLIN2 grant award from the California Institute for Regenerative Medicine ("CIRM") to support the clinical development of gene therapy for LAD-I.

The open-label, single-arm, Phase 1/2 registration-enabling clinical trial of RP-L201 has treated nine severe LAD-I patients to assess the safety and tolerability of RP-L201 to date. The first patient was treated at UCLA with RP-L201 in the third quarter 2019. Enrollment is now complete in both the Phase 1 and 2 portions of the study; nine patients have received RP-L201 at 3 investigative centers in the U.S. and Europe.

In December 2021, we presented positive clinical data at the 63rd Annual Meeting of ASH. The ASH oral presentation included preliminary data from eight of nine severe LAD-I patients, as defined by CD18 expression of less than 2%, who received RP-L201 treatment as of the November 8, 2021, data cut-off date. Eight patients had follow-up data of at least three months, and four of the eight patients had been followed for 12 months or longer. All infusions of RP-L201 were well tolerated and no drug product-related serious adverse events were reported. Evidence of preliminary efficacy was observed in all eight evaluable patients. All eight patients demonstrated neutrophil CD18 expression that exceeded the 4-10% threshold associated with survival into adulthood and consistent with reversal of the severe LAD-I phenotype including six patients with at least 6 months of follow-up. Peripheral blood VCN levels have been stable and in the 0.54 – 2.94 copies per genome range. No patients had LAD-I related infections requiring hospitalization after hematopoietic reconstitution post-RP-L201. Additional updates presented in January 2022 included a ninth patient achieving CD18 expression of 61% at 3 months, with the preliminary observation that all nine of nine patients have demonstrated 26% to 87% CD18 expression at timepoints ranging from 3 to 24 months following RP-L102, with stable CD18 expression levels for each patient subsequent to month 3.

In May 2022, we presented updated data at ASGCT's 25th Annual Meeting. The presentation included efficacy and safety interim data at three to 24 months of follow-up after infusion for all nine treated patients and overall survival data, including survival data for the seven patients with at least 12 months of follow-up after infusion as of the March 9, 2022 cut-off date. All patients, aged three months to nine years, demonstrated sustained CD18 restoration and expression on more than 10% of neutrophils (range: 20%-87%, median: 56%). At one year, the overall survival without allogeneic hematopoietic stem cell transplantation across the cohort is 100% based on the Kaplan-Meier estimate. As of the data cut-off, all nine patients are alive and clinically stable. All patients demonstrated a statistically significant reduction in the rate of all-cause hospitalizations and severe infections, relative to pre-treatment. Evidence of resolution of LAD-I-related skin rash and restoration of wound repair capabilities has been shown along with sustained phenotypic correction. The tolerability profile of RP-L201 has been highly favorable in all patients with no RP-L201-related adverse events. Adverse events related to other study procedures, including busulfan conditioning, have been previously disclosed and consistent with the tolerability profiles of those agents and procedures.

Anticipated Milestones

We have initiated discussions with health authorities on BLA filing plans for RP-L201 for the treatment of severe LAD-I and anticipate making such filing in the first half of 2023.

Pyruvate Kinase Deficiency (PKD):

Red blood cell PKD is a rare autosomal recessive disorder resulting from mutations in the pyruvate kinase L/R ("PKLR") gene encoding for a component of the red blood cell ("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. Market research indicates the application of gene therapy to broader populations could increase the market opportunity from approximately 250 to 500 patients per year.

We currently have one *ex*-vivo LVV-based program targeting PKD, RP-L301. RP-L301 is a clinical stage program that we in-licensed from CIEMAT. The IND for RP-L301 to initiate the global Phase 1 study cleared in October 2019. This program has been granted US and EMA orphan drug disease designation.

This global Phase 1 open-label, single-arm, clinical trial is expected to enroll four to five adult and pediatric PKD patients in the U.S. and Europe. The trial will be comprised of two cohorts to assess RP-L301 in pediatric (age 8-17) and adult populations. The trial is designed to assess the safety, tolerability, and preliminary activity of RP-L301, and initial safety evaluation will occur in the adult cohort before evaluation in pediatric patients. Stanford will serve as the lead site in the U.S. for adult and pediatric patients, HNJ will serve as the lead site in Europe for pediatrics, and Hospital Universitario Fundación Jiménez Díaz will serve as the lead site in Europe for adult patients. In July 2020, we treated the first patient in our clinical trial of RP-L301.

In December 2021, we presented positive clinical data at the 63rd Annual Meeting of ASH. The ASH poster presentation included preliminary data from two adult patients with severe anemia and substantial transfusion requirements who were treated as of the November 3, 2021 cut-off date. Each of these patients had experience extensive PKD-related disease complications including hepatic iron overload. Both patients have had marked improvement in hemoglobin levels, from baselines of 7.4 and 7.0 g/dL to 12-month values of 13.3 and 14.8 g/dL respectively; this represents an improvement from severe (Hb <8g/dL) to normal levels. Both patients have been transfusion independent subsequent to post-treatment hematopoietic reconstitution. Anemia resolution has been accompanied by marked improvement in additional markers of hemolysis, including bilirubin, erythropoietin, and reticulocyte counts. RP-L301 has been well tolerated in these adult patients, with no drug product related serious adverse events or infusion-related complications observed through 12-months post-treatment. Both patients have reported improved QOL following treatment with increases on FACT-An and additional designated QOL evaluations sustained through 12 months following therapy.

In May 2022, we presented updated data at the 25th Annual Meeting of the ASGCT. The presentation included data from two adult patients with severe or transfusion-dependent anemia as of the April 13, 2022 cut-off date. At 18 months post-infusion, both patients had sustained transgene expression, normalized hemoglobin, improved hemolysis, no red blood cell transfusion requirements post-engraftment and improved QOL both reported anecdotally and as documented via formal QOL assessments. The tolerability profile of RP-L301 appears favorable, with no RP-L-301-related serious adverse events through 18 months post-infusion. Transient transaminase elevation was seen in both patients post-therapy/conditioning, with no clinical stigmata of liver injury and subsequent resolution without clinical sequelae. The pediatric cohort is currently enrolling.

Anticipated Milestones

Enrollment in the PKD pediatric cohort is ongoing, and additional Phase 1 data are expected in the first half of 2023. Initiation of the phase 2 pivotal trial is expected to occur in 2023.

cGMP Manufacturing

Our state-of-the-art, 103,720 square foot manufacturing facility in Cranbury, New Jersey. has been scaled up to manufacture AAV drug product for a planned Phase 2 pivotal study in Danon disease. The facility also houses lab space for research & development and quality. We reached an understanding with the FDA on chemistry, manufacturing, and controls requirements to start AAV cGMP manufacturing at our in-house facility as well as potency assay plans for a Phase 2 pivotal trial in Danon disease. To further strengthen our manufacturing and commercial capabilities we appointed Mayo Pujols, one of the most seasoned cell and gene therapy technical operations and manufacturing leaders in the industry, as our Chief Technical Officer.

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, develop proprietary in-house analytics and manufacturing capabilities and continue to commence registration trials for our currently planned programs. We expect to submit our first BLA for the LAD program in the first half of 2023. In the medium and long-term, pending favorable data, we expect to submit BLAs for the rest of our suite of clinical programs, 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 product approval.

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, R&D activities for our product candidates 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 September 30, 2022, we raised net cash proceeds of approximately $727.2 million from investors through both equity and convertible debt financing to fund operating activities.

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 R&D program expenses consist primarily of external costs incurred for the development of our product candidates. These expenses include:

- expenses incurred under agreements with research institutions and consultants that conduct R&D activities including process development, preclinical, and clinical activities on our 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 R&D activities are recorded as prepaid expenses.

Our direct R&D 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 R&D 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 R&D 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 R&D 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 R&D activities.

Our direct R&D expenses consist principally of external costs, such as fees paid to investigators, consultants, laboratories and CROs in connection with our clinical studies, and costs related to acquiring and manufacturing clinical study materials. We allocate salary and benefit costs directly related to specific programs. We do not allocate personnel-related discretionary bonus or stock-based compensation costs, costs associated with our general discovery platform improvements, depreciation or other indirect costs that are deployed across multiple projects under development and, as such, the costs are separately classified as other R&D expenses.

The following table presents R&D expenses tracked on a program-by-program basis as well as by type and nature of expense for the three and nine months ended September 30, 2022 and 2021.

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Direct Expenses:				
Danon Disease (AAV) RP-A501	$ 8,058	$ 3,497	$ 24,001	$ 11,804
Leukocyte Adhesion Deficiency (LVV) RP-L201	5,719	7,896	15,814	18,167
Fanconi Anemia (LVV) RP-L102	7,141	6,379	16,228	11,986
Pyruvate Kinase Deficiency (LVV) RP-L301	433	1,109	1,919	3,530
Infantile Malignant Osteopetrosis (LVV) RP-L401 (1)	91	729	280	1,975
Other product candidates	4,689	2,128	11,719	3,998
Total direct expenses	26,131	21,738	69,961	51,460
Unallocated Expenses				
Employee compensation	$ 7,983	$ 5,524	$ 20,495	$ 15,265
Stock based compensation expense	3,040	3,084	8,247	9,148
Depreciation and amortization expense	1,090	1,258	2,976	3,627
Laboratory and related expenses	3,033	651	3,940	2,745
Professional Fees	819	400	2,009	1,216
Other expenses	1,287	6,966	7,905	8,998
Total other research and development expenses	17,252	17,883	45,572	40,999
Total research and development expense	$ 43,383	$ 39,621	$ 115,533	$ 92,459

(1) Effective December 2021, a decision was made to no longer pursue Rocket-sponsored clinical evaluation of RP-L401; this program was returned to academic innovators.

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 R&D 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 R&D expenses to increase for the foreseeable future as we continue to invest in R&D 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 R&D projects or when and to what extent we will generate revenue from the commercialization and sale of any of our product candidates.

Our future R&D 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 R&D expenses to increase in future periods for the foreseeable future as we seek to further 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 R&D 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 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, 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 as we continue to operate as a public company with increasing complexity, we will continue to 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 for the three and nine months ended September 30, 2022, was related to our financing lease obligation for the Cranbury, NJ facility. Interest expense for the three and nine months ended September 30, 2021, was related to the 2021 Convertible Notes which converted into common stock on August 2, 2021, the 2022 Convertible Notes, which were redeemed and converted into common stock in April 2021, and the financing lease obligation for the Cranbury, NJ facility.

Interest Income

Interest income was related to interest earned from investments and cash equivalents.

Critical Accounting Policies and Significant Judgments and Estimates

There have been no material changes in our critical accounting policies and estimates in the preparation of our condensed consolidated financial statements during the three months ended September 30, 2022 compared to those disclosed in our 2021 Form 10-K.

Results of Operations

Comparison of the Three Months Ended September 30, 2022 and 2021

	Three Months Ended September 30,		
	2022	2021	Change
Operating expenses:			
Research and development	$ 43,383	$ 39,621	$ 3,762
General and administrative	15,105	10,025	5,080
Total operating expenses	58,488	49,646	8,842
Loss from operations	(58,488)	(49,646)	(8,842)
Research and development incentives	-	-	-
Interest expense	(465)	(534)	69
Interest and other income, net	1,353	806	547
Amortization of premium on investments - net	(156)	(744)	588
Total other income (expense), net	732	(472)	1,204
Net loss	$ (57,756)	$ (50,118)	$ (7,638)

Research and Development Expenses

R&D expenses increased $3.8 million to $43.4 million for the three months ended September 30, 2022 compared to the three months ended September 30, 2021. The increase in R&D expenses was primarily driven by increases in manufacturing and development costs of $4.0 million, compensation and benefits of $2.5 million due to increased R&D headcount, direct materials of $2.2 million, laboratory supplies of $1.8 million and professional fees of $0.9 million. Increases noted were offset by a decrease in licensing fees of $7.6 million attributable to the Neptune Warrant expense recorded in the three months ended September 30, 2021.

General and Administrative Expenses

G&A expenses increased $5.1 million to $15.1 million for the three months ended September 30, 2022, compared to the three months ended September 30, 2021. The increase in G&A expenses was primarily driven by increases in compensation and benefits of $1.3 million due to increased G&A headcount, non-cash stock compensation expense of $0.7 million, office and administrative expenses of $0.4 million and Renovacor pre-acquisition related expenses of $1.3 million.

Other Income (Expense), Net

Other income increased by $1.2 million to $0.7 million for the three months ended September 30, 2022, compared to the three months ended September 30, 2021. The increase in other income was primarily driven by an increase in interest and other income, net, of $0.5 million due to increased interest rates and an increase in investment amortization, net, of $0.6 million.

Comparison of the Nine Months Ended September 30, 2022 and 2021

	Nine Months Ended September 30,		
	2022	2021	Change
Operating expenses:			
Research and development	$ 115,533	$ 92,459	$ 23,074
General and administrative	39,728	30,456	9,272
Total operating expenses	155,261	122,915	32,346
Loss from operations	(155,261)	(122,915)	(32,346)
Research and development incentives	-	500	(500)
Interest expense	(1,395)	(2,514)	1,119
Interest and other income, net	2,644	2,218	426
Amortization of premium on investments - net	(1,128)	(2,111)	983
Total other income (expense), net	121	(1,907)	2,028
Net loss	$ (155,140)	$ (124,822)	$ (30,318)

Research and Development Expenses

R&D expenses increased $23.1 million to $115.5 million for the nine months ended September 30, 2022 compared to the nine months ended September 30, 2021. The increase in R&D expenses was primarily driven by increases in manufacturing and development costs of $15.8 million, laboratory supplies of $4.5 million, compensation and benefits expense of $5.2 million due to increased R&D headcount and direct materials of $3.0 million. Increases noted were offset by a decrease in non-cash stock compensation expense of $0.9 million and a decrease in license fees of $7.7 million attributable to the Neptune Warrant expense recorded in the nine months ended September 30, 2021.

General and Administrative Expenses

G&A expenses increased $9.3 million to $39.7 million for the nine months ended September 30, 2022, compared to the nine months ended September 30, 2021. The increase in G&A expenses was primarily driven by increases in commercial preparation expenses which consists of commercial strategy, medical affairs, market development and pricing analysis of $2.5 million, compensation and benefits of $2.8 million due to increased G&A headcount and legal expense of $1.3 million.

Other Income (Expense), Net

Other income increased by $2.0 million to $0.1 million for the nine months ended September 30, 2022, compared to the nine months ended September 30, 2021. The increase in other income was primarily driven by decreased interest expense of $1.1 million associated with the 2022 Convertible Notes that were redeemed in April 2021 and the 2021 Convertible Notes that were converted in August 2021, an increase in interest and other income, net of $0.4 million due to increased interest rates and an increase in investment amortization, net, of $1.0 million.

Liquidity, Capital Resources and Plan of Operations

We have not generated any revenue and have incurred losses since inception. Operations of the Company are subject to certain risks and uncertainties, including, among others, uncertainty of drug candidate development, technological uncertainty, uncertainty regarding patents and proprietary rights, having no commercial manufacturing experience, 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 R&D 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.

Our drug candidates are in the development and clinical stage. There can be no assurance that our R&D will be successfully completed, that adequate protection for our 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 our product development efforts are successful, it is uncertain when, if ever, we will generate significant revenue from product sales. We operate in an environment of rapid change in technology and substantial competition from pharmaceutical and biotechnology companies.

Our 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. We have incurred net losses and negative cash flows from our operations each year since inception. We had net losses of $155.1 million for the nine months ended September 30, 2022, and $169.1 million for the year ended December 31, 2021. As of September 30, 2022 and December 31, 2021, we had an accumulated deficit of $647.1 million and $491.9 million, respectively. As of September 30, 2022, we had $306.5 million of cash, cash equivalents and investments. We expect such resources would be sufficient to fund our operating expenses and capital expenditure requirements into the second half of 2024. We have funded our operations primarily through the sale of our equity and debt securities.

In the longer term, our future viability is dependent on our ability to generate cash from operating activities or to raise additional capital to finance our operations. If we raise additional funds by issuing equity securities, our stockholders will experience dilution. Any future debt financing into which we enter may impose upon us additional covenants that restrict our operations, including limitations on our ability to incur liens or additional debt, pay dividends, repurchase our common stock, make certain investments and engage in certain merger, consolidation, or asset sale transactions. Any debt financing or additional equity that we raise may contain terms that are not favorable to us or our stockholders. Our failure to raise capital as and when needed could have a negative impact on our financial condition and ability to pursue our business strategies.

Cash Flows

	Nine Months Ended September 30,	
	2022	2021
Net cash used in operating activities	$ (122,121)	$ (90,222)
Net cash provided by investing activities	39,259	1,989
Net cash provided by financing activities	46,848	36,545
Net decrease in cash, cash equivalents and restricted cash	$ (36,014)	$ (51,688)

Operating Activities

During the nine months ended September 30, 2022, operating activities used $122.1 million of cash and cash equivalents, primarily resulting from our net loss of $155.1 million offset by net non-cash charges of $27.1 million, including non-cash stock-based compensation expense of $21.3 million, accretion of discount on investments of $1.1 million, and depreciation and amortization expense of $2.9 million. Changes in our operating assets and liabilities for the nine months ended September 30, 2022 consisted of an increase in accounts payable and accrued expenses of $7.2 million and a decrease in our prepaid expenses of $1.3 million.

During the nine months ended September 30, 2021, operating activities used $90.2 million of cash, primarily resulting from our net loss of $124.8 million offset by net non-cash charges of $34.8 million, including non-cash stock-based compensation expense of $22.2 million, warrant issuance of $7.6 million, accretion of discount on investments of $2.1 million, and depreciation and amortization expense of $2.2 million. Changes in our operating assets and liabilities for the nine months ended September 30, 2021 consisted of an increase in accounts payable and accrued expenses for $2.8 million, a decrease in finance lease liability of $1.6 million and a decrease in our prepaid expenses of $1.0 million.

Investing Activities

During the nine months ended September 30, 2022, net cash provided by investing activities was $39.3 million, primarily resulting from proceeds of $222.1 million from the maturities of investments, offset by purchases of investments of $177.5 million, and purchases of property and equipment of $5.4 million.

During the nine months ended September 30, 2021, net cash provided by investing activities was $2.0 million, consisting of proceeds of $234.1 million from the maturities of investments, offset by purchases of investments of $226.5 million, purchases of property and equipment of $4.9 million, and purchases of internal use software of $0.7 million.

Financing Activities

During the nine months ended September 30, 2022, financing activities provided $46.8 million of cash, primarily resulting from net proceeds of $46.6 million from the sale of shares through our at-the-market facility.

During the nine months ended September 30, 2021, net cash provided by financing activities was $36.5 million, consisting of the issuance of common stock related to the August 2021 private placement of $26.4 million and issuance of common stock pursuant to exercises of stock options.

Contractual Obligations and Commitments

There were no material changes outside the ordinary course of our business to the contractual obligations specified in the table of contractual obligations included in “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in our 2021 Form 10-K. Information regarding contractual obligations and commitments may be found in Note 10 of our unaudited consolidated financial statements in this Quarterly Report on Form 10-Q. We do not have any off-balance sheet arrangements that are material or reasonably likely to become material to our financial condition or results of operations.

Recently Issued Accounting Pronouncements

There were no recent accounting pronouncements that impacted the Company, or which had a significant effect on the consolidated financial statements.

Item 3 Quantitative and Qualitative Disclosures About Market Risk

None

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 September 30, 2022, 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 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 three months ended September 30, 2022, 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, the Company may be subject to 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.

Item 1A. Risk Factors

Our material risk factors are disclosed in Item 1A of our 2021 Form 10-K. Except as set forth below, there have been no material changes from the risk factors previously disclosed in such filing.

Recent volatility in capital markets and lower market prices for our securities may affect our ability to access new capital through sales of shares of our common stock or issuance of indebtedness, which may harm our liquidity, limit our ability to grow our business, pursue acquisitions or improve our operating infrastructure and restrict our ability to compete in our markets.

Our operations consume substantial amounts of cash, and we intend to continue to make significant investments to support our business growth, respond to business challenges or opportunities, develop new solutions, retain or expand our current levels of personnel, improve our existing solutions, enhance our operating infrastructure, and potentially acquire complementary businesses and technologies. Our future capital requirements may be significantly different from our current estimates and will depend on many factors, including the need to:

- finance unanticipated working capital requirements;
- continue to advance our product candidates through clinical development;
- continue our efforts to expand our pipeline of development programs;
- develop proprietary in-house analytics and manufacturing capabilities;
- pursue acquisitions or other strategic relationships; and
- respond to competitive pressures.

Accordingly, we may need to pursue equity or debt financings to meet our capital needs. With uncertainty in the capital markets and other factors, such financing may not be available on terms favorable to us or at all. If we raise additional funds through further issuances of equity or convertible debt securities, our existing stockholders could suffer significant dilution, and any new equity securities we issue could have rights, preferences, and privileges superior to those of holders of our common stock. Any debt financing secured by us in the future could involve additional restrictive covenants relating to our capital-raising activities and other financial and operational matters, which may make it more difficult for us to obtain additional capital and to pursue business opportunities, including potential acquisitions. If we are unable to obtain adequate financing or financing on terms satisfactory to us, we could face significant limitations on our ability to invest in our operations and otherwise suffer harm to our business.

We may fail to realize the anticipated benefits of the acquisition of Renovacor.

The success of the acquisition of Renovacor will depend on, among other things, our ability to close the transaction and to combine our business with Renovacor in a manner that allows us to achieve developmental and operational synergies. The closing of the acquisition is subject to certain closing conditions including, among other things, approval by our and Renovacor's stockholders, receipt of required regulatory approvals and the satisfaction of other customary closing conditions. We cannot guarantee that these conditions will be satisfied in a manner that will allow us to close the acquisition within the expected timeframe or at all. Lawsuits or other legal proceedings brought in connection with the acquisition could also delay or prevent the closing of the transaction and may cause us to incur additional costs and divert management's attention from the acquisition process and our core business operations. If we are unable to complete the acquisition within the expected timeframe or at all, our business, financial condition and results of operations would be negatively impacted. Moreover, even if the acquisition closes within the expected timeframe, it is possible that the integration process could result in the loss of key employees; the disruption of our ongoing business or the ongoing business of Renovacor; or inconsistencies in standards, controls, procedures, or policies, in each case, that could adversely affect our ability to achieve the anticipated benefits of the acquisition. Integration efforts between the two companies will also divert management's attention from our core business and other opportunities that could have been beneficial to our shareholders. An inability to realize the full extent of, or any of, the anticipated benefits of the acquisition, as well as any delays encountered in the integration process, could have an adverse effect on our business and results of operations, which may affect the value of the shares of our common stock after the completion of the acquisition. If we are unable to achieve these objectives, the anticipated benefits of the acquisition may not be realized fully or at all or may take longer or cost more to realize than expected. In particular, the acquisition may not be accretive to our stock value in the near or long term. In addition, the acquisition of Renovacor may impact the market price for shares of our common stock, which could result in substantial losses for our stockholders.

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)
2.2	Agreement and Plan of Merger, dated September 19, 2022, by and among Parent, the Company, Merger Sub I and Merger Sub II (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 20, 2022).
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, 2019
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	Form of Parent Voting Agreement (incorporated by reference to Exhibit 99.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on September 20, 2022).
10.2	Form of Company Voting Agreement (incorporated by reference to Exhibit 99.2 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on September 20, 2022).
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	Inline XBRL Instance Document.
101.SCH	Inline XBRL Taxonomy Extension Schema Document.
101.CAL	Inline XBRL Taxonomy Extension Calculation Document.
101.DEF	Inline XBRL Taxonomy Extension Definition Linkbase Document.
101.LAB	Inline XBRL Taxonomy Extension Labels Linkbase Document.
101.PRE	Inline XBRL Taxonomy Extension Presentation Link Document.
104	Cover Page Interactive Data File (the cover page XBRL tags are embedded within the Inline XBRL document)

* Filed herewith.

** The certification furnished in Exhibit 32.1 hereto is deemed to be furnished with this Quarterly Report on Form 10-Q and will not be deemed "filed" for purposes of Section 18 of the Securities Exchange Act of 1934, as amended, except to the extent that the Registrant specifically incorporates it by reference

SIGNATURES

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

ROCKET PHARMACEUTICALS, INC.

November 4, 2022 By: /s/ Gaurav Shah, MD

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

November 4, 2022 By: /s/ John Militello

John Militello
VP of Finance, Senior Controller and Treasurer
(Interim Principal Financial Officer and Principal Accounting Officer)

Exhibit 31.1

CERTIFICATIONS

I, Gaurav Shah, MD, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended September 30, 2022 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: November 4, 2022

/s/ Gaurav Shah, MD

Gaurav Shah, MD
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 September 30, 2022 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: November 4, 2022

/s/ John Militello

John Militello
VP of Finance, Senior Controller and Treasurer
(Interim Principal Financial Officer and Principal 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 September 30, 2022, 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: November 4, 2022

/s/ Gaurav Shah, MD

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

Date: November 4, 2022

/s/ John Militello

John Militello
VP of Finance, Senior Controller and Treasurer
(Interim Principal Financial Officer and Principal Accounting Officer)