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

Form 10-Q

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

☒

For the quarterly period ended March 31, 2024 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 May 1, 2024, there were

90,782,211
shares of common stock, $0.01 par value per share, outstanding.

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Summary of Abbreviated Terms

Rocket Pharmaceuticals, Inc. may be referred to as Rocket, the Company, we, our or us, in this Quarterly Report, unless the context otherwise indicates. Throughout this Quarterly Report, we have used terms which are defined below:

2023 Form 10-K	Annual Report on Form 10-K for the fiscal year ended December 31, 2023
AAV	Adeno-associated virus
ACM	Arrhythmogenic cardiomyopathy
ASC	Accounting Standard Codification
ASGCT	American Society of Gene & Cell Therapy
BLA	Biologics License Application
BNP	Brain natriuretic peptide
cGMP	Current Good Manufacturing Practice
CIEMAT	Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas
CIRM	California Institute for Regenerative Medicine
DCM	Dilated Cardiomyopathy
DD	Danon Disease
DNA	Deoxyrubonucleic acid
EMA	European Medicines Agency
EU	European Union
Europe	EU
FA	Fanconi Anemia
FASB	Financial Accounting Standards Board
FDA	U.S. Food and Drug Administration
GOSH	Great Ormond Street Hospital
HNJ	Hospital Infantil de Nino Jesus
ICD	Implantable cardiac defibrillator
IND	Investigational New Drug application
IPR&D	In process research and development
KCCQ	Kansas City Cardiovascular Questionnaire
LAD-I	Leukocyte Adhesion Deficiency-I
LV	Lentiviral vector
MHRA	Medicines and Healthcare Products Regulatory Agency
NYHA	New York Heart Association
PKD	Pyruvate Kinase Deficiency
PKP2-ACM	Plakophilin-2 Arrhythmogenic Cardiomyopathy
PSU	Performance stock unit
R&D	Research and development
Renovacor	Renovacor, Inc. acquired by Rocket on December 1, 2022
RSU	Restricted stock unit
SEC	Securities and Exchange Commission
Stanford	Center for Definitive and Curative Medicine at Stanford University School of Medicine
U.S.	United States
U.S. GAAP	U.S. Generally Accepted Accounting Principles
UCLA	University of California, Los Angeles

Cautionary Statement Regarding Forward-Looking Statements

This Quarterly Report on Form 10-Q for the quarter ended March 31, 2024 contains forward-looking statements that involve risks and uncertainties, as well as assumptions that, if they do not 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 "aim," "anticipate," "believe," "can," "contemplate," "continue," "could," "design," "develop," "estimate," "expect," "expand," "future," "hope," "intend," "likely," "may," "plan," "potential," "predict," "project," "pursue," "seek," "should," "strategy," "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:

- our ability to meet our anticipated milestones for our various drug candidates with respect to the initiation and timing of clinical studies;
- federal, state, and non-U.S. regulatory requirements, including regulation of our current or any other future product candidates by the 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 develop our sales and marketing capabilities or enter into agreements with third parties to sell and market any of our 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;
- the development of our direct manufacturing capabilities for our AAV programs;
- our ability to expand our pipeline to target additional indications that are compatible with our gene therapy technologies;
- 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;
- our ability to obtain and enforce patents to protect our product candidates, and our ability to successfully defend ourselves against unforeseen third-party infringement claims;
- anticipated trends and challenges in our business and the markets in which we operate;
- our estimates regarding our capital requirements; and
- our ability to obtain additional financing and raise capital as necessary to fund operations or pursue business opportunities.

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, 2023, 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.

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PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

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

	March 31, 2024 (unaudited)	December 31, 2023
Assets		
Current assets:		
Cash and cash equivalents	$ 35,276	$ 55,904
Investments	295,037	317,271
Prepaid expenses and other current assets	14,846	5,047
Total current assets	345,159	378,222
Property and equipment, net	39,337	39,172
Goodwill	39,154	39,154
Intangible assets	25,150	25,150
Restricted cash	1,362	1,372
Deposits	533	533
Investments	-	34,320
Operating lease right-of-use assets, net	4,768	3,901
Finance lease right-of-use asset, net	43,979	44,517
Total assets	$ 499,442	$ 566,341
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 30,085	$ 45,789
Operating lease liabilities, current	1,064	925
Finance lease liability, current	1,808	1,791

Total current liabilities	32,957	48,505
Operating lease liabilities, non-current	3,714	2,973
Finance lease liability, non-current	19,363	19,353
Other liabilities	1,906	2,936
Total liabilities	57,940	73,767
Commitments and contingencies (Note 13)		
Stockholders' equity:		
Preferred stock, $ 0.01 par value, authorized 5,000,000 shares:		
Series A convertible preferred stock; 300,000 shares designated; 0 shares issued and outstanding	-	-
Series B convertible preferred stock; 300,000 shares designated; 0 shares issued and outstanding	-	-

Common stock, $ 0.01 par value, 120,000,000 shares authorized; 90,646,590 and 90,282,267 shares issued and outstanding at March 31, 2024 and December 31, 2023, respectively	906	903
Additional paid-in capital	1,462,155	1,450,722
Accumulated other comprehensive income (loss)	(135)	319
Accumulated deficit	(1,021,424)	(959,370)
Total stockholders' equity	441,502	492,574
Total liabilities and stockholders' equity	$ 499,442	$ 566,341

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 March 31,	
	2024	2023
Revenue	$ -	$ -
Operating expenses:		
Research and development	45,227	46,371
General and administrative	22,148	15,823
Total operating expenses	67,375	62,194
Loss from operations	(67,375)	(62,194)
Interest expense	(471)	(468)
Interest and other income, net	3,029	1,908
Accretion of discount on investments, net	2,763	2,419
Net loss	$ (62,054)	$ (58,335)
Net loss per share - basic and diluted	$ (0.66)	$ (0.73)
Weighted-average common shares outstanding - basic and diluted	93,549,884	79,453,519

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

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

	Three Months Ended March 31,	
	2024	**2023**
Net loss	$ (62,054)	$ (58,335)
Other comprehensive loss:		
Net unrealized (loss) gain on investments	(454)	272
Total comprehensive loss	$ (62,508)	$ (58,063)

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

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Rocket Pharmaceuticals, Inc.
Consolidated Statements of Stockholders' Equity
For the Three Months Ended March 31, 2024 and 2023
($ in thousands except share amounts)
(unaudited)

	Common Stock Shares	Common Stock Amount	Treasury Stock	Additional Paid-In Capital	Accumulated Other Comprehensive Income/(Loss)	Accumulated Deficit	Total Stockholders' Equity
Balance at December 31, 2023	90,282,267	$ 903	$ -	$ 1,450,722	$ 319	$ (959,370)	$ 492,574
Issuance of common stock pursuant to exercise of stock options	73,745	-	-	1,184	-	-	1,184
Issuance of common stock pursuant to vesting of restricted stock units	290,578	3	-	(3)	-	-	-
Unrealized comprehensive loss on investments	-	-	-	-	(454)	-	(454)
Stock-based compensation	-	-	-	10,252	-	-	10,252
Net loss	-	-	-	-	-	(62,054)	(62,054)
Balance at March 31, 2024	90,646,590	$ 906	$ -	$ 1,462,155	$ (135)	$ (1,021,424)	$ 441,502

	Common Stock Shares	Common Stock Amount	Treasury Stock	Additional Paid-In Capital	Accumulated Other Comprehensive Income/(Loss)	Accumulated Deficit	Total Stockholders' Equity
Balance at December 31, 2022	79,123,312	$ 791	$ (47)	$ 1,203,074	$ (357)	$ (713,775)	$ 489,686
Issuance of common stock pursuant to exercise of stock options	88,429	1	-	1,113	-	-	1,114
Issuance of common stock pursuant to vesting of restricted stock units	126,060	1	-	(1)	-	-	-
Issuance of common stock pursuant to exercise of warrants	126,093	1	-	6	-	-	7
Issuance of common stock pursuant to the at-the-market offering program, net of issuance costs	948,300	10	-	17,212	-	-	17,222

Unrealized comprehensive gain on investments	-	-	-	-	267	-	267
Stock-based compensation	-	-	-	8,915	-	-	8,915
Net loss	-	-	-	-	-	58,335	(58,335)
Balance at March 31, 2023	80,412,194	$ 804	$ (47)	$ 1,230,319	$ (90)	$ (772,110)	$ 458,876

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

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

	Three Months Ended March 31,	
	2024	2023
Operating activities:		
Net loss	$ (62,054)	$ (58,335)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization of property and equipment	1,716	1,135
Amortization of finance lease right of use asset	538	538
Stock-based compensation	10,252	8,915
Accretion of discount on investments, net	(2,754)	(2,343)
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(1,151)	866
Accounts payable and accrued expenses	(2,413)	(7,750)
Operating lease liabilities and right of use assets, net	13	97
Finance lease liability	27	37
Other liabilities	(1,030)	(720)
Net cash used in operating activities	(56,856)	(57,560)
Investing activities:		
Purchases of investments	(63,947)	(96,034)
Proceeds from maturities of investments	101,016	62,335
Payments made to acquire right of use asset	-	(7)
Purchases of property and equipment	(2,035)	(3,015)
Net cash provided by (used in) investing activities	35,034	(36,721)
Financing activities:		
Issuance of common stock, pursuant to exercise of stock options	1,184	1,114

Issuance of common stock, pursuant to the at-the-market offering program, net of issuance costs	-	17,222
Issuance of common stock, pursuant to exercise of warrants	-	7
Net cash provided by financing activities	1,184	18,343
Net change in cash, cash equivalents and restricted cash	(20,638)	(75,938)
Cash, cash equivalents and restricted cash at beginning of period	57,276	141,857
Cash, cash equivalents and restricted cash at end of period	$ 36,638	$ 65,919
Supplemental disclosure of non-cash financing and investing activities:		
Accrued purchases of property and equipment, ending balance	$ 923	$ 1,794
Investment maturity receivables and purchase payables, ending balance	8,648	-
Operating lease liabilities	1,134	-
Unrealized (loss) gain on investments	$ (454)	$ 267

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. is a fully integrated, late-stage 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. The Company has

three
clinical-stage *ex vivo* lentiviral vector programs, which include programs for:

- Fanconi Anemia, 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, a genetic disorder that causes the immune system to malfunction; and
- Pyruvate Kinase Deficiency, a red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia.

In September 2023, the FDA accepted the Biologics License Application and granted priority review for RP-L201 for the treatment of severe LAD-I. Treatments in the FA Phase 2 studies were completed in 2023 with regulatory filings in the U.S. and EU for FA anticipated in 2024. Additional work on a gene therapy program for the less common FA subtypes C and G is ongoing.

In the U.S., the Company also has

two
clinical stage and

one
pre-clinical stage *in vivo* adeno-associated virus programs, which include programs for:

- Danon Disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. The DD program is currently in an ongoing Phase 2 trial.
- Plakophilin-2 Arrhythmogenic Cardiomyopathy, an inheritable cardiac disorder that is characterized by a progressive loss of cardiac muscle mass, severe right ventricular dilation, dysplasia, fibrofatty replacement of the myocardium and a high propensity to arrhythmias and sudden death. This program received FDA clearance of an Investigational New Drug application and the Company has initiated a Phase 1 study.
- BAG3 Dilated Cardiomyopathy, which is the most common form of cardiomyopathy and is characterized by progressive thinning of the walls of the heart resulting in enlarged heart chambers that are unable to pump blood. The Company utilizes recombinant AAV9-based gene therapy designed to slow or halt progression of BAG3-DCM.

The Company has global commercialization and development rights to all of these product candidates under royalty-bearing license agreements.

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 $

1.02
billion as of March 31, 2024. As of March 31, 2024, the Company had $

330.3
million of cash, cash equivalents and investments. Excluded from the $

330.3
million of cash, cash equivalent and investments are receivables from maturity of securities that have yet to be received of $

8.6
million recorded as part of prepaid expenses and other current assets. The net balance of cash, cash equivalents and investments when adjusting for this receivable would have been $

338.9
million. The Company expects such resources will be sufficient to fund the Company's operating expenses and capital expenditure requirements into 2026.

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
million (the "Shares") through Cowen as its sales agent. On September 12, 2023, the Company and Cowen entered into an amendment (the "Amended Sales Agreement") pursuant to which the aggregate offering amount available under the at-the-market offering program was reduced to $

180.0
million. Through March 31, 2024, the Company has sold

4.2
million shares of common stock for net proceeds of $

63.8
million pursuant to the at-the-market offering program (see Note 8 "Stockholders' Equity"). The Company did not sell any shares under the at-the-market offering program during the three months ended March 31, 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, 2023 included in the Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 27, 2024. 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 March 31, 2024 and the results of its operations and its cash flows for the three months ended March 31, 2024. The financial data and other information disclosed in these consolidated notes related to the three months ended March 31, 2024 and 2023 are unaudited. The results for the three months ended March 31, 2024 are not necessarily indicative of results to be expected for the year ending December 31, 2024 and any other interim periods or any future year or period.

Significant Accounting Policies

The significant accounting policies used in the preparation of these consolidated financial statements for the three months ended March 31, 2024 are consistent with those disclosed in Note 3 to the consolidated financial statements in the 2023 Form 10-K with most significant policies also being listed here.

Principles of Consolidation

The consolidated financial statements represent the consolidation of the accounts of the Company and its subsidiaries in conformity with 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 and intangible asset impairments, the accrual of R&D expenses, and 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, 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 12 "Leases" for additional disclosures) and a deposit collateralizing a letter of credit issued by a bank supporting the Company's corporate credit cards. Cash, cash equivalents and restricted cash consist of the following:

	March 31, 2024	December 31, 2023
Cash and cash equivalents	$ 35,276	$ 55,904
Restricted cash	1,362	1,372
Total cash, cash equivalents and restricted cash	$ 36,638	$ 57,276

Concentrations of credit risk and off-balance sheet risk

Financial instruments that subject the Company to credit risk primarily consist of cash and cash equivalents and available-for-sale securities. The Company maintains its cash and cash equivalent balances with high quality financial institutions and, consequently, the Company believes that such funds are subject to minimal credit risk. The Company's marketable securities consist of U.S. Treasury Securities and Corporate Bonds. The Company's investment policy limits the amounts the Company may invest in any one type of investment and requires all investments held by the Company to be at least AA-/Aa3 rated, thereby reducing credit risk exposure.

Investments

Investments consist of U.S. Treasury Securities and Corporate Bonds. Management determines the appropriate classification of these securities at the time they are acquired and evaluates the appropriateness of such classifications at each balance sheet date. The Company classifies its investments as available-for-sale pursuant to FASB ASC 320, Investments-Debt and Equity Securities. Investments are recorded at fair value, with unrealized gains and losses included as a component of accumulated other comprehensive income (loss) in stockholders' equity and a component of total comprehensive loss in the consolidated statements of comprehensive loss, until realized. Realized gains and losses are included in investment income on a specific-identification basis. The Company estimates expected credit losses for investments when unrealized losses exist. Unrealized losses that are credit related are recognized in the Company's Consolidated Statements of Operations and unrealized losses that are not credit related are recognized in accumulated other comprehensive income (loss). For the three months ended March 31, 2024 and 2023, there were

no

unrealized losses that were credit related. For the three months ended March 31, 2024, there was $

0.5

million of net unrealized loss on investments. For the three months ended March 31, 2023 there was $

0.3

million of net unrealized gains on investments.

Intangible Assets

Intangible assets related to in process research and development projects are considered to be indefinite-lived until the completion or abandonment of the associated R&D efforts. If and when development is complete, which generally occurs if and when regulatory approval to market a product is obtained, the associated assets would be deemed finite-lived and would then be amortized based on their respective estimated useful lives at that point in time. IPR&D intangible assets which are determined to have had a decrease in their fair value are adjusted downward and an expense is recognized in R&D expenses in the Consolidated Statements of Operations. These IPR&D intangible assets are tested at least annually or when a triggering event occurs that could indicate a potential impairment based on indicators including progress of R&D activities, changes in projected development of assets, and changes in regulatory environment and future commercial markets. If a triggering event occurs that would indicate a potential impairment, the Company will perform a quantitative analysis to determine whether it is more likely than not that the fair value is below carrying amount.

Fair Value Measurements

The Company is required to disclose information on all assets and liabilities reported at fair value that enables an assessment of the inputs used in determining the reported fair values. FASB ASC 820, Fair Value Measurements and Disclosures, establishes a hierarchy of inputs used when available. Observable inputs are inputs that market participants would use in pricing the asset or liability based on market data obtained from sources independent of the Company. Unobservable inputs are inputs that reflect the Company's assumptions about the inputs that market participants would use in pricing the asset or liability and are developed based on the best information available in the circumstances. The fair value hierarchy applies only to the valuation inputs used in determining the reported fair value of the investments and is not a measure of the investment credit quality. The three levels of the fair value hierarchy are described below:

- Level 1 - Valuations based on unadjusted quoted prices in active markets for identical assets or liabilities that the Company has the ability to access at the measurement date.
- Level 2 - Valuations based on quoted prices for similar assets or liabilities in markets that are not active or for which all significant inputs are observable, either directly or indirectly.
- Level 3 - Valuations that require inputs that reflect the Company's own assumptions that are both significant to the fair value measurement and unobservable.

To the extent that valuation is based on models or inputs that are less observable or unobservable in the market, the determination of fair value requires more judgment. Accordingly, the degree of judgment exercised by the Company in determining fair value is greatest for instruments categorized in Level 3. A financial instrument's level within the fair value hierarchy is based on the lowest level of any input that is significant to the fair value measurement. The fair value of the Company's financial instruments, including cash and cash equivalents, restricted cash, deposits, accounts payable and accrued expenses approximate their respective carrying values due to the short-term nature of most of these instruments.

Warrants

The Company accounts for stock warrants as either equity instruments, liabilities or derivative liabilities in accordance with FASB ASC 480, Distinguishing Liabilities from Equity and/or FASB ASC 815, Derivatives and Hedging, depending on the specific terms of the warrant agreement. Liability-classified warrants are recorded at their estimated fair values at each reporting period until they are exercised, terminated, reclassified or otherwise settled. Changes in the estimated fair value of liability-classified warrants are included in interest and other income in the Company's Consolidated Statement of Operations.

Stock-Based Compensation

The Company issues stock-based awards to employees and non-employees, generally in the form of stock options, RSUs and PSUs.

The Company measures the compensation expense of employee and non-employee services received in exchange for an award of equity instruments based on the fair value of the award on the grant date. That cost of stock options and RSUs is recognized over the requisite service period of the awards on a straight-line basis with forfeitures recognized as they occur. The vesting condition for PSUs is performance based and the cost of PSUs is recognized when it is likely that the performance goals associated with the PSUs will be achieved and the awards will vest.

The Company classifies stock-based compensation expense in its Consolidated Statements of Operations in the same manner in which the award recipient's payroll costs and services are classified or in which the award recipient's service payments are classified.

Recent Accounting Pronouncements

Accounting Pronouncements Not Adopted as of March 31, 2024

ASU 2023-09: Income Taxes Topic 740 - Improvements to Income Tax Disclosures. This update standardizes categories for the effective tax rate reconciliation, requires disaggregation of income taxes and additional income tax-related disclosures. This update is required to be effective for the Company for fiscal periods beginning after December 15, 2024. As this accounting standard only impacts disclosures, it will not have a material impact on the Company's Consolidated Financial Statements.

ASU 2023-07: Segment Reporting Topic 280 - Improvements to Reportable Segment Disclosures. This update requires expanded annual and interim disclosures for significant segment expenses that are regularly provided to the chief operating decision maker and included within each reported measure of segment profit or loss. This update will be effective for fiscal years beginning after December 15, 2023, and is to be applied retrospectively to all periods presented in the financial statements. Early adoption is permitted. As this accounting standard only impacts disclosures, it will not have a material impact on the Company's consolidated financial statements.

4. Fair Value of Financial Instruments

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

	Fair Value Measurements as of March 31, 2024 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market mutual funds	$ 17,107	$ -	$ -	$ 17,107
	17,107	-	-	17,107
Investments:				
U.S. Treasury securities	-	273,846	-	273,846
Corporate Bonds	-	21,191	-	21,191
	-	295,037	-	295,037
Total assets	$ 17,107	$ 295,037	$ -	$ 312,144
Liabilities:				
Warrant liability	$ -	$ -	$ 765	$ 765
Total liabilities	$ -	$ -	$ 765	$ 765

	Fair Value Measurements as of December 31, 2023 Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market mutual funds	$ 50,737	$ -	$ -	$ 50,737
U.S. Treasury Securities	-	2,487	-	2,487

	50,737	2,487	-	53,224
Investments:				
U.S. Treasury securities	-	312,696	-	312,696
Corporate Bonds	-	38,895	-	38,895
	-	351,591	-	351,591
Total assets	$ 50,737	$ 354,078	$ -	$ 404,815
Liabilities:				
Warrant liability	$ -	$ -	$ 1,876	$ 1,876
Total liabilities	$ -	$ -	$ 1,876	$ 1,876

The Company classifies its money market mutual funds 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 U.S. Treasury Securities and Corporate 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.

The reconciliation of the Company's warrant liability, which is recorded as part of other liabilities in the Consolidated Balance Sheets, measured at fair value on a recurring basis using unobservable inputs (Level 3) is as follows:

	Warrant Liability
Balance, December 31, 2023	$ 1,876
Fair value adjustments	(1,111)
Balance, March 31, 2024	$ 765

The Company utilizes a Black-Scholes model to value the warrant liability (see Note 10 "Warrants") at each reporting period, with changes in fair value recognized in the Consolidated Statements of Operations. The estimated fair value of the warrant liability is determined using Level 3 inputs. Inherent in an options pricing model are assumptions related to expected share-price volatility, expected life, risk-free interest rate and dividend yield. The Company estimates the expected volatility of its common stock based on historical volatility of the Company and of a peer group, considering the expected remaining life of the warrants. The risk-free interest rate is based on the U.S. Treasury zero-coupon yield curve on the valuation date for a maturity similar to the expected remaining life of the warrants. The expected life of the warrants is assumed to be equivalent to their remaining contractual term. The dividend rate is based on the historical rate, which the Company anticipates will remain at zero**.**

The fair value of the warrant liability has been estimated with the following assumptions:

	March 31, 2024	December 31, 2023
Stock price	$ 26.94	$ 29.50
Exercise price	$ 65.23	$ 65.23
Expected volatility	63.34 %	68.83 %
Risk-free interest rate	5.04 %	4.70 %
Expected dividend yield	-	-
Expected life (years)	1.06	1.31
Fair value per warrant	$ 1.24	$ 3.04

5. Property and Equipment, Net

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

	March 31, 2024	December 31, 2023
Laboratory equipment	$ 30,777	$ 29,232
Machinery and equipment	12,508	12,325
Computer equipment	244	244

Furniture and fixtures	2,777	2,777
Leasehold improvements	6,876	6,723
Internal use software	1,903	1,903
	55,085	53,204
Less: accumulated depreciation and amortization	(15,748)	(14,032)
Total property and equipment, net	$ 39,337	$ 39,172

During the three months ended March 31, 2024 and 2023, the Company recognized $

1.7
million and $

1.1
million of depreciation and amortization expense, respectively.

6. Intangible Assets and Goodwill

The Company's indefinite lived intangible assets consist of an acquired IPR&D asset received in the acquisition of Renovacor on December 1, 2022. Intangible assets as of March 31, 2024 and December 31, 2023 are summarized as follows:

March 31, 2024	Gross Carrying Value	Accumulated Amortization	Intangible Assets, Net
In process research & development	$ 25,150	$ -	$ 25,150
Total intangible assets	$ 25,150	$ -	$ 25,150

December 31, 2023	Gross Carrying Value	Accumulated Amortization	Intangible Assets, Net
In process research & development	$ 25,150	$ -	$ 25,150
Total intangible assets	$ 25,150	$ -	$ 25,150

The carrying value of Goodwill as of March 31, 2024 and December 31, 2023 was $ 39.2 million.

7. Accounts Payable and Accrued Expenses

The Company's accounts payable and accrued expenses consisted of the following:

	March 31, 2024	December 31, 2023
Research and development	$ 15,216	$ 13,867
Investment payable	-	13,137
Employee compensation	4,192	9,930
Property and equipment	923	1,077
Professional fees	7,003	6,006
Other	2,751	1,772
Total accounts payable and accrued expenses	$ 30,085	$ 45,789

The $ 13.1 million investment payable at December 31, 2023 was related to investment purchases of available-for-sale securities in 2023 that settled in 2024.

8. 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 has reimbursed Cowen for certain expenses incurred in connection with the Sales Agreement. On September 12, 2023, the Company and Cowen entered into the Amended Sales Agreement pursuant to which the aggregate offering amount available under the at-the-market offering program was reduced to $

180.0

million. Through March 31, 2024, the Company sold

4.2

million shares under the at-the-market offering program for gross proceeds of $

65.8

million, less commissions of $

2.0

million for net proceeds of $

63.8

million. The Company did

no

t sell any shares under the at-the-market offering program during the three months ended March 31, 2024.

9. Stock-Based Compensation

Stock Option Valuation

The weighted average assumptions that the Company used in a Black-Scholes pricing model to determine the fair value of stock options granted to employees, non-employees and directors were as follows:

	Three Months Ended March 31,	
	2024	2023
Risk-free interest rate	5.16%	4.02%
Expected term (in years)	5.88	5.88
Expected volatility	73.62%	73.54%
Expected dividend yield	0.00%	0.00%
Exercise price	$ 29.46	$ 20.17
Fair value of common stock	$ 29.46	$ 20.17

The following table summarizes stock option activity for the three months ended March 31, 2024:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Contractual Term (Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2023	14,863,996	$ 15.07	5.16	$ 250,602
Granted	1,174,939	29.46	9.88	
Exercised	(73,745)	16.05		956
Cancelled or forfeited	(41,384)	18.18		
Outstanding as of March 31, 2024	15,923,806	$ 16.12	5.24	$ 208,603
Options vested and exercisable as of March 31, 2024	12,351,611	$ 14.14	4.14	$ 190,665
Options unvested as of March 31, 2024	3,572,195	$ 22.94	9.04	$ 17,937

The weighted average grant-date fair value per share of stock options granted during the three months ended March

31, 2024, and 2023 was $

19.92
and $

13.50
, respectively.

The total fair value of options vested during the three months ended March 31, 2024 and 2023 was $

11.5
million and $

11.4
million, respectively.

Restricted Stock Units

The following table summarizes the Company's RSU activity for the three months ended March 31, 2024:

	Number of Shares	Weighted Average Grant Date Fair Value
Unvested as of December 31, 2023	1,490,357	$ 18.53
Granted	566,366	29.24
Vested	(290,578)	19.38
Forfeited	(25,400)	17.04
Unvested as of March 31, 2024	1,740,745	$ 21.89

Performance Stock Units

The following table summarizes the Company's PSU activity for the three months ended March 31, 2024:

	Number of Shares	Weighted Average Grant Date Fair Value
Unvested as of December 31, 2023	-	$ -
Granted	139,323	28.71
Vested	-	-
Forfeited	-	-
Unvested as of March 31, 2024	139,323	$ 28.71

PSU vesting and expense recognition is based on achievement of specific performance goals within certain time periods. PSU awards that are not achieved within specific time periods are forfeited. No performance goals were probable of achievement and no time periods had expired on grant date and as of March 31, 2024.

Stock-Based Compensation Expense

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

	Three Months Ended March 31, 2024	2023
Stock options	$ 6,938	$ 6,985
Restricted stock units	3,314	1,930
Total stock-based compensation expense	$ 10,252	$ 8,915

Stock-based compensation expense by classification included within the Consolidated Statements of Operations and Comprehensive Loss was as follows:

	Three Months Ended March 31, 2024	2023
Research and development	$ 4,637	$ 3,819
General and administrative	5,615	5,096
Total stock-based compensation expense	$ 10,252	$ 8,915

As of March 31, 2024, the Company had an aggregate of $

85.3

million of unrecognized stock-based compensation expense related to stock options, RSU and PSU grants. The stock options and RSU grants had an aggregate of $

81.3

million of unrecognized stock-based compensation expense, which is expected to be recognized over a weighted average period of 1.98 years.

10. Warrants

A summary of the warrants outstanding as of March 31, 2024 is as follows:

Exercise Price	Outstanding	Grant/Assumption Date	Expiration Date
$ 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
65.23	617,050	December 1, 2022	April 23, 2025
65.23	760,086	December 1, 2022	December 1, 2026
$ 0.01	3,126,955	September 15, 2023	N/A
Total	5,715,078		

The following table is a summary of changes in warrants to purchase common stock for the three months ended March 31, 2024:

	Number of Warrant Shares Outstanding and Exercisable	Exercise Price per Share
Balance as of December 31, 2023	5,715,078	
Issued	-	$ -
Exercised	-	-
Expired	-	$ -
Balance as of March 31, 2024	5,715,078	

Warrants Issued in Public Offering

On September 15, 2023, the Company completed a public offering that included pre-funded warrants to purchase

3,126,955
shares of common shares at a price of $

0.01
per share (see Note 8 “Stockholders’ Equity” and Note 17 “Related Party Transactions”).

Assumed Renovacor Public Warrants

In conjunction with the acquisition of Renovacor, Rocket assumed pre-acquisition public warrants (“Public Warrants”) that were converted into Rocket warrants with a right to purchase

760,086
of Rocket common shares at an exercise price of $

65.23
per share.

The Company determined that the Public Warrants met all of the criteria for equity classification. Accordingly, upon closing of the acquisition, the Public Warrants were recorded as a component of additional paid-in capital of $

3.4
million.

Assumed Renovacor Private Warrants

In conjunction with the acquisition of Renovacor, Rocket assumed pre-acquisition private warrants (“Private Warrants”) that were converted into Rocket warrants with a right to purchase

617,050
of Rocket common shares at an exercise price of $

65.23
per share.

The Company determined that the Private Warrants did not meet all of the criteria for equity classification. Accordingly, the Company classifies the Private Warrants as derivative liabilities in its Consolidated Balance Sheets. The Company measures the fair value of the warrants at the end of each reporting period and recognizes changes in the fair value from the prior period in the Company’s operating results for the current period. See Note 4 “Fair Value of Financial Instruments” for discussion of fair value measurement of the warrant liability.

11. Net Loss Per Share

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

	Three Months Ended March 31,	
	2024	2023

Numerator:		
Net loss attributable to common stockholders	$ (62,054)	$ (58,335)
Denominator:		
Weighted-average common shares outstanding - basic and diluted	93,549,884	79,453,519
Net loss per share attributable to common stockholders - basic and diluted	$ (0.66)	$ (0.73)

For the three months ended March 31, 2024, the Company included the 3,126,955 potential shares from the pre-funded warrants acquired by RTW as it was determined that these met the definition for equity classification as the holder is only required to pay $ 0.01 per share upon exercise of the pre-funded warrants.

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

	Three Months Ended March 31,	
	2024	2023
Warrants exercisable for common shares	2,588,123	2,595,174
Restricted stock units	1,740,745	1,622,457
Performance stock units	139,323	-
Options to purchase common shares	15,923,806	14,651,390
Total potential shares excluded from diluted net loss per share	20,391,997	18,869,021

12. Leases

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 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 as part of deposits in the Consolidated Balance Sheets as of March 31, 2024 and December 31, 2023.

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 $

0.8
million. On March 26, 2021, the Company entered into 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. On March 29, 2024, the Company entered into Amendment No. 2 to the ESB Lease Agreement that extended the term of the lease agreement to July 31, 2027. The Empire LOC serves as the Company's security deposit on the lease in which the landlord is the beneficiary and expires September 30, 2027 .

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 March 31, 2024 and December 31, 2023.

On November 15, 2022, the Company entered into a lease agreement with a lease term until October 31, 2024 , for laboratory space in Madrid, Spain. The lease commenced on April 1, 2023 and the Company recognized a right-of-use asset and corresponding lease liability of approximately $

0.2
million each.

On December 1, 2022, in connection with the acquisition of Renovacor, the Company acquired the Renovacor operating leases for space at facilities in Hopewell, New Jersey and Cambridge, Massachusetts with remaining lease terms of approximately 10.3 and 1.3 years, respectively. The Company recognized total right-of-use assets of $

3.8
million with corresponding total lease liabilities of $

3.6
million at lease commencement dates. The Company signed an agreement to sublease one of these facilities in January 2024 and intends to sublease the other remaining facilities. Rental income received under the sublease agreement totaled $

0.1
million for the three months ended March 31, 2024.

Rent expense under operating leases was $

0.6
million and $

0.6
million for the three months ended March 31, 2024 and 2023, respectively.

The total restricted cash balance for the Company's operating and finance leases as of March 31, 2024 and December 31, 2023 was $

0.8

million.

Operating lease cost was $

0.4
million and $

0.4
million for the three months ended March 31, 2024 and 2023, respectively.

The following table summarizes lease cost for the three months ended March 31, 2024 and 2023:

Lease cost	Three Months Ended March 31, 2024	2023
Operating lease cost	$ 369	$ 358
Finance lease cost:		
Amortization of right of use assets	538	538
Interest on lease liabilities	471	468
Total lease cost	$ 1,378	$ 1,364

The following table summarizes the future lease payments of the Company's operating and finance lease liabilities on an undiscounted cash flow basis:

Fiscal Year Ending December 31,	March 31, 2024
2024 (nine months)	$ 815
2025	1,001
2026	1,005
2027	759
2028	522
Thereafter	2,420
Total lease payments	$ 6,522
Less: interest	(1,744)
Total operating lease liabilities	$ 4,778

Fiscal Year Ending December 31,	March 31, 2024
2024 (nine months)	$ 1,348
2025	1,856
2026	1,911

2027		1,969
2028		2,028
Thereafter		41,003
Total lease payments	$	50,115
Less: interest		(28,944)
Total finance lease liability	$	21,171

The following table summarizes the operating and financing lease liabilities and right-of-use assets as of March 31, 2024 and December 31, 2023:

Leases	**March 31, 2024**	**December 31, 2023**
Operating right-of-use assets	$ 4,768	$ 3,901
Operating current lease liabilities	$ 1,064	$ 925
Operating noncurrent lease liabilities	3,714	2,973
Total operating lease liabilities	$ 4,778	$ 3,898
Finance right-of-use assets	$ 43,979	$ 44,517
Finance current lease liability	$ 1,808	$ 1,791
Finance noncurrent lease liability	19,363	19,353
Total finance lease liability	$ 21,171	$ 21,144

Other Information	Three Months Ended March 31, 2024	2023
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases	$ 333	$ 261
Cash flows from finance lease	$ 443	$ 431
Weighted-average remaining lease term - operating leases	7.1 years	8.2 years
Weighted-average remaining lease term - finance lease	20.4 years	21.4 years
Weighted-average discount rate - operating leases	8.82%	8.08%
Weighted-average discount rate - finance lease	8.96%	8.96%

13. Commitments and Contingencies

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.

14. 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 R&D activities has no alternative future uses.

15. CIRM Grants

LAD-I CIRM Grant

On April 30, 2019, the California Institute for Regenerative Medicine awarded the Company up to $

7.5

million under a CLIN2 grant award to support the clinical development of its LV-based gene therapy for RP-L201. Proceeds from the grant would 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 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. As of March 31, 2024, the Company has received $

5.9

million in total RP-L201 grants from CIRM. The Company received a final milestone grant of $

0.05

million on January 2, 2024 and

no
additional payments are available under the grant awards program as of March 31, 2024.

16. Related Party Transactions

In June 2023, the Company entered into a consulting agreement with the spouse of one of the Company's executive officers for information technology advisory services. The Company incurred expenses of approximately $

0.002
million for the three months ended March 31, 2024, relating to services provided under this agreement.

In September 2023, in connection with a public offering, the Company sold approximately

3.1
million pre-funded warrants to purchase shares of the Company's common stock to funds affiliated with RTW Investments, LP, the Company's largest shareholder (see Note 8 "Stockholders' Equity").

17. 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 months ended March 31, 2024 and 2023, was $

0.4
million and $

0.3
million, respectively.

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

You should read the following discussion and analysis of our financial condition and results of operations together with the 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 27, 2024 with the SEC. 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 2023 Form 10-K and 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 2023 Form 10-K.

We are a fully integrated, late-stage 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 programs, which include programs for:

- Fanconi Anemia, 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, a genetic disorder that causes the immune system to malfunction; and
- Pyruvate Kinase Deficiency, a red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia.

In September 2023, the FDA accepted the Biologics License Application and granted priority review for RP-L201 for the treatment of severe LAD-I. Treatments in the FA Phase 2 studies were completed in 2023 with regulatory filings in the U.S. and EU for FA anticipated in 2024. Additional work on a gene therapy program for the less common FA subtypes C and G is ongoing.

In the U.S., we also have two clinical stage and one pre-clinical stage *in vivo* adeno-associated virus programs, which include programs for:

- Danon Disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. The DD program is currently in an ongoing Phase 2 trial.
- Plakophilin-2 Arrhythmogenic Cardiomyopathy, an inheritable cardiac disorder that is characterized by a progressive loss of cardiac muscle mass, severe right ventricular dilation, dysplasia, fibrofatty replacement of the myocardium and a high propensity to arrhythmias and sudden death. This program received FDA clearance of an Investigational New Drug application and we have initiated a Phase 1 study.
- BAG3 Dilated Cardiomyopathy, which is the most common form of cardiomyopathy and is characterized by progressive thinning of the walls of the heart resulting in enlarged heart chambers that are unable to pump blood. Our program utilizes recombinant AAV9-based gene therapy designed to slow or halt progression of BAG3-DCM.

We have global commercialization and development rights to all of these product candidates under royalty-bearing license agreements.

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 our 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 have reimbursed Cowen for certain expenses incurred in connection with the Sales Agreement. On September 12, 2023, the Company and Cowen entered into an amendment pursuant to which the aggregate offering amount available under the at-the-market offering program was reduced to $180.0 million. Through March 31, 2024, we sold 4.2 million shares under the at-the-market offering program for gross proceeds of $65.8 million, less commissions of $2.0 million for net proceeds of $63.8 million. We did not sell any shares under the at-the-market offering program during the three months ended March 31, 2024.

Gene Therapy Overview

Genes are composed of sequences of deoxyribonucleic acid, which provide the 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.

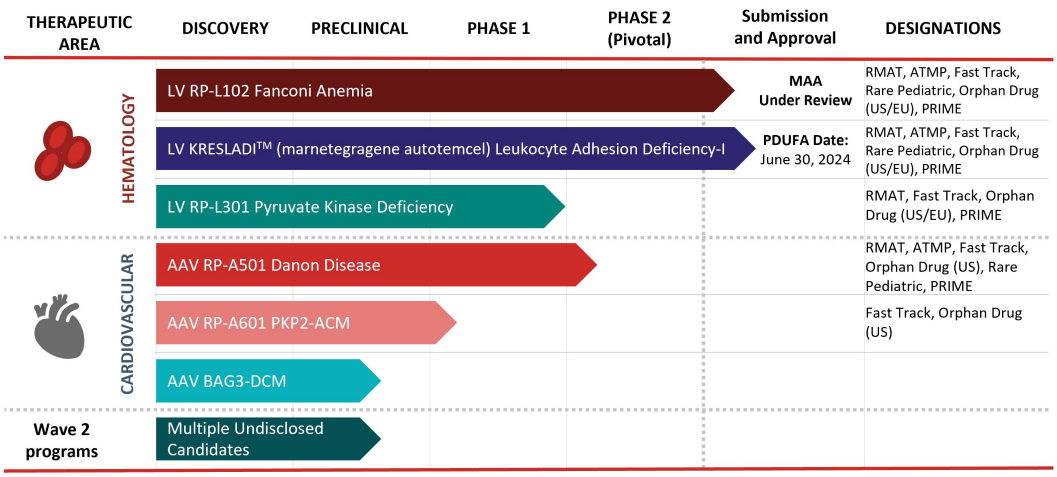
We are using modified non-pathogenic viruses for the development of our gene therapy treatments. Viruses are particularly well suited as delivery vehicles because they are adept at penetrating cells and delivering genetic material inside a cell. In creating our viral delivery vehicles, the viral (pathogenic) 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, LV and AAV. We believe that our LV and AAV-based programs have the potential to offer a significant and long-lasting therapeutic benefit to patients.

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 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 our programs and product candidates:



Cardiovascular Programs

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, 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 are not believed to modify progression to end-stage congestive heart failure. Patients with end-stage congestive heart failure 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 DD, which is estimated to have a prevalence of 15,000 to 30,000 patients in the U.S. and the EU.

DD 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. DD predominantly affects males early in life and is characterized by absence of *LAMP2B* expression in the heart and other tissues. Preclinical models of DD have demonstrated that AAV-mediated transduction of the heart results in reconstitution of *LAMP2B* expression and improvement in cardiac function.

We currently have one AAV program targeting DD, RP-A501. We have treated seven patients in the RP-A501 Phase 1 clinical trial, which enrolled adult/older adolescent 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 a pediatric cohort at a low dose level (6.7e13 gc/kg; n=2).

As previously disclosed, a patient receiving therapy on the high dose cohort (1.1e14 gc/kg dose) had progressive heart failure and underwent a heart transplant at month five following therapy. This patient had more advanced disease than the four other adult/older adolescent patients who received treatment in the low and high dose cohorts, as evidenced by diminished baseline left ventricle ejection fraction (35%) on echocardiogram and markedly elevated left ventricle filling pressure prior to treatment. The patient's clinical course was characteristic of DD progression. The patient is doing well post-transplant.

Based on the initial efficacy observed in the low dose cohort and to mitigate complement-mediated safety concerns observed in the high dose cohort (thrombotic microangiopathy) 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.

We conducted a variety of efficacy assessments in the Phase I clinical study to measure the prospect of benefit for patients. These assessments included the following:

- New York Heart Association Functional Classification 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. Class III and IV are considered more severe or advanced heart failure.
- Brain natriuretic peptide is a blood-based evaluation and a key marker of heart failure with prognostic significance in congestive heart failure and cardiomyopathies. Elevations in BNP are strongly associated with worsening heart failure and poor outcomes in cardiovascular disease.
- High sensitivity troponin I is a blood-based evaluation and a key marker of cardiac injury, one that is (like BNP) frequently elevated in DD patients and has been shown to be markedly elevated in patients with advanced stage disease.
- Echocardiographic measurements of heart thickness, most notably, left ventricular mass and maximal left ventricular wall
thickness, indicate the degree of hypertrophy present in the heart.

• Kansas City Cardiovascular Questionnaire is a validated, patient-reported outcomes assessment that measures a patients perception of their heart failure symptoms, impact of disease on physical and social function, and the impact of their heart failure on overall health status and quality of life. Assessment scores range from 0 (very poor health status) to 100 (excellent health status). Changes in KCCQ score of +/- 5 points are considered meaningful and have been shown to correlate with outcomes.

• Histologic examination of endomyocardial biopsies via hematoxylin and eosin histology and electron microscopy is used to detect 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.

• LAMP2 gene expression in endomyocardial biopsy samples is measured via both immunohistochemistry and Western blot and confirms the presence of LAMP2 protein in DD cardiac tissue following RP-A501 treatment.

On January 9, 2023, we presented positive efficacy updates from our Phase I study of RP-A501 during the 41st Annual J.P. Morgan Healthcare Conference. The data presented included several additional months of follow-up, which showed further improvements in key biomarkers, echocardiographic and functional measures. A summary of these updates is provided in the table below. We also provided additional natural history comparator data, which showed the marked divergence of the course of Phase I patients from that of untreated patients in terms of key biomarkers (BNP) and functional measures (NYHA Class). Furthermore, RP-A501 continued to be well tolerated at 2-3 years post treatment in both adult/older adolescent high and low-dose cohorts and at 8 to 13 months in the pediatric cohort. In the pediatric cohort, no significant immediate or delayed toxicities, significant skeletal myopathy, or late transaminase elevation have been observed.

Improvement or Stabilization Observed Across Key Biomarker, Echo Findings and Functional Measures in Phase 1 RP-A501 study

Cohort	Patient ID	Most recent visit (months)	Δ hsTnI	Δ BNP	Δ LV mass	Δ LV max wall thickness	Δ NYHA class	Δ KCCQ score
Low dose pediatric	1008	12	↓86%	↓83%	↓29%[1]	↓15%[1]	II -> I	+32.3
	1009	6	↓90%	↓62%	↓21%	↑3%	II -> I	+26
Low dose adult/ adolescent	1001	36	↓98%	↑8%	↓32%	↓9%	II -> II	+5.3
	1002	36	↓96%	↓94%	↓48%	↓40%	II -> I[2]	+17.8
	1005	30	↓46%	↑6%	↓14%	↓27%	II -> I	+8.3[3]
High dose adult/ adolescent	1006	24	↓63%	↓69%	↓27%	↓15%	II -> I	+3.1

Darker Green = improved; Lighter Green = minimal change (stabilization).
Does not include Patient 1007 in Phase 1 trial who had advanced heart failure with ejection fraction < 40% at enrollment and received heart transplantation 5 months following treatment due to pre-existing advanced heart failure. Patient is currently stable. Data cut-off September 27, 2022.

[1] Patient 1008 echocardiographic parameters are M9 visit (M12 pending).

[2] Patient 1002 NYHA class depicted for M30 visit (M36 pending).

[3] Patient 1005 KCCQ score depicted for M24 visit (M30 pending).

In addition to these clinical updates, we also provided updates on our in-house manufacturing activities. As of January 2023, we had successfully produced 2 cGMP RP-A501 batches that have superior specifications to Phase I material in both titer and full versus empty particles. We believe the improved quality of our in-house manufactured product will allow for full dosing with lower total viral particles, potentially further optimizing the safety profile of RP-A501. Furthermore, we have agreement from the FDA on the continued utilization of HEK-293 cell-based process through commercialization as well as our comparability approach and potency assay.

In May 2023, we presented previously disclosed results from the Phase I study of RP-A501 at the ASGCT 26th Annual Meeting. As of the most recent data extraction, all six patients that remain in follow-up continued to show signs of improvement or stabilization.

Results from the ongoing Phase 1 DD trial represent one of the most comprehensive investigational gene therapy datasets for any cardiac condition. RP-A501 was generally well tolerated with evidence of durable treatment activity and improvement of DD for both pediatric patients with up to nine months of follow-up and four adult/older adolescent patients with up to 36 months of follow-up. All adult/older adolescent and pediatric patients who received a closely monitored immunomodulatory regimen showed improvements across tissue, laboratory, and imaging-based biomarkers, as well as in NYHA class (from II to I) and KCCQ scores with follow-up of six to 36 months.

On September 12, 2023, we announced that alignment was reached with the FDA on the global Phase 2 pivotal trial of RP-A501 for DD. The global, single-arm, multi-center Phase 2 pivotal trial will evaluate the efficacy and safety of RP-A501 in 12 patients with DD, including a pediatric safety run-in (n=2), with a natural history comparator and a dose level of 6.7×10^{13} GC/kg.

- To support accelerated approval, the study will assess the efficacy of RP-A501 as measured by the biomarker-based co-primary endpoint consisting of improvements in LAMP2 protein expression (≥ Grade 1, as measured by immunohistochemistry), and reductions in left ventricular mass.
- Key secondary endpoint is change in troponin. Additional secondary endpoints will include natriuretic peptide, KCCQ, NYHA class, event free survival to 24 months and treatment emergent safety events. These endpoints could support full approval with longer-term follow-up.
- A global natural history study will serve as an external comparator and run concurrently to the Phase 2 pivotal trial.
- In-house manufacturing has been completed with sufficient high-quality drug product produced to fully supply the Phase 2 pivotal study. Potency assays have been developed and qualified in accordance with FDA guidance.

Phase 2 enrollment and treatment in the U.S. is ongoing. We have filed the Clinical Trial Application and Investigational Medicinal Product Dossier for RP-A501 with the relevant Member States through the EU Clinical Trial Information System and the Medical and Healthcare Products Regulatory Agency. In January 2024, we received CTIS approval and MHRA approval for these clinical trial applications. We are initiating Phase 2 pivotal trial activities in Europe and the UK.

Recently Achieved Milestones

On February 7, 2023, we announced that RP-A501 received regenerative medicine advanced therapy designation from the FDA, and on May 31, 2023, we received priority medicines designation from the EMA. On September 12, 2023, we announced our alignment with the FDA on our pivotal study design for RP-A501 in DD and enrollment in the global study is ongoing.

Plakophilin-2 Arrhythmogenic Cardiomyopathy

Arrhythmogenic cardiomyopathy is an inheritable cardiac disorder that is characterized by a high propensity for arrhythmias and sudden death, a progressive loss of cardiac muscle mass, severe right ventricular dilation, dysplasia, and fibrofatty replacement of the myocardium. Most commonly, the cardiomyopathy initially manifests in the right ventricular free wall, so the disease was termed arrhythmogenic right ventricular dysplasia/cardiomyopathy. However, since left dominant and biventricular forms have also been observed, this has led more recently to the use of the term ACM. Mutations in the PKP2 gene comprise the most frequent genetically identified etiology of familial ACM. PKP2 encodes for the protein Plakophilin-2, which is a component of the desmosome, an intercellular complex involved in cell-cell adhesion. PKP2 is also involved in transcriptional regulation of calcium signaling between cardiomyocytes. Patients with mutations in PKP2 are typically heterozygous and demonstrate reduced expression of PKP2 in the myocardium. Mean presentation is at the age of 35, and patients have a very high lifetime risk of ventricular arrhythmias, structural ventricular abnormalities, and sudden cardiac death.

There are no specific available medical therapies available that have been shown to be highly effective for ACM, and current treatment protocols follow standard ventricular arrhythmia and cardiomyopathy guidelines, which involve lifestyle modifications (i.e. exercise limitation) and include drug treatments such as beta blockers, anti-arrhythmics and diuretics. The use of these therapies is driven by the arrhythmia burden and severity of cardiomyopathy. These therapies do not modify the course of the disease, and generally provide only symptomatic and/or palliative support. Upon diagnosis, a substantial percentage of patients receive an implantable cardiac defibrillator for primary or secondary prevention of ventricular arrhythmias and sudden cardiac death. Of note, ICDs are not curative, and breakthrough life-threatening arrythmias may persist with ongoing risk of death. Furthermore, ICDs do not prevent the progression to end-stage heart failure. ICD firings, although lifesaving, are physically and emotionally traumatic events. Patients whose condition progresses to end-stage heart failure are considered for cardiac transplantation which, while curative of underlying disease, is itself associated with significant morbidity and mortality. Hence there exists a high unmet medical need in this population. PKP2-ACM is estimated to have a prevalence of 50,000 patients in the U.S. and EU.

We currently have one AAV program targeting PKP2-ACM, RP-A601, which is a recombinant AAVrh.74 vector expressing PKP2a. PKP2-ACM is typically caused by heterozygous pathogenic mutations in the PKP2 gene resulting in reduced PKP2 expression in the myocardium. A once-administered gene therapy that addresses the root cause of the disease (PKP2 deficiency) early in the disease course, could mitigate the early electrical remodeling and diminish the risk of life-threatening arrhythmias and sudden cardiac death associated with ACM, potentially impeding the development of irreversible cardiac structural changes. Prevention of syncopal episodes, life-threatening arrythmias, sudden cardiac death, ICD shocks and the resulting anxiety, discomfort and hospitalizations is anticipated to result in a vastly improved quality of life and survival benefit. Furthermore, such an approach could spare patients the need for lifelong adherence to multiple arrhythmia and heart failure drugs that are nonspecific for PKP2-ACM and are associated with their own side effects, enabling patients an opportunity to live without exercise restrictions and with diminished concern for arrhythmias, palpitations, ICD shocks and progression to end-stage heart failure.

In May 2023, we presented preclinical efficacy data for RP-A601 at the American Society of Gene and Cell Therapy 26th Annual meeting. Nonclinical studies conducted by the Sponsor, RP-A601 have demonstrated efficacy in altering the natural history of PKP2-driven ACM. 100% of PKP2 cKO animals treated with the study drug exhibited extended survival to the longest timepoint measured (5 months), reduced cardiac dilation and fibrofatty replacement/fibrosis of the myocardium, preserved left ventricular function, and mitigation of the arrhythmic phenotype. Untreated PKP2 cKO mice had a median survival of approximately one month. These results were published in January 2024 in the journal Circulation: Genomic and Precision Medicine.

We have initiated a multi-center Phase 1 study for RP-A601. The multi-center Phase 1, dose escalation trial will evaluate the safety and preliminary efficacy of RP-A601 in at least six adult PKP2-ACM patients with ICDs and overall high risk for arrhythmias. The study will assess the impact of RP-A601 on PKP2 myocardial protein expression, cardiac biomarkers, and clinical predictors of life-threatening ventricular arrhythmias and sudden cardiac death. Patients in the dose-escalation trial will receive a single dose of RP-A601. The starting dose will be 8×10^{13} GC/kg.

Recently Achieved Milestones

We have achieved pre-clinical proof-of-concept for RP-A601 in an animal model representative of PKP2-ACM, completed pharmacology and GLP toxicology studies, produced GMP drug product, and developed an appropriate potency assay to support a Phase I study. On May 9, 2023, we announced FDA clearance of the IND, and on June 8, 2023, we announced receipt of FDA Fast Track and Orphan Drug Designations. Enrollment in the U.S. Phase 1 study is ongoing.

BAG3 Dilated Cardiomyopathy

Dilated cardiomyopathy is the most common form of cardiomyopathy and is characterized by progressive thinning of the walls of the heart resulting in enlarged heart chambers that are unable to pump blood. A familial association of DCM can be identified in 20-50% of DCM patients, with up to 40% of familial patients having an identifiable genetic cause. Mutations in the BAG3 gene (BCL-2-associated athanogene 3) are among the more common pathogenic genetic variants observed in familial DCM and these variants are highly penetrant, with approximately 80% of individuals with disease-causing genetic variants in the BAG3 gene developing DCM at > 40 years of age. BAG3 protein is associated with a variety of cellular functions including cardiac contractility, protein quality control (as a co-chaperone), cardiomyocyte structural support and anti-apoptosis. BAG3 associated dilated cardiomyopathy (BAG3-DCM) leads to early onset, rapidly progressing heart failure and significant mortality and morbidity. We estimate that the prevalence of BAG3-associated DCM in the U.S. to be as many as 30,000 individuals.

Currently, DCM patients with a BAG3 mutation are treated with the standard of care for heart failure, which include angiotensin converting enzyme inhibitors, angiotensin receptor blockers, neprilysin inhibitors, beta-adrenergic receptor antagonists, or beta-blockers, aldosterone antagonists and/or diuretics, along with certain lifestyle changes, and do not address the underlying cause of disease. Patients who meet specific parameters may also undergo placement of an implantable cardioverter defibrillator, a cardiac resynchronization device or a combination of the two. There is no current therapy directly targeting the underlying mechanism of BAG3 associated DCM, and patients diagnosed with BAG3 associated DCM appear to progress to end-stage heart failure and death more rapidly than patients with DCM not associated with BAG3 variants. For example, approximately 19% of patients with BAG3-DCM require mechanical cardiac support, heart transplant, or have heart failure related death at 12 months after diagnosis, nearly twice the rate of similarly staged non-BAG3-DCM patients.

In December 2022 we completed our acquisition of Renovacor which provided Rocket with Renovacor's recombinant AAV9-based gene therapy program designed to deliver a fully functional BAG3 gene to augment BAG3 protein levels in cardiomyocytes and slow or halt progression of BAG3-DCM. Initial proof of concept for AAV9-BAG3 has been demonstrated in studies of BAG3-knockout mouse models, which show treated mice have improved ejection fraction versus untreated knockout mice and comparable ejection fraction to walk test controls at timepoints 4- and 6-weeks post injection.

Recently Achieved Milestones

We are in the process of evaluating the optimal development pathway for this program and plan to submit an IND for BAG3-DCM in 2024.

Hematology Programs

Fanconi Anemia Complementation Group A

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 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, currently the most frequently utilized therapy for FA, have resulted in frequent hematologic correction of the disorder, hematopoietic stem cell transplant is associated with both acute and long-term risks, including transplant-related mortality, graft failure, and graft versus host disease, 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 early in the disease course and administered without conditioning. We believe that the development of a broadly applicable autologous gene therapy can be transformative for these patients.

Each of our hematology programs utilize third-generation, self-inactivating LV 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 bone marrow failure, acute myeloid leukemia, and myeloid dysplastic syndrome types of blood cancers. FA patients also typically present with 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 LV-based program that we in-licensed from Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas, which is a leading research institute in Madrid, Spain. Our Phase 2 registrational enabling clinical trials treating FA patients with RP-L102 at the Center for Definitive and Curative Medicine at Stanford University School of Medicine, Great Ormond Street Hospital in London and Hospital Infantil de Nino Jesus in Spain completed treatment. The trial has treated a total of 12 patients from the U.S. and EU. Two additional patients were treated in the U.S. Phase 1 study at Stanford such that a total of 14 patients have received RP-L102 on Rocket-sponsored clinical trials. Patients 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 2022, we presented positive clinical data for RP-L102 at the 64th Annual Meeting of ASH. RP-L102 conferred phenotypic correction in at least six of 10 evaluable patients with ≥12 months of follow-up as demonstrated by increased resistance to MMC in bone marrow derived colony forming cells, concomitant genetic correction and hematologic stabilization. A seventh patient has displayed evidence of progressively increasing genetic correction as demonstrated by peripheral blood and bone marrow VCN’s, with recent development of MMC resistance and possible indicators of hematologic stability after 36 months of follow-up. 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. The safety profile of RP-L102 has been highly favorable, and the treatment, administered without any cytotoxic conditioning, has been well tolerated. No signs of bone marrow dysplasia, clonal dominance or insertional mutagenesis related to RP-L102 have been observed.

We had previously 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.

In May 2023, we presented updated clinical data for RP-L102 at the ASGCT 26th Annual Meeting. As of the data cut-off (April 17, 2023), RP-L102 conferred sustained genetic correction in eight of 12 evaluable patients and comprehensive phenotypic correction in seven of 12 evaluable patients with ≥12 months of follow up as demonstrated by increased resistance to mitomycin-C (MMC) in bone marrow-derived colony forming cells and hematologic stabilization. The safety profile of RP-L102 continues to be highly favorable with no signs of bone marrow dysplasia, clonal dominance or insertional mutagenesis related to RP-L102. Polyclonal integration patterns have been observed in each of the seven patients with phenotypic, genetic, and hematologic evidence of engraftment. Pivotal trial enrollment and treatment have been completed.

Anticipated Milestones

On April 2, 2024 we announced that the European Medicines Agency accepted our Marketing Authorization Application for RP-L102. We are finalizing the Chemistry, Manufacturing, and Controls package with the FDA and anticipate filing a BLA with the FDA in the first half of 2024.

Leukocyte Adhesion Deficiency-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 across the spectrum of severity 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 hematopoietic stem cell transplant. 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. 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 are serving as the lead clinical sites in Spain and London, respectively. This study has received a $5.9 million CLIN2 grant award from the 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. The first patient was treated at UCLA with RP-L201 in the third quarter of 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 2022, we presented positive clinical data at the 64th Annual Meeting of ASH. The presentation included previously disclosed top-line data at three to 24 months of follow-up after RP-L201 infusion for all patients and overall survival data for seven patients at 12 months or longer after infusion. We observed 100% overall survival at 12 months post-infusion via Kaplan Meier estimate and a statistically significant reduction in all hospitalizations, infection and inflammatory-related hospitalizations and prolonged hospitalizations for all nine LAD-I patients with three to 24 months of available follow-up. 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%). Data also shows evidence of resolution of LAD-I-related skin rash and restoration of wound repair capabilities. The safety profile of RP-L201 has been highly favorable in all patients with no RP-L201-related serious adverse events to date. 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.

In May 2023, at the ASGCT 26th Annual Meeting, we presented updated top-line data at 12 to 24 months of follow-up for all nine patients in our Phase 1/2 clinical trial showing 100% overall survival at 12 months post-infusion. All patients continue to demonstrate evidence of resolution of LAD-I-related skin rash and restoration of wound repair capabilities, and the safety profile of RP-L201 remains highly favorable with follow-up of 12-36 months. No evidence of replication-competent lentivirus has been observed. Insertion site analyses indicate highly polyclonal integration patterns across the entire cohort.

Recently Achieved and Anticipated Milestones

A BLA filing for RP-L201 was accepted by the FDA with priority review in October of 2023 with an initial Prescription Drug User Fee Act date of March 31, 2024. On February 13, 2024, the review time was extended by three months, to June 30, 2024, to allow additional time to review clarifying Chemistry, Manufacturing, and Controls information submitted by Rocket in response to FDA information requests. The FDA has further confirmed that an advisory committee meeting is not needed.

Pyruvate Kinase Deficiency

Red blood cell PKD is a rare autosomal recessive disorder resulting from mutations in the pyruvate kinase L/R gene encoding for a component of the red blood cell glycolytic pathway. PKD is characterized by chronic non-spherocytic hemolytic anemia, a disorder in which red blood cells 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 red blood cell 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 red blood cell 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 pyruvate kinase L/R 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. 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* LV-based program targeting PKD, RP-L301. RP-L301 is a clinical stage program that we in-licensed from CIEMAT.

We are conducting a global Phase 1 open-label, single-arm, clinical study has enrolled 2 adult patients and 2 pediatric patients (age 8-17) in the U.S. and Europe and is intended to assess the safety, tolerability, and preliminary activity of RP-L301. Stanford serves as the lead site in the U.S. for adult and pediatric patients, HNJ serves as the lead site in Europe for pediatrics, and Hospital Universitario Fundación Jiménez Díaz serves as the lead site in Europe for adult patients. Both adult and pediatric enrollment is completed in the Phase 1 study.

In May 2023, we presented positive updated clinical data at the ASGCT 26th Annual Meeting (data cut-off May 3, 2023), which included up to 30 months of follow-up from the two treated adult patients and early clinical data from the first pediatric patient treated with RP-L301. Robust and sustained efficacy was observed in both adult patients at up to 30 months post-infusion evidenced by normalized hemoglobin (from baseline pre-treatment levels in the 7.0-7.5 g/dL range), improved hemolysis parameters, and red blood cell transfusion independence. Furthermore, both adult patients reported improved quality of life with documented improvements via formal quality of life assessments. The safety profile continues to appear highly favorable, with no RP-L301-related serious adverse events in either of the adult patients. Insertion site analyses in peripheral blood and bone marrow in both adult patients through 24 months post-RP-L301 demonstrated highly polyclonal patterns and there has been no evidence of insertional mutagenesis. The first pediatric patient infusion of RP-L301 was well tolerated, with engraftment achieved at day +15, hospital discharge less than one month following infusion, no RP-L301 related serious adverse events and early signs of efficacy. There were no red blood cell transfusion requirements following engraftment. Both adult and pediatric enrollment is completed in the Phase 1 study.

In October 2023, we presented positive updated clinical data at the 30th Annual Congress at ESGCT (data cut-off October 9, 2023), including up to 36 months of follow-up in the adult cohort and more limited follow-up of 6 months in the pediatric cohort. Sustained efficacy has been demonstrated in adult cohort including hemoglobin normalization, transfusion independence, decreased hemolysis, and quality of life improvement; hemoglobin improvement relative to pre-treatment baseline has been observed in pediatric cohort. The safety profile remains favorable.

Recently Achieved Milestones

In early 2023, we announced receipt of FDA regenerative medicine advanced therapy designation and EMA priority medicines designation for RP-L301 based on the robust efficacy observed in the Phase 1 treated patients.

We have reached agreement with FDA on study design of Phase 2 pivotal trial of RP-L301. Based on positive safety and efficacy data from the Phase 1 study, we have aligned with the FDA on the pivotal study design to support accelerated approval and are initiating a 10-patient, single-arm Phase 2 pivotal trial with a primary endpoint of ≥1.5 point Hgb improvement at 12 months.

cGMP Manufacturing

Our 103,720 square foot manufacturing facility in Cranbury, New Jersey has been scaled up to manufacture AAV drug product for our Phase 2 pivotal study in DD. 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 DD.

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. 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 March 31, 2024, we raised net cash proceeds of approximately $1.0 billion 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.

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 that 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.

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 months ended March 31, 2024 and 2023:

	Three Months Ended March 31,	
	2024	2023
Direct Expenses:		
Danon Disease (AAV) RP-A501	$ 6,821	$ 6,403
Plakophilin-2 Arrhythmogenic Cardiomyopathy (AAV) RP-A601	1,193	-
Leukocyte Adhesion Deficiency (LVV) RP-L201	5,135	5,841
Fanconi Anemia (LVV) RP-L102	3,520	6,548
Pyruvate Kinase Deficiency (LVV) RP-L301	2,784	299
Other product candidates	2,386	3,439
Total direct expenses	21,839	22,530
Unallocated Expenses:		
Employee compensation	$ 13,617	$ 11,210
Stock based compensation expense	4,637	3,819
Depreciation and amortization expense	1,467	1,137
Laboratory and related expenses	1,034	5,102
Professional fees	1,148	985
Other expenses	1,485	1,588
Total other research and development expenses	23,388	23,841
Total research and development expense	$ 45,227	$ 46,371

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 clinical studies as well as any clinical studies and other R&D activities that we undertake in the future;
- 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 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 commercial, 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 continue to increase for the foreseeable future due to anticipated increases in headcount to support the continued advancement of our product candidates and our progression to commercial operations. 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 months ended March 31, 2024 and 2023 was related to our financing lease obligation for our Cranbury, NJ facility.

Interest and Other Income

Interest and other income related to interest earned from investments and cash equivalents and reduced fair value of warrant liability.

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 consolidated financial statements during the three months ended March 31, 2024 compared to those disclosed in our 2023 Form 10-K.

Results of Operations

Comparison of the Three Months Ended March 31, 2024 and 2023

	Three Months Ended March 31,		
	2024	2023	Change
Operating expenses:			
Research and development	$ 45,227	$ 46,371	$ (1,144)
General and administrative	22,148	15,823	6,325
Total operating expenses	67,375	62,194	5,181
Loss from operations	(67,375)	(62,194)	(5,181)
Interest expense	(471)	(468)	(3)
Interest and other income, net	3,029	1,908	1,121
Accretion of discount on investments, net	2,763	2,419	344
Total other income, net	5,321	3,859	1,462
Net loss	$ (62,054)	$ (58,335)	$ (3,719)

Research and Development Expenses

R&D expenses decreased $1.1 million to $45.2 million for the three months ended March 31, 2024 compared to the three months ended March 31, 2023. The decrease in R&D expenses was primarily driven by decreases in manufacturing and development and direct costs of $5.8 million. Decreases were partially offset by increases in the costs for compensation and benefits of $1.4 million due to increased R&D headcount, professional fees of $1.1 million, laboratory supplies of $0.9 million, non-cash stock compensation expense of $0.8 million, and clinical trial costs of $0.6 million.

General and Administrative Expenses

G&A expenses increased $6.3 million to $22.1 million for the three months ended March 31, 2024, compared to the three months ended March 31, 2023. The increase in G&A expenses was primarily driven by increases in commercial preparation related expenses of $3.3 million, legal expenses of $1.5 million, and non-cash stock compensation expense of $0.5 million.

Other Income, Net

Other income increased $1.5 million to $5.3 million for the three months ended March 31, 2024, compared to the three months ended March 31, 2023. The increase in other income was primarily driven by an increase in accretion of discount on investments, net, of $0.3 million and an increase in interest and other income, net, of $1.1 million. The increase in interest and other income, net, of $1.1 million was due to primarily decreased fair value of warrant liability of $1.1 million.

Liquidity and Capital Resources

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, those related to drug candidate development, technology and data security, patents and proprietary rights, our lack of commercial manufacturing marketing or sales 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. Rocket has incurred net losses and negative cash flows from its operations each year since inception. Rocket incurred net losses of $62.1 million for the three months ended March 31, 2024, and $245.6 million for the year ended December 31, 2023. We have experienced negative cash flows from operations and as of March 31, 2024 and December 31, 2023, we had an accumulated deficit of $1.02 billion and $959.4 million, respectively. As of March 31, 2024, we had $330.3 million of cash, cash equivalents and investments. Excluded from the $330.3 million of cash, cash equivalent and investments are receivables from maturity of securities that have yet to be received of $8.6 million recorded as part of prepaid expenses and other current assets. The net balance of cash, cash equivalent and investments balance when adjusting for this receivable would have been $338.9 million. We expect such resources would be sufficient to fund our operating expenses and capital expenditure requirements into 2026. We have funded our operations primarily through the sale of equity.

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

The following table summarizes our cash flows from operating, investing and financing activities, in thousands, for each of the periods presented:

	Three Months Ended March 31,	
	2024	2023
Net cash used in operating activities	$ (56,856)	$ (57,560)
Net cash provided by (used in) investing activities	35,034	(36,721)
Net cash provided by financing activities	1,184	18,343
Net decrease in cash, cash equivalents and restricted cash	$ (20,638)	$ (75,938)

Operating Activities

During the three months ended March 31, 2024, operating activities used $56.9 million of cash and cash equivalents, primarily resulting from our net loss of $62.1 million offset by net non-cash charges of $9.8 million, including non-cash stock-based compensation expense of $10.3 million, depreciation and amortization expense of $2.2 million, partially offset by accretion of discount on investments of $2.8 million. Changes in our operating assets and liabilities for the three months ended March 31, 2024 included a decrease in accounts payable and accrued expenses of $2.4 million, an increase in our prepaid expenses of $1.2 million, and a decrease in other liabilities of $1.0 million.

During the three months ended March 31, 2023, operating activities used $57.6 million of cash and cash equivalents, primarily resulting from our net loss of $58.3 million offset by net non-cash charges of $8.2 million, including non-cash stock-based compensation expense of $8.9 million, depreciation and amortization expense of $1.7 million, partially offset by accretion of discount on investments of $2.3 million. Changes in our operating assets and liabilities for the three months ended March 31, 2023, consisted of a decrease in accounts payable and accrued expenses of $7.8 million, a decrease in our prepaid expenses of $0.9 million, and a decrease in other liabilities of $0.7 million.

Investing Activities

During the three months ended March 31, 2024, net cash provided by investing activities was $35.0 million, primarily resulting from proceeds of $101.0 million from the maturities of investments, offset by purchases of investments of $63.9 million, and purchases of property and equipment of $2.0 million.

During the three months ended March 31, 2023, net cash used by investing activities was $36.7 million, primarily resulting from proceeds of $62.3 million from the maturities of investments, offset by purchases of investments of $96.0 million, and purchases of property and equipment of $3.0 million.

Financing Activities

During the three months ended March 31, 2024, net cash provided by financing activities was $1.2 million, consisting of proceeds of $1.2 million from the exercise of stock options.

During the three months ended March 31, 2023, financing activities provided $18.3 million of cash, primarily resulting from net proceeds of $17.2 million from the sale of shares through our at-the-market facility.

Contractual Obligations and Commitments

Information regarding contractual obligations and commitments may be found in Note 13 of our unaudited interim 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

Our exposure to market risk is principally confined to our cash, cash equivalents and marketable securities. We invest in U.S. treasury securities, corporate and agency bonds, which as of March 31, 2024, were classified as available-for-sale securities. We maintain our cash and cash equivalent balances with high-quality financial institutions and, consequently, we believe that such funds are subject to minimal credit risk. Our investment policy limits the amounts that we may invest in any one type of investment and requires all investments held by the Company to be at least AA-/Aa3 rated, thereby reducing credit risk exposure.

Our available-for-sale securities are subject to interest rate risk and will fall in value if market interest rates increase. If market interest rates were to increase immediately and uniformly by 100 basis points, or one percentage point, from levels at March 31, 2024, the net effect on the net fair value of our interest-sensitive marketable securities would have resulted in a hypothetical decline of $1.3 million. While we believe our cash, cash equivalents, and marketable securities do not contain excessive risk, we cannot provide absolute assurance that, in the future, our investments will not be subject to adverse changes in market value. In addition, we maintain significant amounts of cash, cash equivalents, and marketable securities at one or more financial institutions that are in excess of federally insured limits. Given the potential instability of financial institutions, we cannot provide assurance that we will not experience losses on these deposits. We do not utilize interest rate hedging agreements or other interest rate derivative instruments.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our principal executive and our principal financial officer,evaluated, as of the end of the period covered by this Quarterly Report on Form 10-Q, the effectiveness of our disclosure controls and procedures. Based on that evaluation of our disclosure controls and procedures as of March 31, 2024, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures as of such date were effective at the reasonable assurance level. The term “disclosure controls and procedures,” as defined in Rules 13a-15(f) and 15d-15(f) 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 during the period covered by this report that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II – OTHER INFORMATION

Item 1. Legal Proceedings

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

Item 1A. Risk Factors

Our material risk factors are disclosed in Item 1A of our 2023 Form 10-K. There have been no material changes from the risk factors previously disclosed in such filing.

Item 2. Unregistered Sales of Equity Securities, Use of Proceeds and Issuer Purchases of Equity Securities

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

During the three months ended March 31, 2024, none of our directors or officers adopted or terminated a “Rule 10b5-1 trading arrangement” or “non-Rule 10b5-1 trading arrangement,” as each term is defined in Item 408(a) of Regulation S-K.

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 Rocket Pharmaceuticals, Renovacor, Inc., Zebrafish Merger Sub, Inc. and Zebrafish Merger Sub II, LLC (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 (Declassify Board of Directors) 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)
4.1	Form of Pre-Funded Warrant (incorporated by reference to Exhibit 4.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on September 15, 2023)
10.1#*	Offer Letter, dated March 25, 2024 by and between the registrant and Aaron Ondrey
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 With Embedded Linkbase Documents.
104	Cover Page Interactive Data File (the cover page XBRL tags are embedded within the Inline XBRL document)

* Filed herewith.
# Indicates management contract or compensatory plan.
** 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.

May 7, 2024

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

May 7, 2024

By: /s/ Aaron Ondrey
Aaron Ondrey
Chief Financial Officer
(Principal Financial Officer)

Exhibit 10.1

EXECUTIVE EMPLOYMENT AGREEMENT

THIS EXECUTIVE EMPLOYMENT AGREEMENT (this "**Agreement**") is between Rocket Pharmaceuticals, Inc. a Delaware corporation (the "**Company**") and Aaron Ondrey ("**Executive**") and is dated as of March 25, 2024 (the "**Effective Date**"). Each of the Company and Executive are sometimes referred to in this Agreement individually as a "**Party**" and together as the "**Parties**."

WHEREAS, the Company and Executive wish for Executive to be employed by the Company on the terms and conditions set forth in this Agreement as of the Effective Date.

NOW, THEREFORE, in consideration of the mutual promises, terms, covenants and conditions set forth in this Agreement and the performance of each, the Parties, intending to be legally bound, hereby agree as follows:

1. Employment.

(a) *Duties.* The Company hereby employs Executive in the position of Chief Financial Officer. Executive will have such responsibilities, duties and authorities as will be determined from time to time by the Board of Directors of the Company (the "**Board**"), which responsibilities, duties and authorities are consistent with the position of a Chief Financial Officer. Executive will report to the Company's Chief Executive Officer. Executive will work on a full-time basis out of the Company's Cranbury, New Jersey office and remotely.

(b) *Full-time Employment.* Executive hereby accepts this employment upon the terms and conditions contained in this Agreement and agrees to devote substantially all of Executive's business time, attention and efforts to promote and further the business, interests, objectives and affairs of the Company, and Executive will not be engaged in any other business activity pursued for gain, profit or other pecuniary advantage without the prior written consent of the Company. The foregoing limitations will not be construed as prohibiting Executive from serving on civic, charitable or other boards or committees, managing personal or family investments and personal passive investments in securities or from engaging in other activities from time to time, in each case, that will not interfere in any material respect with the performance of Executive's duties under this Agreement. Executive will faithfully adhere to, execute and fulfill in all material respects all policies established by the Company in writing and made available to Executive, consistent with the other terms of this Agreement.

2. Compensation. For all services rendered by Executive in any capacity required under this Agreement, the Company will compensate Executive as follows:

(a) *Base Salary.* During the Tenn, the Company will pay Executive, as compensation for Executive's services, a base salary at a gross annual rate of $485,000.00, less all required tax withholdings and other applicable deductions, in accordance with the Company's standard payroll procedures. The annual compensation specified in this Section 2(a), together with any modifications in such compensation that the Company may make from time to time in accordance with the following sentence, is referred to in this Agreement as the "**Base Salary**." Executive's Base Salary will be subject to review in accordance with the Company's normal performance review practices. Effective as of the date of any change to Executive's Base Salary, the Base Salary as so changed will be considered the new Base Salary for all purposes of this Agreement.

(b) *Benefits and Other Compensation.* Executive will be eligible to receive additional benefits and compensation from the Company as follows:

(i) The Company will allow Executive to participate in all Company-wide employee benefits as may, from time to time, be made available generally to any other executives of the Company, including the Company's defined contribution 401(k) retirement plan.

(ii) Executive will be entitled to such periods of paid time off each year and paid holidays as provided from time to time under the Company's written policies.

(iii) Executive will be eligible to receive reimbursement for business travel and other out-of-pocket expenses reasonably incurred by Executive in the performance of Executive's duties, including without limitation, mobile phone expenses and membership fees associated with related professional associations. All reimbursable expenses will be subject to any pre-approval process established by Company policy and will be appropriately documented in

reasonable detail by Executive upon submission of any request for reimbursement in a format consistent with the Company's expense reporting policy and will be reimbursed promptly.

(c) *Annual Incentive Bonus.* Subject to the subsections set forth herein, Executive will be eligible to receive an annual cash incentive bonus (the "**Incentive Bonus**"). The Compensation Committee of the Board (the "**Compensation Committee**") will establish the applicable performance goals required to be met by the Company for a fiscal year in order for Executive to be eligible for an Incentive Bonus, payable no later than March 15 of the following fiscal year. If the Company achieves the applicable performance goals for any such fiscal year, the Compensation Committee will determine Executive's actual Incentive Bonus amount in the Compensation Committee's sole and absolute discretion based on its evaluation of Executive's performance. Executive's target Incentive Bonus, assuming Executive fully and satisfactorily meets all expectations and obligations as Chief Financial Officer, as determined by the Compensation Committee in its sole and absolute discretion, shall be 45% of Executive's Base Salary.

(i) If Executive's employment with the Company is terminated for any reason whatsoever prior to December 31 of any fiscal year, whether by the Company with or without Cause (as defined below) or by Executive with or without Good Reason (as defined below), Executive will not be entitled to receive an Incentive Bonus payment for such fiscal year.

(ii) If Executive's employment with the Company is terminated by the Company for Cause prior to the date on which any Incentive Bonus for the prior fiscal year to which Executive may be entitled is to be paid, Executive will not be entitled to receive, and shall forfeit all right, interest and entitlement to, any such Incentive Bonus payment for such fiscal year.

(iii) If Executive's employment with the Company is terminated by the Company without Cause, or by Executive with or without Good Reason, after December 31 of any fiscal year but prior to the date on which any Incentive Bonus for the prior fiscal year to which Executive may be entitled is to be paid, Executive shall remain eligible to receive such Incentive Bonus, if any, for such prior fiscal year, subject to the sole and absolute discretion of the Compensation Committee based on its evaluation of Executive's performance.

(iv) "**Cause**" means, as determined by the Board, in its discretion exercised in good faith, Executive's dismissal as a result of: (1) any material breach by Executive of any agreement between Executive and the Company; (2) the conviction of, indictment for or plea of nolo contendere by Executive to a felony or a crime involving moral turpitude; or (3) any material misconduct or willful and deliberate nonperformance (other than by reason of Executive's death or Disability) by Executive of Executive's duties to the Company.

(v) "**Good Reason**" means the occurrence, without Executive's express written consent, which circumstances are not remedied by the Company within thirty (30) days of its receipt of a written notice from Executive describing the applicable circumstances (which notice must be provided by Executive within ninety (90) days of Executive's knowledge of the applicable circumstances), of one or more of the following: (1) any material, adverse change in Executive's duties, responsibilities, authority, title or reporting structure; (2) a material reduction in Executive's base salary or bonus opportunity; or (3) a geographical relocation of Executive's principal office location by more than fifty (50) miles.

(d) *Long-Term Incentive Awards.*

(i) Effective the first Monday of the month following the Effective Date, Executive will be granted two equity awards covering the Company's common stock (the "**Grants**") under the Amended and Restated Rocket Pharmaceuticals, Inc. 2014 Stock Option and Incentive Plan (the "**Stock Plan**"), as may be amended from time to time or any successor plan. The Grants will have an aggregate grant date fair value for accounting purposes of $2,500,000 (the "**Total Value**") and will be apportioned among the two Grants as set in this Section 2(d) using the closing price on the day of the grant. One of the two Grants, representing 50% of the Total Value, will be in the

form of an option to purchase shares of the Company's common stock, and the other of the two Grants, representing 50% of the Total Value, will be in the form of restricted stock units settled in shares of the Company's common stock. The Grants will be subject to the terms and conditions of the Plan and the grant agreements issued by the Company thereunder.

(ii) Executive will be eligible to receive additional annual long-term incentive awards under the Plan in such forms and in such amounts as determined in the sole discretion of the Compensation Committee.

(e) *No Other Compensation or Benefits; Payment.* The compensation and benefits specified in this Section 2 will be in lieu of any and all other compensation and benefits, *provided* that nothing in this Agreement will prevent the Board from increasing the Base Salary or awarding additional incentive compensation to Executive in its sole and absolute discretion. Payment of all compensation and benefits to Executive under this Agreement will be made in accordance with the relevant Company policies in effect from time to time, including normal payroll practices, and will be subject to all applicable withholding and deductions.

(f) *Cessation of Employment.* In the event Executive ceases to be employed by the Company for any reason, Executive's compensation and benefits will cease on the date of such cessation of employment, except as otherwise provided in this Agreement or in any applicable Company employee benefit plan or program.

(g) *Taxes.* Executive will make payment of all required taxes, whether federal, state, provincial, local or foreign in nature, including but not limited to income taxes, Social Security taxes, Federal Unemployment Compensation or any other taxes that are required to be paid by Executive pursuant to any applicable law. The Company will have the right to withhold from the sums payable to Executive under this Agreement such amounts, if any, as may be required by the Internal Revenue Code of 1986, as amended (the "**Code**") or any other like statute that is, or may become, applicable to the provisions of this Agreement.

3. Term and Termination. The term of this Agreement will begin on the Effective Date and continue until terminated in accordance with the provisions of this Agreement (the "**Term**"). This Agreement and Executive's employment under this Agreement may be terminated by the Company at any time, with or without reason or notice. This Agreement and Executive's employment under the Agreement may be terminated by Executive for any reason by providing the Company at least 30 days' advance written notice of such termination. The date of any such termination of this Agreement and Executive's employment hereunder shall be known as the "**Termination Date**." Regardless of the reason for Executive's termination of employment, Executive will, effective as of the Termination Date, be deemed to have resigned from the Board and any positions as an officer of the Company or any of its subsidiaries, as applicable, and will complete any paperwork requested by the Company to document such resignations.

(a) *Termination for Cause or Without Good Reason.* If Executive's employment is terminated by the Company for Cause or by Executive without Good Reason, Executive will be entitled to receive:

(i) Any accrued but unpaid base salary and accrued but unused vacation, which will be paid within one month following the Termination Date in accordance with the Company's customary payroll procedures or such earlier date as may be required by applicable law;

(ii) Reimbursement for unreimbursed business expenses properly incurred by Executive, which will be subject to and paid in accordance with the Company's expense reimbursement policy; and

(iii) Such employee benefits, if any, to which Executive may be entitled under the Company's employee benefit plans as of the Termination Date; *provided* that, in no event will Executive be entitled to any payments in the nature of severance or termination payments except as specifically provided in this Section 3.

The items described under Sections 3(a)(i) through 3(a)(iii) are referred to collectively as the "**Accrued Amounts**."

(b) *Without Cause or for Good Reason.* If Executive's employment with the Company is terminated by the Company without Cause or by Executive for Good Reason, Executive will be entitled to receive the Accrued Amounts. If Executive executes a release of claims in favor of the Company, its affiliates and their respective officers and directors in a form provided by the Company (the "**Release**"), then Executive will receive, subject to such Release becoming irrevocable, in addition to the Accrued Amounts:

(i) A lump sum payment equal to nine months of Executive's base

salary for the year in which the Termination Date occurs, which will be paid within 30 days following Executive's execution and non-revocation of the Release, *provided* that if the effective date of such Release could span two calendar years depending on the date on which Executive signs the Release, the payment will not be made until the later calendar year;

(ii) Any Incentive Bonus to which Executive may be entitled pursuant to Section 2(c)(iii); and

(iii) If Executive timely elects health continuation coverage under the Consolidated Omnibus Budget Reconciliation Act of 1985 ("**COBRA**"), the Company will reimburse Executive for the monthly COBRA premium paid by Executive for Executive's own coverage and coverage for Executive's dependents for a period of nine months following the Termination Date.

(c) *Death or Disability.* Executive's employment under this Agreement will terminate automatically upon Executive's death, and the Company may terminate Executive's employment on account of Executive's Disability. If Executive's is terminated on account of Executive's death or Disability, Executive (or Executive's estate or beneficiaries, as the case may be) will be entitled to receive the following:

(i) The Accrued Amounts; and

(ii) A pro-rata portion of the Incentive Bonus, if any, that Executive would have earned for the calendar year in which the Termination Date occurs, the determination of such Incentive Bonus to remain in the sole and absolute discretion of the Compensation Committee.

(d) *Change in Control Termination.* Notwithstanding any other provision contained herein, if Executive's employment under this Agreement is terminated by Executive for Good Reason or by the Company without Cause (other than on account of Executive's death or Disability), in each case within 12 months following a Change in Control, Executive will be entitled to receive, subject to Executive's execution and non-revocation of a Release:

(i) The Accrued Amounts;

(ii) A lump sum amount equal to Executive's annual salary during the fiscal year in which the termination occurs (not including any reduction of such salary leading to Executive's termination with Good Reason), which will be paid within 30 days following Executive's execution and non-revocation of the Release, *provided* that if the effective date of such Release could span two calendar years depending on the date on which Executive signs the Release, the payment will not be made until the later calendar year;

(iii) A lump sum amount equal to any Incentive Bonus to which Executive would have been entitled during the fiscal year in which the termination occurred, such Incentive Bonus to be determined in the sole and absolute discretion of the Compensation Committee and consistent with any bonus awarded to the Company's Chief Executive Officer in that same year; and

(iv) If Executive timely elects health continuation coverage under COBRA, the Company will reimburse Executive for the monthly COBRA premium paid by Executive for Executive's own coverage and coverage for Executive's dependents for a period of 12 months following the Termination Date.

4. Return of Corporation Property and Termination of Employment. At such time as Executive's employment with the Company is terminated for any reason, Executive will be required to participate in an exit interview for the purpose of assuring a proper termination of Executive's employment and Executive's obligations under this Agreement. On or before the actual date of Executive's termination of employment with the Company, Executive will return to the Company all records, materials and other physical objects relating to Executive's employment with the Company, including, without limitation, all Company credit cards, computers, personal digital assistants and access keys and all materials and things embodying, relating to, containing or derived from any Inventions, Trade Secrets or Confidential Information.

5. No Prior Agreements. Executive hereby represents and warrants to the Company that the execution of this Agreement by Executive and Executive's employment by the Company and the performance of Executive's duties under this Agreement will not violate or be a breach of any agreement with a former employer, client or any other person or entity.

6. Confidentiality. Inventions and Restrictive Covenants. The Company and Executive will enter into the Company's standard Proprietary Information, Inventions and Non-Solicitation/Non-Competition Agreement (the "**Confidentiality and Restrictive Covenant Agreement**") simultaneously with this Agreement.

7. D&O Indemnification. The Company and Executive will enter into the Company's standard D&O Indemnification Agreement (the "**Indemnification Agreement**") simultaneously with this Agreement.

8. Pre-Employment Conditions. For purposes of federal immigration law, Executive will be required, if Executive has not already, to provide to the Company documentary evidence of Executive's identity and eligibility for employment in the United States. Such documentation must be provided to the Company within three business days of the Effective Date, or the Company's employment relationship with Executive may be terminated.

9.Section 409A of the Code.

(a) *General Compliance.* All payments and benefits under this Agreement are intended to comply with, or be exempt from, Section 409A of the Code and all applicable guidance and regulations thereunder, and any ambiguities or ambiguous terms this Agreement will be interpreted and operated to be exempt from or so comply with the requirements of Section 409A of the Code. In no event will the Company reimburse Executive for any taxes, penalties or interest imposed by Section 409A of the Code resulting from any amount paid under the Agreement or otherwise. The Company and Executive will work together in good faith to consider either amendments to this Agreement or revisions to the Agreement with respect to the payment of any benefits to Executive under this Agreement, which are necessary or appropriate to avoid imposition of any additional tax or income recognition prior to the actual payment to Executive under Section 409A of the Code. Notwithstanding anything in the Agreement to the contrary, the Company reserves the right, in its sole discretion and without the consent of Executive, to take such reasonable actions and make any amendments to this Agreement as it deems necessary, advisable or desirable to comply with Section 409A of the Code or to otherwise avoid income recognition under Section 409A of the Code or imposition of any additional tax prior to the actual payment of any benefits under this Agreement.

(b) *Reimbursements.* To the extent any reimbursement of costs and expenses provided for under this Agreement constitutes taxable income to Executive for federal income tax purposes, such reimbursements will be made as soon as practicable after Executive provides proper documentation supporting reimbursement but in no event later than December 31 of the calendar year next following the calendar year in which the expenses to be reimbursed are incurred. With regard to any provision in this Agreement that provides for reimbursement of expenses or in-kind benefits, except as permitted by Section 409A of the Code, (a) the right to reimbursement or in-kind benefits is not subject to liquidation or exchange for another benefit, and (b) the amount of expenses eligible for reimbursement, or in-kind benefits, provided during any taxable year will not affect the expenses eligible for reimbursement, or in-kind benefits to be provided, in any other taxable year.

(c) *Specified Employee.* Notwithstanding any other provision of this Agreement, if any payment or benefit provided to Executive in connection with Executive's termination of employment is determined to constitute "nonqualified deferred compensation" within the meaning of Section 409A of the Code and Executive is determined to be a "specified employee" as defined in Section 409A(a)(2)(b)(i) of the Code, then such payment or benefit will not be paid until the first payroll date to occur following the six-month anniversary of the Termination Date or, if earlier, on Executive's death (the "**Specified Employee Payment Date**"). The aggregate of any payments that would otherwise have been paid before the Specified Employee Payment Date will be paid to Executive in a lump sum on the Specified Employee Payment Date and thereafter, any remaining payments will be paid without delay in accordance with their original schedule.

10.Section 2800 of the Code. If any of the payments or benefits received or to be received by Executive (including, without limitation, any payment or benefits received in connection with a Change in Control or Executive's termination of employment, whether pursuant to the terms of this Agreement or any other plan, arrangement or agreement, or otherwise) (all such payments collectively referred to as the "**280G Payments**") constitute "parachute payments" within the meaning of Section 280G of the Code and would, but for this Section 12, be subject to the excise tax imposed under Section 4999 of the Code (the "**Excise Tax**"), then prior to making the 280G Payments, a calculation

will be made comparing (i) the Net Benefit (as defined below) to Executive of the 280G Payments after payment of the Excise Tax to (ii) the Net Benefit to Executive if the 280G Payments are limited to the extent necessary to avoid being subject to the Excise Tax. Only if the amount calculated under (i) above is less than the amount under (ii) above will the 280G Payments be reduced to the minimum extent necessary to ensure that no portion of the 280G Payments is subject to the Excise Tax. For purposes of this Agreement, "**Net Benefit**"

means the present value of the 280G Payments net of all federal, state, local or foreign income, employment and excise taxes. Any reduction made pursuant to this Section 10 will be made in a manner determined by the Company that is consistent with the requirements of Section 409A of the Code. All calculations and determinations under this Section 10 will be made by an independent accounting firm or independent tax counsel appointed by the Company ("**Tax Counsel**") whose determinations will be conclusive and binding on the Company and Executive for all purposes. For purposes of making the calculations and determinations required by this Section 10. Tax Counsel may rely on reasonable, good faith assumptions and approximations concerning the application of Sections 280G and 4999 of the Code. The Company and Executive will furnish the Tax Counsel with such information and documents as Tax Counsel may reasonably request to make its determinations under this Section 10, and the costs of such determination will be borne by the Company.

11. Cooperation with Litigation. During and following Executive's Termination of Employment with the Company (regardless of the reason for Executive's Termination of Employment with the Company and which Party initiates the Termination of Employment with the Company), Executive agrees to cooperate with and be readily available to the Company, the Company's General Counsel (or equivalent position within the Company) or the Company's advisers, as the Company may reasonably request, to assist it in any matter regarding the Company and its subsidiaries, including giving truthful testimony in any litigation, potential litigation or any internal investigation or administrative, regulatory, judicial or quasi-judicial proceedings involving the Company or its subsidiaries, with respect to which Executive has knowledge, experience or information. Executive acknowledges that this could involve, but is not limited to, responding to or defending any regulatory or legal process, providing information in relation to any such process, preparing witness statements and giving evidence in person on behalf of the Company. The Company will work with Executive to ensure that such cooperation does not unduly burden Executive, work in good faith to accommodate Executive's other commitments and reimburse any reasonable expenses incurred by Executive, including reasonable attorneys' fees and costs, as a consequence of complying with Executive's obligations under this Section 10, *provided* that such expenses are approved in advance by the Company.

12. Notice. All notices, requests, permissions, waivers and other communications under this Agreement will be in writing and will be deemed to have been duly given (a) five business days following sending by registered or certified mail, postage prepaid, (b) when sent, if sent by e- mail during normal business hours and received at the recipient's location during normal business hours, and otherwise on the next business day, (c) when delivered, if delivered personally to the intended recipient and (d) one business day following sending by overnight delivery via a national courier service and, in each case, addressed to a Party at the following address for such Party:

To the Company:

Rocket Pharmaceuticals, Inc.
9 Cedarbrook Drive
Cranbury, NJ 08512
Attn: General Counsel
Telephone Number: ****
E-Mail: ****

To Executive:

Aaron Ondrey at the contact information on file with the Company

Either Party may, by notice given in accordance with this Section 12, specify a new address for notices under this Agreement.

13. Binding Effect and Assignment. This Agreement will be binding on, inure to the benefit of and be enforceable by the Parties and their respective heirs, legal representatives, successors and permitted assigns. Executive understands that Executive has been selected for employment by the Company on the basis of Executive's personal qualifications, experience and skills. Executive agrees, therefore, that Executive cannot assign all or any portion of Executive's performance under this

Agreement.

14. Entire Agreement. This Agreement, the Confidentiality and Restrictive Covenant Agreement, the Indemnification Agreement and any exhibits attached thereto constitute the entire

agreement and understanding between the Parties with respect to the subject matter of such agreements, and supersede all other understandings and negotiations with respect thereto.

15. Severability and Headings. It is the intention of the Parties that the provisions in this Agreement will be enforceable to the fullest extent permitted under applicable law, and that the unenforceability of any provisions of this Agreement, or any portion thereof, will not render unenforceable or otherwise impair any other provisions or portions thereof. Each term, condition, covenant or provision of this Agreement will be viewed as separate and distinct, and m the event that any such term, covenant or provision is held by a court of competent jurisdiction to be invalid, the remaining provisions will continue in full force and effect. The section headings in this Agreement are for reference purposes only and are not intended in any way to describe, interpret, define or limit the extent or intent of this Agreement or of any part of this Agreement.

16. No Third-Party Beneficiaries. Except as otherwise provided in this Agreement, this Agreement is for the sole benefit of the Parties (and their respective heirs, legal representatives, successors and permitted assigns), and nothing expressed or implied in this Agreement will give or be construed to give to any person, other than the Parties (and their respective heirs, legal representatives, successors and permitted assigns), any legal or equitable rights under this Agreement.

17. Governing Law. This Agreement will be governed by and construed in accordance with the laws of the State of New Jersey, without giving effect to the principles of conflicts of law thereof that would result in the application of the law of any other jurisdiction.

18. Dispute Resolution. Any and all controversies, disputes or claims arising out of, or relating to, this Agreement and its negotiation, execution, performance, non-performance, interpretation, termination, construction or the transactions contemplated hereby will be heard and determined in the courts of the State of New Jersey and the United States District Court for the District of New Jersey. The Parties hereby irrevocably submit to the exclusive jurisdiction and venue of such courts in any such proceeding and irrevocably and unconditionally waive the defense of an inconvenient forum, or lack of jurisdiction to the maintenance of any such proceeding. The consents to jurisdiction and venue set forth in this Agreement will not constitute general consents to service of process in the State of New Jersey and will have no effect for any purpose except as provided in this Section 18 and will not be deemed to confer rights on any Person other than the Parties. Each Party agrees that the service of process on such Party in any proceeding arising out of or relating to this Agreement will be effective if notice is given by overnight courier at the address set forth in the books and records of the Company. Each of the Parties also agrees that any judgment against a Party in connection with any proceeding arising out of or relating to this Agreement may be enforced in any court of competent jurisdiction, either within or outside of the United States. A certified or exemplified copy of such judgment will be conclusive evidence of the fact and amount of such judgment.

19. Counterparts. This Agreement may be executed in any number of counterparts (which may be delivered by facsimile or in PDF format), each of which will be deemed an original, but all of which together will constitute one and the same instrument.

20. Amendments and Waivers. No amendment or modification of the terms or conditions of this Agreement will be valid unless in writing and signed by the Parties. A waiver by either Party of a breach of any provision of this Agreement will not constitute a general waiver, or prejudice the other Party's right otherwise to demand strict compliance with that provision.

21. Certain Acknowledgements. EXECUTIVE ACKNOWLEDGES THAT, BEFORE SIGNING THIS AGREEMENT, EXECUTIVE WAS GIVEN AN OPPORTUNITY TO READ IT CAREFULLY EVALUATE IT AND ASK ANY QUESTIONS EXECUTIVE MAY HAVE HAD REGARDING IT OR ITS PROVISIONS. EXECUTIVE ALSO ACKNOWLEDGES THAT EXECUTIVE HAD THE RIGHT TO HAVE THIS AGREEMENT REVIEWED BY AN ATTORNEY OF EXECUTIVE'S CHOOSING AND THAT THE COMPANY GAVE EXECUTIVE A REASONABLE PERIOD OF TIME TO DO SO IF EXECUTIVE SO WISHED. EXECUTIVE FURTHER ACKNOWLEDGES

THAT EXECUTIVE IS NOT BOUND BY ANY AGREEMENT THAT WOULD PREVENT EXECUTIVE FROM PERFORMING EXECUTIVE'S DUTIES AS SET FORTH IN THIS AGREEMENT, NOR DOES EXECUTIVE KNOW OF ANY OTHER REASON WHY EXECUTIVE WOULD NOT BE ABLE TO PERFORM EXECUTIVE'S DUTIES AS SET FORTH IN THIS AGREEMENT.

[Signature Page Follows]

IN WITNESS WHEREOF, the Parties have executed this Executive Employment Agreement as of the day and year first above written.

Company:

ROCKET PHARMACEUTICALS, INC.

By:
Name:
Title:

Executive:

Aaron Ondrey

[SIGNATURE PAGE TO EXECUTIVE EMPLOYMENT AGREEMENT]

Exhibit 31.1

CERTIFICATIONS

I, Gaurav Shah, MD, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended March 31, 2024 of Rocket Pharmaceuticals, Inc.;

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

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

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

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

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

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

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

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

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

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

Date: May 7, 2024

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

Exhibit 31.2

CERTIFICATIONS

I, Aaron Ondrey, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended March 31, 2024 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 and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:

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

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

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

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

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

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

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

Date: May 7, 2024

By: /s/ Aaron Ondrey
Aaron Ondrey
Chief Financial Officer
(Principal Financial Officer)

Exhibit 32.1

CERTIFICATION PURSUANT TO

18 U.S.C. SECTION 1350,
AS ADOPTED PURSUANT TO

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the quarterly report on Form 10-Q of Rocket Pharmaceuticals, Inc. (the "Company") for the period ended March 31, 2024, as filed with the United States Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers hereby certifies, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, 18 U.S.C. Section 1350, that to his knowledge:

1) the Report which this statement accompanies fully complies with the requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended; and
2) the information contained in the Report fairly presents, in all material respects, the financial condition and results of operations of the Company.

Date: May 7, 2024

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

Date: May 7, 2024

By: /s/ Aaron Ondrey
Aaron Ondrey
Chief Financial Officer
(Principal Financial Officer)