**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, 2023**

**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, 2023, there were 80,461,335 shares of common stock, $0.01 par value per share, outstanding.

## Cautionary Statement Regarding Forward-Looking Statements

This Quarterly Report on Form 10-Q for the quarter ended March 31, 2023 contains forward-looking statements that involve risks and uncertainties, as well as assumptions that, if they never materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q are forward-looking statements. In some cases, you can identify forward-looking statements by words such as "aim," "anticipate," "believe," "can," "contemplate," "continue," "could," "design," "estimate," "expect," "future," "intend," "likely," "may," "plan," "potential," "predict," "project," "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:

- federal, state, and non-U.S. regulatory requirements, including regulation of our current or any other future product candidates by the U.S. Food and Drug Administration ("FDA");
- the timing of and our ability to submit regulatory filings with the FDA and to obtain and maintain FDA or other regulatory authority approval of, or other action with respect to, our product candidates;
- our competitors' activities, including decisions as to the timing of competing product launches, pricing, and discounting;
- whether safety and efficacy results of our clinical trials and other required tests for approval of our product candidates provide data to warrant progression of clinical trials, potential regulatory approval, or further development of any of our product candidates;
- our ability to develop, acquire and advance product candidates into, enroll a sufficient number of patients into, and successfully complete, clinical studies, and our ability to apply for and obtain regulatory approval for such product candidates, within currently anticipated timeframes, or at all;
- our ability to establish key collaborations and vendor relationships for our product candidates and any other future product candidates;
- our ability to 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 obtain additional funding to conduct our planned research and development efforts;
- 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 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, 2022, on Form 10-K, that could cause actual results or events to differ materially from the forward-looking statements that we make. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures, or investments we may make or enter into.

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

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

## PART I — FINANCIAL INFORMATION

**Item 1. Financial Statements**

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

| | March 31, 2023 (unaudited) | December 31, 2022 |
|---|---|---|
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 64,579 | $ 140,517 |
| Investments | 266,505 | 215,877 |
| Prepaid expenses and other current assets | 6,949 | 7,666 |
| Total current assets | 338,033 | 364,060 |
| Property and equipment, net | 30,588 | 29,009 |
| Goodwill | 39,154 | 39,154 |
| Intangible assets | 25,724 | 25,724 |
| Restricted cash | 1,340 | 1,340 |
| Deposits | 459 | 608 |
| Investments | 28,957 | 43,276 |
| Operating lease right-of-use assets | 4,369 | 1,972 |
| Finance lease right-of-use asset | 46,133 | 46,664 |
| Total assets | $ 514,757 | $ 551,807 |
| **Liabilities and stockholders' equity** | | |
| Current liabilities: | | |
| Accounts payable and accrued expenses | $ 28,609 | $ 36,660 |
| Operating lease liabilities, current | 849 | 773 |
| Finance lease liability, current | 1,748 | 1,736 |
| Total current liabilities | 31,206 | 39,169 |
| Operating lease liabilities, non-current | 3,506 | 1,088 |
| Finance lease liability, non-current | 19,294 | 19,269 |
| Other liabilities | 1,875 | 2,595 |
| Total liabilities | 55,881 | 62,121 |
| Commitments and contingencies (Note 12) | | |
| | | |
| Stockholders' equity: | | |
| Preferred stock, $0.01 par value, authorized 5,000,000 shares: | | |
| Series A convertible preferred stock; 300,000 shares designated as Series A; 0 shares issued and outstanding | - | - |
| Series B convertible preferred stock; 300,000 shares designated as Series B; 0 shares issued and outstanding | - | - |
| Common stock, $0.01 par value, 120,000,000 shares authorized; 80,412,194 and 79,123,312 shares issued and 80,409,623 and 79,120,741 shares outstanding at March 31, 2023 and December 31, 2022, respectively | 804 | 791 |
| Treasury stock, at cost, 2,571 common shares at March 31, 2023 and December 31, 2022, respectively | (47) | (47) |
| Additional paid-in capital | 1,230,319 | 1,203,074 |
| Accumulated other comprehensive loss | (90) | (357) |
| Accumulated deficit | (772,110) | (713,775) |
| Total stockholders' equity | 458,876 | 489,686 |
| Total liabilities and stockholders' equity | $ 514,757 | $ 551,807 |

*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, | |
|---|---|---|
| | **2023** | **2022** |
| Revenue | $ - | $ - |
| Operating expenses: | | |
| Research and development | 46,371 | 30,794 |
| General and administrative | 15,823 | 11,770 |
| Total operating expenses | 62,194 | 42,564 |
| Loss from operations | (62,194) | (42,564) |
| Interest expense | (468) | (464) |
| Interest and other income, net | 1,908 | 623 |
| Accretion of discount and amortization of premium on investments, net | 2,419 | (577) |
| Net loss | $ (58,335) | $ (42,982) |
| Net loss per share - basic and diluted | $ (0.73) | $ (0.67) |
| Weighted-average common shares outstanding - basic and diluted | 79,453,519 | 64,509,721 |

*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, | |
|---|---|---|
| | 2023 | 2022 |
| Net loss | $ (58,335) | $ (42,982) |
| Other comprehensive loss | | |
| Net unrealized gain (loss) on investments | 272 | (468) |
| Total comprehensive loss | $ (58,063) | $ (43,450) |

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

**Rocket Pharmaceuticals, Inc.**
**Consolidated Statements of Stockholders' Equity**
**For the Three Months Ended March 31, 2023 and 2022**
**(in thousands except share amounts)**
**(unaudited)**

| | Common Stock | | Treasury | Additional Paid-In | Accumulated Other Comprehensive | Accumulated | Total Stockholders' |
|---|---|---|---|---|---|---|---|
| | Shares | Amount | Stock | Capital | Income/(Loss) | Deficit | Equity |
| **Balance at December 31, 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 |

| | Common Stock | | Treasury | Additional Paid-In | Accumulated Other Comprehensive | Accumulated | Total Stockholders' |
|---|---|---|---|---|---|---|---|
| | Shares | Amount | Stock | Capital | (Loss) | Deficit | Equity |
| **Balance at December 31, 2021** | 64,505,889 | $ 645 | $ - | $ 946,152 | $ (161) | $ (491,912) | $ 454,724 |
| Issuance of common stock pursuant to exercise of stock options | 16,168 | - | - | 76 | - | - | 76 |
| Unrealized comprehensive loss on investments | - | - | - | - | (468) | - | (468) |
| Stock-based compensation | - | - | - | 6,270 | - | - | 6,270 |
| Net loss | - | - | - | - | - | (42,982) | (42,982) |
| **Balance at March 31, 2022** | 64,522,057 | $ 645 | $ - | $ 952,498 | $ (629) | $ (534,894) | $ 417,620 |

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

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

| | Three Months Ended March 31, | |
|---|---|---|
| | **2023** | **2022** |
| **Operating activities:** | | |
| Net loss | $ (58,335) | $ (42,982) |
| Adjustments to reconcile net loss to net cash used in operating activities: | | |
| Depreciation and amortization of property and equipment | 1,135 | 767 |
| Amortization of finance lease right of use asset | 538 | 535 |
| Write down of property and equipment, net | - | 40 |
| Stock-based compensation | 8,915 | 6,270 |
| Amortization of premium and accretion of discount on investments, net | (2,343) | 577 |
| **Changes in operating assets and liabilities:** | | |
| Prepaid expenses and other assets | 866 | (3,936) |
| Accounts payable and accrued expenses | (7,750) | (491) |
| Operating lease liabilities | 97 | (33) |
| Finance lease liability | 37 | 45 |
| Other liabilities | (720) | (15) |
| Net cash used in operating activities | (57,560) | (39,223) |
| **Investing activities:** | | |
| Purchases of investments | (96,034) | (143,023) |
| Proceeds from maturities of investments | 62,335 | 81,983 |
| Payments made to acquire right of use asset | (7) | - |
| Purchases of property and equipment | (3,015) | (1,955) |
| Net cash used in investing activities | (36,721) | (62,995) |
| **Financing activities:** | | |
| Issuance of common stock, pursuant to exercise of stock options | 1,114 | 76 |
| Exercise of warrants | 7 | - |
| Issuance of common stock pursuant to the at-the-market offering program, net of issuance costs | 17,222 | - |
| Net cash provided by financing activities | 18,343 | 76 |
| Net change in cash, cash equivalents and restricted cash | (75,938) | (102,142) |
| Cash, cash equivalents and restricted cash at beginning of period | 141,857 | 234,037 |
| Cash, cash equivalents and restricted cash at end of period | $ 65,919 | $ 131,895 |
| | | |
| **Supplemental disclosure of non-cash financing and investing activities:** | | |
| Accrued purchases of property and equipment, ending balance | $ 1,794 | $ 1,635 |
| Unrealized gain (loss) on investments | $ 267 | $ (468) |

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

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

## 1. Nature of Business

Rocket Pharmaceuticals, Inc. ("Rocket" or the "Company") is a clinical-stage, multi-platform biotechnology company focused on the development of 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 ("LV") programs. These include programs for Fanconi Anemia ("FA"), a genetic defect in the bone marrow that reduces production of blood cells or promotes the production of faulty blood cells, Leukocyte Adhesion Deficiency-I ("LAD-I"), a genetic disorder that causes the immune system to malfunction and Pyruvate Kinase Deficiency ("PKD"), a rare red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia. Of these, both the Phase 2 FA program and the Phase 1/2 LAD-I program produced data read outs in 2022 and regulatory filings in the United States ("U.S.") and Europe ("EU") are anticipated in 2023. 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 a clinical stage in vivo adeno-associated virus ("AAV") program for Danon disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. The Danon program is currently in an ongoing Phase 1 trial and pivotal Phase 2 study initiation expected in the second quarter of 2023. Additionally, the Company has an AAV vector program targeting Plakophilin-2 Arrhythmogenic Cardiomyopathy ("PKP2-ACM"), 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, also referred to as Pegasus, will be approaching IND submission in the second quarter of 2023. As a result of the Company's acquisition of Renovacor Inc. ("Renovacor") (see Note 14 "Renovacor Acquisition"), the Company is able to utilize recombinant AAV9-based gene therapy designed to slow or halt progression of BAG3 Dilated Cardiomyopathy ("DCM"), 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 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 $772.1 million as of March 31, 2023. As of March 31, 2023, the Company had $360.0 million of cash, cash equivalents and short-term and long-term investments. The Company expects such resources will be sufficient to fund the Company's operating expenses and capital expenditure requirements into the first half of 2025.

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. Through March 31, 2023, 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"), including 0.9 million shares for net proceeds of $17.2 million during the three months ended March 31, 2023.

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, 2022 included in the Annual Report on Form 10-K filed with the Securities and Exchange Commission ("SEC") on February 28, 2023 ("2022 Form 10-K"). The unaudited interim consolidated financial statements have been prepared on the same basis as the audited annual financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for the fair statement of the Company's consolidated financial position as of March 31, 2023 and the results of its operations and its cash flows for the three months ended March 31, 2023. The financial data and other information disclosed in these consolidated notes related to the three months ended March 31, 2023 and 2022 are unaudited. The results for the three months ended March 31, 2023 are not necessarily indicative of results to be expected for the year ending December 31, 2023 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, 2023 are consistent with those disclosed in Note 3 to the consolidated financial statements in the 2022 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 accounting principles generally accepted in the United States ("U.S. GAAP"). All intercompany accounts have been eliminated in consolidation.

***Use of Estimates***

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

***Cash, Cash Equivalents and Restricted Cash***

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

Restricted cash consists of deposits collateralizing letters of credit issued by a bank in connection with the Company's operating leases (see Note 12 "Commitments and Contingencies" for additional disclosures) and a deposit collateralizing a letter of credit issued by a bank supporting the Company's corporate credit card. Cash, cash equivalents and restricted cash consist of the following:

| | March 31, 2023 | December 31, 2022 |
|---|---|---|
| Cash and cash equivalents | $ 64,579 | $ 140,517 |
| Restricted cash | 1,340 | 1,340 |
| | $ 65,919 | $ 141,857 |

***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, Commercial Paper and Corporate and Agency 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+/Aa1 rated, thereby reducing credit risk exposure.

***Investments***

Investments consist of investments in U.S. Treasury Securities, Commercial Paper and Corporate and Agency 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 Financial Accounting Standards Board ("FASB") Accounting Standard Codification ("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. For the three months ended March 31, 2023, there were net unrealized gains on investments of $0.3 million. For the three months ended March 31, 2022, there were net unrealized losses on investments of $0.5 million.

***Intangible Assets***

Intangible assets related to in process research and development ("IPR&D") 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 drop 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.

***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 ASC Topic 480, Distinguishing Liabilities from Equity ("ASC 480") and/or ASC Topic 815, Derivatives and Hedging ("ASC 815"), 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 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 is recognized over the requisite service period of the awards on a straight-line basis with forfeitures recognized as they occur.

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.

***Income Taxes***

In May 2022, the Company received a notice from the New York City Department of Finance regarding an audit of the NYC Biotechnology Credit for the tax periods ended December 31, 2018 through December 31, 2020, which is ongoing as of March 31, 2023.

***Recent Accounting Pronouncements***

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

**4. Fair Value of Financial Instruments**

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

| | Fair Value Measurements as of March 31, 2023 Using: | | | |
|---|---|---|---|---|
| | **Level 1** | **Level 2** | **Level 3** | **Total** |
| Assets: | | | | |
| Cash equivalents: | | | | |
| Money market mutual funds | $ 31,353 | $ - | $ - | $ 31,353 |
| Corporate Bonds | - | 3,778 | - | 3,778 |
| United States Treasury securities | 7,670 | - | - | 7,670 |
| | 39,023 | 3,778 | - | 42,801 |
| Investments: | | | | |
| Commercial Paper | - | 5,147 | - | 5,147 |
| United States Treasury securities | 228,443 | - | - | 228,443 |
| Corporate Bonds | - | 54,159 | - | 54,159 |
| Agency Bonds | - | 7,713 | - | 7,713 |
| | 228,443 | 67,019 | - | 295,462 |
| Total assets | $ 267,466 | $ 70,797 | $ - | $ 338,263 |
| Liabilities: | | | | |
| Warrant liability | $ - | $ - | $ 815 | $ 815 |
| Total liabilities | $ - | $ - | $ 815 | $ 815 |

| | Fair Value Measurements as of December 31, 2022 Using: | | | |
|---|---|---|---|---|
| | **Level 1** | **Level 2** | **Level 3** | **Total** |
| Assets: | | | | |
| Cash equivalents: | | | | |
| Money market mutual funds | $ 90,527 | $ - | $ - | $ 90,527 |
| Commercial Paper | - | 3,899 | - | 3,899 |
| United States Treasury Securities | 3,848 | - | - | 3,848 |
| Corporate Bonds | - | 8,618 | - | 8,618 |
| | 94,375 | 12,517 | - | 106,892 |
| Investments: | | | | |
| Commercial Paper | - | 1,151 | - | 1,151 |
| United States Treasury securities | 189,444 | - | - | 189,444 |
| Corporate Bonds | - | 60,905 | - | 60,905 |
| Agency Bonds | - | 7,653 | - | 7,653 |
| | 189,444 | 69,709 | - | 259,153 |
| Total assets | $ 283,819 | $ 82,226 | $ - | $ 366,045 |
| Liabilities: | | | | |
| Warrant liability | $ - | $ - | $ 1,512 | $ 1,512 |
| Total liabilities | $ - | $ - | $ 1,512 | $ 1,512 |

The Company classifies its money market mutual funds and U.S. Treasury securities as Level 1 assets under the fair value hierarchy, as these assets have been valued using quoted market prices in active markets without any valuation adjustment. The Company classifies its Commercial Paper and Corporate and Agency Bonds as Level 2 assets as these assets are not traded in an active market and have been valued through a third-party pricing service based on quoted prices for similar assets.

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, 2022 | $ 1,512 |
| Fair value adjustments | (697) |
| Balance, March 31, 2023 | $ 815 |

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 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, 2023 | December 31, 2022 |
|---|---|---|
| Stock price | $ 17.13 | $ 18.39 |
| Exercise price | $ 65.23 | $ 65.23 |
| Expected volatility | 67.38% | 71.25% |
| Risk-free interest rate | 4.04% | 4.14% |
| Expected dividend yield | - | - |
| Expected life (years) | 2.07 | 2.39 |
| Fair value per warrant | $ 1.32 | $ 2.45 |

## 5. Property and Equipment, Net

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

| | March 31, 2023 | December 31, 2022 |
|---|---|---|
| Laboratory equipment | $ 23,317 | $ 21,905 |
| Machinery and equipment | 11,443 | 11,326 |
| Computer equipment | 244 | 244 |
| Furniture and fixtures | 2,216 | 2,135 |
| Leasehold improvements | 1,694 | 589 |
| Internal use software | 1,903 | 1,903 |
| | 40,817 | 38,102 |
| Less: accumulated depreciation and amortization | (10,229) | (9,093) |
| | $ 30,588 | $ 29,009 |

During the three months ended March 31, 2023 and 2022, the Company recognized $1.1 million and $0.8 million of depreciation and amortization expense, respectively.

## 6. Intangible Assets and Goodwill

The Company's indefinite lived intangible assets consists of acquired IPR&D asset and a mice colony model received from the acquisition of Renovacor.

Intangible assets as of March 31, 2023 and December 31, 2022 are summarized as follows:

| | Gross Carrying Value | Accumulated Amortization | Intangible Assets, Net |
|---|---|---|---|
| In process research & development | $ 25,150 | $ - | $ 25,150 |
| Mice colony model | 574 | - | 574 |
| Total intangible assets | $ 25,724 | $ - | $ 25,724 |

The gross carrying value of intangible assets was due to the acquisition of Renovacor (see Note 14 "Renovacor Acquisition").

The carrying value of Goodwill was $39.2 million as of March 31, 2023 and included $8.3 million as a result of the acquisition of Renovacor (see Note 14 "Renovacor Acquisition").

## 7. Accounts Payable and Accrued Expenses

As of March 31, 2023 and December 31, 2022, the Company's accounts payable and accrued expenses consisted of the following:

| | March 31, 2023 | December 31, 2022 |
|---|---|---|
| Research and development | $ 17,536 | $ 19,100 |
| Employee compensation | 4,199 | 10,006 |
| Property and equipment | 1,794 | 2,095 |
| Professional fees | 2,813 | 1,436 |
| Acquisition related expenses | - | 1,153 |
| Government grant payable | 597 | 597 |
| Other | 1,670 | 2,273 |
| | $ 28,609 | $ 36,660 |

## 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 reimbursed Cowen for certain expenses incurred in connection with the Sales Agreement. Through March 31, 2023, 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. During the three months ended March 31, 2023, the Company sold 0.9 million shares under the at-the-market offering program for gross proceeds of $17.8 million, less commission of $0.6 million for net proceeds of $17.2 million.

## 9. Stock Based Compensation

***Stock Option Valuation***

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

| | Three Months Ended March 31, | |
|---|---|---|
| | 2023 | 2022 |
| Risk-free interest rate | 4.02% | 1.88% |
| Expected term (in years) | 5.88 | 5.86 |
| Expected volatility | 73.54% | 74.07% |
| Expected dividend yield | 0.00% | 0.00% |
| Exercise price | $ 20.17 | $ 17.85 |
| Fair value of common stock | $ 20.17 | $ 17.85 |

The following table summarizes stock option activity for the three months ended March 31, 2023, under the Second Amended and Restated 2014 Stock Option and Incentive Plan:

| | Number of Shares | Weighted Average Exercise Price | Weighted Average Contractual Term (Years) | Aggregate Intrinsic Value |
|---|---|---|---|---|
| Outstanding as of December 31, 2022 | 13,138,870 | $ 14.52 | 5.46 | $ 118,767 |
| Granted | 1,792,097 | 20.36 | 6.86 | |
| Exercised | (88,429) | 12.59 | | 631 |
| Cancelled | (191,148) | 33.99 | | |
| Outstanding as of March 31, 2023 | 14,651,390 | $ 14.99 | 5.85 | $ 97,263 |
| | | | | |
| Options vested and exercisable as of March 31, 2023 | 10,586,141 | $ 12.60 | 4.52 | $ 92,808 |
| Options unvested as of March 31, 2023 | 4,065,249 | $ 21.24 | 9.32 | $ 4,455 |

The weighted average grant-date fair value per share of stock options granted during the three months ended March 31, 2023, and 2022 was $13.50 and $11.60, respectively.

The total fair value of options vested during the three months ended March 31, 2023 and 2022 was $11.4 million and $12.5 million, respectively.

***Restricted Stock Units ("RSU")***

The following table summarizes the Company's RSU activity for the three months ended March 31, 2023:

| | Number of Shares | Weighted Average Grant Date Fair Value |
|---|---|---|
| Unvested as of December 31, 2022 | 992,874 | $ 16.49 |
| Granted | 764,204 | 20.23 |
| Vested[1] | (126,145) | 17.37 |
| Forfeited | (8,476) | 17.19 |
| Unvested as of March 31, 2023 | 1,622,457 | $ 18.18 |

(1) Common stock issued is net of 85 shares related to taxes.

***Stock-based Compensation***

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

| | Three Months Ended March 31, 2023 | 2022 |
|---|---|---|
| Stock options | $ 6,985 | $ 5,961 |
| Restricted stock units | 1,930 | 309 |
| Total stock-based compensation expense | $ 8,915 | $ 6,270 |

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

| | Three Months Ended March 31, 2023 | 2022 |
|---|---|---|
| Research and development | $ 3,819 | $ 2,318 |
| General and administrative | 5,096 | 3,952 |
| Total stock-based compensation expense | $ 8,915 | $ 6,270 |

As of March 31, 2023, the Company had an aggregate of $75.4 million of unrecognized stock-based compensation expense related to both stock options and RSU grants, which is expected to be recognized over the weighted average period of 1.52 years.

**10. Warrants**

A summary of the warrants outstanding as of March 31, 2023 is as follows:

| Exercise Price | Outstanding | Grant/Assumption Date | Expiration Date |
|---|---|---|---|
| 24.42 | 7,051 | June 28, 2013 | June 28, 2023 |
| 57.11 | 603,386 | December 21, 2020 | December 21, 2030 |
| 33.63 | 301,291 | August 9, 2021 | August 9, 2031 |
| 22.51 | 153,155 | December 17, 2021 | December 17, 2031 |
| 22.51 | 153,155 | December 17, 2021 | December 17, 2031 |
| 65.23 | 617,050 | December 1, 2022 | April 23, 2025 |
| 65.23 | 760,086 | December 1, 2022 | December 1, 2026 |
| Total | 2,595,174 | | |

The following table below is a summary of changes in warrants to purchase common stock for the three months ended March 31, 2023:

| | Number of Warrant Shares Outstanding and Exercisable | Exercise Price per Share |
|---|---|---|
| Balance as of December 31, 2022 | 2,721,267 | |
| Granted | - | |
| Exercised | (126,093) | $ 0.06 |
| Balance as of March 31, 2023 | 2,595,174 | |

**Assumed Renovacor Public Warrants**

In conjunction with the Renovacor acquisition (see Note 14 “Renovacor Acquisition”), 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 Merger, 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 Renovacor acquisition (see Note 14 "Renovacor Acquisition"), 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 for discussion of fair value measurement of the warrant liabilities.

**Assumed Renovacor Pre-Funded Warrants**

In conjunction with the Renovacor acquisition (see Note 14 "Renovacor Acquisition"), Rocket assumed pre-funded warrants ("Pre-Funded Warrants") that were converted into Rocket warrants with a right to purchase 126,093 of Rocket common shares at an exercise price of $0.06 per share. These warrants were exercised in January 2023.

## 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, | |
|---|---|---|
| | 2023 | 2022 |
| Numerator: | | |
| Net loss attributable to common stockholders | $ (58,335) | $ (42,982) |
| Denominator: | | |
| Weighted-average common shares outstanding - basic and diluted | 79,453,519 | 64,509,721 |
| Net loss per share attributable to common stockholders - basic and diluted | $ (0.73) | $ (0.67) |

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, | |
|---|---|---|
| | 2023 | 2022 |
| Warrants exercisable for common shares | 2,595,174 | 1,218,038 |
| Restricted stock units convertible for common shares | 1,622,457 | 457,709 |
| Options to purchase common shares | 14,651,390 | 12,047,299 |
| | 18,869,021 | 13,723,046 |

## 12. Commitments and Contingencies

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

***Finance Lease***

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

Estimated rent payments for the NJ Lease Agreement are $1.2 million per annum, payable in monthly installments, depending upon the nature of the leased space, and subject to annual base rent increases of 3%. The total commitment under the lease is estimated to be approximately $29.3 million over the 15-year term of the lease. The Company paid a cash security deposit of $0.3 million to the landlord in connection with the NJ Lease Agreement which has been reflected in deposits in the consolidated balance sheets as of March 31, 2023 and December 31, 2022.

***Operating Leases***

On June 7, 2018, the Company entered into a three-year lease agreement for office space in the Empire State Building in New York, NY (the "ESB Lease Agreement"). In connection with the ESB Lease Agreement, the Company established an irrevocable standby letter of credit (the "Empire LOC") for $0.9 million. On March 26, 2021, the Company entered in Amendment No. 1 to the ESB Lease Agreement ("ESB Lease Amendment") that extended the term of the lease agreement to June 30, 2024, reduced the rent payments going forward, and reduced the Empire LOC to $0.8 million. The Empire LOC serves as the Company's security deposit on the lease in which the landlord is the beneficiary and expires August 29, 2024. The Company has 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, 2023 and December 31, 2022.

On January 4, 2018, in connection with the Reverse Merger with Inotek, the Company assumed an operating lease for Inotek's former headquarters in Lexington, Massachusetts, with a term which ended on February 28, 2023. In July 2018, the Company signed an agreement to sublease a portion of the Lexington, Massachusetts space and in September 2018, the Company signed an agreement to sublease the remaining portion of the Lexington, Massachusetts space. Rental income received under the sublease agreement totaled $0.1 million for the three months ended March 31, 2023 and 2022. These amounts are netted against rent expense in the consolidated statements of operations for the three months ended March 31, 2023 and 2022. A security deposit of $0.2 million was returned to the Company in April 2023 and is reflected in other current assets as of March 31, 2023.

On December 1, 2022, in connection with the Renovacor acquisition (see Note 14 "Renovacor Acquisition"), Rocket acquired the Renovacor operating leases for space at facilities in Hopewell, New Jersey and Cambridge, Massachusetts with remaining lease terms of approximately 10.25 and 1.3 years, respectively. As of March 31, 2023, lease commencement dates have occurred for all leases and the Company recognized total right-of-use assets of $3.8 million with corresponding total lease liabilities of $3.6 million. The Company intends to sublease both premises through the remainder of their lease terms.

Rent expense was $0.4 million and $0.3 million for the three months ended March 31, 2023 and 2022, respectively.

The total restricted cash balance for the Company's operating and finance leases as of each of March 31, 2023 and December 31, 2022 was $0.8 million.

| **Lease cost** | **March 31, 2023** |
|---|---|
| Operating lease cost | $ 358 |
| Finance lease cost | |
| Amortization of right of use assets | 538 |
| Interest on lease liabilities | 468 |
| Total lease cost | $ 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 and a reconciliation to the operating and finance lease liabilities as of March 31, 2023:

| **Fiscal Year Ending December 31,** | **March 31, 2023** |
|---|---|
| 2023 (nine months) | 869 |
| 2024 | 798 |
| 2025 | 538 |
| 2026 | 545 |
| 2027 | 506 |
| Thereafter | 2,941 |
| Total lease payments | $ 6,197 |
| Less: interest | (1,842) |
| Total operating lease liabilities | $ 4,355 |

| Fiscal Year Ending December 31, | March 31, 2023 |
|---|---|
| 2023 (nine months) | 1,305 |
| 2024 | 1,791 |
| 2025 | 1,856 |
| 2026 | 1,912 |
| 2027 | 1,969 |
| Thereafter | 43,032 |
| Total lease payments | $ 51,865 |
| Less: interest | (30,823) |
| Total finance lease liability | $ 21,042 |

| Leases | March 31, 2023 |
|---|---|
| Operating right-of-use assets | $ 4,369 |
| | |
| Operating current lease liabilities | 849 |
| Operating noncurrent lease liabilities | 3,506 |
| Total operating lease liabilities | $ 4,355 |
| | |
| Finance right-of-use assets | $ 46,133 |
| | |
| Finance current lease liability | 1,748 |
| Finance noncurrent lease liability | 19,294 |
| Total finance lease liability | $ 21,042 |

**Other information**

| Cash paid for amounts included in the measurement of lease liabilities: | |
|---|---|
| Operating cash flows from operating leases | $ 261 |
| Cash flows from finance lease | $ 431 |
| Weighted-average remaining lease term - operating leases | 8.2 years |
| Weighted-average remaining lease term - finance lease | 21.4 years |
| Weighted-average discount rate - operating leases | 8.08% |
| Weighted-average discount rate - finance lease | 8.96% |

***Litigation***

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

***Indemnification Arrangements***

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

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

## 13. Agreements Related to Intellectual Property

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

## 14. Renovacor Acquisition

On September 19, 2022, the Company entered into an Agreement and Plan of Merger (the "Merger Agreement") with Renovacor, a Delaware corporation pursuant to which, on December 1, 2022, the Company acquired Renovacor (the "Renovacor Acquisition"). On December 1, 2022, pursuant to the terms of the Merger Agreement, (i) Merger Sub I merged with and into the Company (the "First Merger") and (ii) the Company, as the surviving company of the First Merger merged with and into Merger Sub II, with Merger Sub II surviving the Second Merger. Subject to the terms and conditions of the Merger Agreement, at the closing of the Renovacor Acquisition each share of Renovacor's common stock outstanding immediately prior to the effective time of the First Merger were canceled and converted into the right to receive 0.1763 (the "Exchange Ratio") of fully paid and non-assessable shares of the Company common stock, which was determined on the basis of the exchange formula set forth in the Merger Agreement that was subject to adjustment depending on the level of the Renovacor's net cash at the closing. Prior to the market opening on December 1, 2022, Renovacor shares ceased to trade on NYSE and upon the closing of the acquisition, Renovacor's outstanding common stock were converted into 3,391,976 shares of Rocket common stock.

Total consideration for the Renovacor Acquisition was $72.3 million, consisting of $62.4 million for common stock outstanding, $2.7 million for the portion of equity compensation attributable to the pre-combination service period, and $7.2 million for assumed warrants. The consideration was based on the estimated fair values on the acquisition date of (i) 3,391,976 common shares issued for shares outstanding for common shares of Renovacor, (ii) estimated fair value of employee stock options to acquire 367,852 common shares of the Company, (iii) 28,798 common shares issued for employee time-vesting RSUs, and (iv) warrants to acquire 1,503,229 common shares (see Note 10 "Warrants").

The total consideration for the acquisition of Renovacor of $72.3 million consisted of the following:

| | Shares | Value | Total |
|---|---|---|---|
| Stock consideration | 3,391,976 | $ 18.39 | $ 62,378 |
| Cash consideration[1] | | | 29 |
| Stock options | 367,852 | | 2,163 |
| Time-vesting RSUs | 28,798 | | 512 |
| Assumed warrants[2] | 1,503,229 | | 7,183 |
| Total consideration | 5,291,855 | | $ 72,265 |

(1) Represents consideration paid for cash in lieu of fractional shares.
(2) Assumed Renovacor Warrants of $7,183 with $5,671 classified as equity and $1,512 classified as liabilities.

The acquisition has been accounted for as a business combination using the acquisition method of accounting which requires that assets acquired and liabilities assumed be recognized at their fair values as of the acquisition date and that the fair value of acquired IPR&D assets are classified as indefinite-life assets until the successful completion or abandonment of the associated research and development efforts.

The preliminary purchase price allocation resulted in the following amounts being allocated to the assets acquired and liabilities assumed at the acquisition date based on their respective preliminary fair values summarized below:

| | |
|---|---|
| Working capital[1] | $ (5,210) |
| Cash and cash equivalents | 42,755 |
| Property and equipment | 1,414 |
| Operating lease right-of-use assets | 1,161 |
| Other non-current assets | 113 |
| IPR&D | 25,150 |
| Other intangible asset | 574 |
| Operating lease liability | (970) |
| Deferred tax liability | (1,061) |
| **Net assets acquired** | 63,926 |
| Goodwill | 8,339 |
| **Purchase consideration** | $ 72,265 |

(1) Includes other receivables, prepaid expenses, account payable and accrued liabilities.

The fair value assigned to acquired IPR&D was based on the present value of expected after-tax cash flows attributable to Renovacor's most advanced AAV-based gene therapy targeting BAG3-DCM. The present value of expected after-tax cash flows was determined by estimating the after-tax costs to complete development into a commercially viable product, estimating future revenue and ongoing expenses to produce, and discounting the resulting net cash flows to present value. The cost and revenue projections used were reduced based on the assessed probabilities of different stages of development. Acquired IPR&D will be accounted for as an indefinite-lived intangible asset until regulatory approval in a major market or discontinuation of development.

The excess of purchase price over the fair value of amounts assigned to identifiable assets acquired and liabilities assumed represents the goodwill amount of $8.3 million resulting from the acquisition. The goodwill recorded as part of the acquisition is primarily attributable to the broadening of the Company's portfolio and research capabilities, deferred taxes and the assembled workforce. The goodwill attributable to the acquisition has been recorded as a non-current asset in the Company's consolidated balance sheet and is not amortized, but subject to review for impairment annually.

## 15. CIRM Grants

### *LAD-1 CIRM Grant*

On April 30, 2019, the California Institute for Regenerative Medicine ("CIRM") awarded the Company up to $7.5 million under a CLIN2 grant award to support the clinical development of its LV-based gene therapy for RP-L201. Proceeds from the grant will help fund clinical trial costs as well as manufactured drug product for Phase 1/2 patients enrolled at the U.S. clinical site, University of California, Los Angeles ("UCLA") Mattel Children's Hospital, led by principal investigator Donald Kohn, M.D., UCLA Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics (Hematology/Oncology), Molecular and Medical Pharmacology and member of the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at UCLA. Through March 31, 2023, the Company has received $5.9 million in total RP-L201 grants from CIRM. No additional milestones were achieved as of March 31, 2023.

## 16. Related Party Transactions

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

## 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, 2023 and 2022, was $0.3 million and $0.2 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 28, 2023 with the SEC (the "2022 Form 10-K"). This discussion contains forward-looking statements based upon current plans, expectations and beliefs that involve risks and uncertainties. Our actual results may differ materially from those anticipated in these forward-looking statements as a result of various factors, including, but not limited to, those discussed in the section entitled "Risk Factors" and elsewhere in this Quarterly Report on Form 10-Q. In preparing this MD&A, we presume that readers have access to and have read the MD&A in our 2022 Form 10-K, pursuant to Instruction 2 to paragraph of Item 303 of Regulation S-K. Unless stated otherwise, references in this Quarterly Report on Form 10-Q to "us," "we," "our," or our "Company" and similar terms refer to Rocket Pharmaceuticals, Inc.*

We are a clinical-stage, multi-platform biotechnology company focused on the development of first, only and best-in-class gene therapies, with direct on-target mechanism of action and clear clinical endpoints, for rare and devastating diseases. We have three clinical-stage *ex vivo* lentiviral vector ("LV") programs. These include programs for Fanconi Anemia ("FA"), a genetic defect in the bone marrow that reduces production of blood cells or promotes the production of faulty blood cells, Leukocyte Adhesion Deficiency-I ("LAD-I"), a genetic disorder that causes the immune system to malfunction and Pyruvate Kinase Deficiency ("PKD"), a rare red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia. Of these, both the Phase 2 FA program and the Phase ½ LAD-I program produced data read out in 2022 and regulatory filings in the United States ("U.S.") and Europe ("EU") are anticipated in 2023. 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 a clinical stage in vivo adeno-associated virus ("AAV") program for Danon disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. The Danon program is currently in an ongoing Phase 1 trial and pivotal Phase 2 study initiation expected in the second quarter of 2023. Additionally, we have an AAV vector program targeting Plakophilin-2 Arrhythmogenic Cardiomyopathy ("PKP2-ACM"), 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, also referred to as Pegasus, will be approaching IND submission in the second quarter of 2023. As a result of our acquisition of Renovacor, Inc. ("Renovacor"), we are now able to utilize recombinant AAV9-based gene therapy designed to slow or halt progression of BAG3 Dilated Cardiomyopathy ("DCM"), 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. We have global commercialization and development rights to all of these product candidates under royalty-bearing license agreements.

Effective December 2021, a decision was made to no longer pursue Rocket-sponsored clinical evaluation of RP-L401; this program was returned to academic innovators. Although we believe that gene therapy may be beneficial to patients afflicted with this disorder, we have opted to focus available resources towards advancement of RP-A601, RP-A501, RP-L102, RP-L201 RP-L301, and BAG3-DCM based on the compelling clinical data to date and potential for therapeutic advancement in these severe disorders of childhood and young adulthood.

### Recent Developments

#### *At-the-Market Offering Program*

On February 28, 2022, we entered into the Sales Agreement with Cowen with respect to an at-the-market offering program pursuant to which we may offer and sell, from time to time at its sole discretion, shares through Cowen as our sales agent. The shares to be offered and sold under the Sales Agreement, if any, will be offered and sold pursuant to our shelf registration statement on Form S-3. We filed a prospectus supplement with the SEC on February 28, 2022 in connection with the offer and sale of the shares pursuant to the Sales Agreement. We will pay Cowen a cash commission of 3.0% of gross proceeds from the sale of the shares pursuant to the Sales Agreement. We also agreed to provide Cowen with customary indemnification and contribution rights. We reimbursed Cowen for certain expenses incurred in connection with the Sales Agreement. Through March 31, 2023, 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. During the three months ended March 31, 2023, the Company sold 0.9 million shares under the at-the-market offering program for gross proceeds of $17.8 million, less commission of $0.6 million for net proceeds of $17.2 million.

#### *Renovacor Acquisition*

On September 19, 2022, the Company entered into an Agreement and Plan of Merger (the "Merger Agreement") with Renovacor, a Delaware corporation pursuant to which the Company acquired Renovacor (the "Renovacor Acquisition"). The Renovacor Acquisition closed on December 1, 2022. Subject to the terms and conditions of the Merger Agreement, each share of Renovacor's common stock, par value $0.0001 per share outstanding immediately prior to the effective time of the Renovacor Acquisition was canceled and converted into the right to receive 0.1763 (the "Exchange Ratio") fully paid and non-assessable shares of the Company's common stock, $0.01 par value per share, which was determined on the basis of an exchange formula set forth in the Merger Agreement. The Company issued a total of 3,391,976 shares of common stock in connection with the Renovacor Acquisition and incurred approximately $1.3 million of acquisition related costs.

***Gene Therapy Overview***

Genes are composed of sequences of deoxyribonucleic acid ("DNA"), 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.

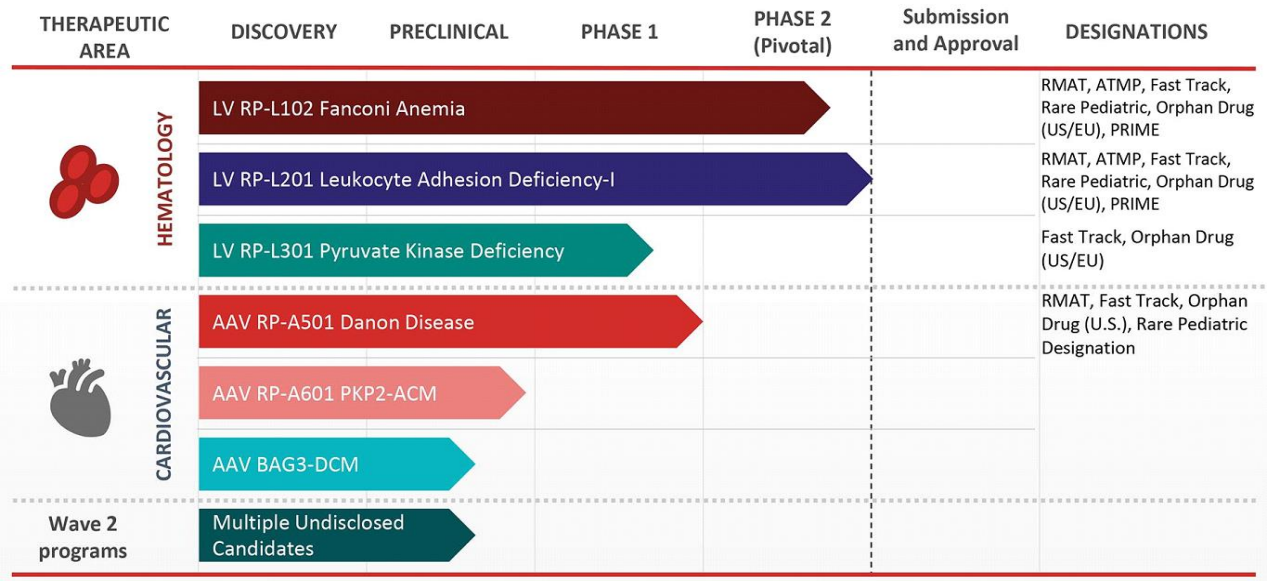
We are using modified non-pathogenic viruses for the development of our gene therapy treatments. Viruses are particularly well suited as delivery vehicles because they are adept at penetrating cells and delivering genetic material inside a cell. In creating our viral delivery vehicles, the viral (pathogenic)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

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

We believe that scientific advances, clinical progress, and the greater regulatory acceptance of gene therapy have created a promising environment to advance gene therapy products as these products are being designed to restore cell function and improve clinical outcomes, which in many cases include prevention of death at an early age. The FDA approval of several gene therapies in recent years indicates that there is a regulatory pathway forward for gene therapy products.

***Pipeline Overview***

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



**Cardiovascular Programs**

***Danon Disease***

Danon disease ("DD") is a multi-organ lysosomal-associated disorder leading to early death due to heart failure. DD is caused by mutations in the gene encoding lysosome-associated membrane protein 2 ("LAMP-2"), a mediator of autophagy. This mutation results in the accumulation of autophagic vacuoles, predominantly in cardiac and skeletal muscle. Male patients often require heart transplantation and typically die in their teens or twenties from progressive heart failure. Along with severe cardiomyopathy, other DD-related manifestations can include skeletal muscle weakness and intellectual impairment. There are no specific therapies available for the treatment of DD and medications typically utilized for the treatment of congestive heart failure ("CHF") are not believed to modify progression to end-stage CHF. Patients with end-stage CHF may undergo heart transplant, which currently is available to a minority of patients, is associated with significant short- and long-term complications and is not curative of the disorder in the long-term. RP-A501 is in clinical trials as an *in vivo* therapy for 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 ("TMA")) 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 are conducting a variety of efficacy assessments in the Phase I clinical study to measure the prospect of benefit for patients. These assessments include the following:

- New York Heart Association ("NYHA") 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 ("BNP") is a blood-based evaluation and a key marker of heart failure with prognostic significance in CHF and cardiomyopathies. Elevations in BNP are strongly associated with worsening heart failure and poor outcomes in cardiovascular disease.
- High sensitivity troponin I ("hsTnI") is a blood-based evaluation and a key marker of cardiac injury, one that is (like BNP) frequently elevated in 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 ("LVM") and maximal left ventricular wall thickness ("MLVWT"), indicate the degree of hypertrophy present in the heart.
- Kansas City Cardiovascular Questionnaire ("KCCQ") 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 ("H&E") 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.
- LAMP2B gene expression in endomyocardial biopsy samples is measured via both immunohistochemistry and Western blot and confirms the presence of LAMP2B protein in DD cardiac tissue following RP-A501 treatment.

In September 2022, we presented interim data for the ongoing Phase 1 trial of RP-A501 at the Heart Failure Society of America ("HFSA") meeting, including updated safety and initial efficacy parameters for the pediatric cohort and longer-term efficacy parameters for the low and high dose adult/older adolescent cohort (patients aged 15 and older; n=5) (data cut-off September 27, 2022). This data was also presented in November 2022 at the 75th American Heart Association ("AHA") Annual Meeting. During these presentations we provided incremental safety updates across cohorts. As previously outlined, RP-A501 was generally well tolerated at the 6.7e13 gc/kg dose level and no unexpected and serious drug product-related adverse events or severe adverse events were observed in both adult/older adolescent and pediatric low dose cohorts. All observed adverse effects at both doses were reversible and no lasting sequelae were observed with follow-up of 2-3 years from treatment for the adult/older adolescent cohort and 6-11 months for the pediatric cohort. Any early transaminase and creatinine kinase elevations returned to baseline or decreased, and any transient exacerbation of DD-associated skeletal myopathy resolved upon discontinuation of corticosteroid therapy. The updated safety data presented at HFSA in September 2022 and at AHA in November 2022 reconfirmed that RP-A501 was generally well tolerated at the low dose with a manageable safety profile across pediatric and adult/older adolescent cohorts.

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

On December 22, 2022, we announced updates from our end-of-Phase 1 meeting with the FDA regarding RP-A501. During the meeting, we reviewed the positive Phase 1 dataset with the FDA and proposed a study design and endpoints for ongoing clinical development of the investigational gene therapy. Following discussions with the FDA, we anticipate proceeding with a dose of 6.7e13 GC/kg, and we anticipate utilizing a single arm open-label trial design with a robust natural history comparator, pursuant to the FDA's acknowledgment of the challenges associated with executing a randomized controlled trial in DD. The FDA has also expressed an openness to considering a biomarker-based composite endpoint supported by functional and quality-of-life assessments as measures of patient benefit. We look forward to continued dialogue with the FDA on the design for our proposed pivotal trial, including discussion of appropriate external controls for the study and appropriate endpoints to support accelerated approval. We are now in discussion with the FDA about a trial design that will enable evaluation of two pediatric patients treated with drug product manufactured at our in-house cGMP AAV facility as an initial component of a modestly sized global pivotal study.

On January 9, 2023, we presented additional 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[2] | +5.3 |
| | 1002 | 36 | ↓96% | ↓94% | ↓48% | ↓40% | II -> I | +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 pt 1007 in Ph1 trial who had advanced HF with EF<40% at enrollment and received HTx 5M following tx due to pre-existing advanced HF. Patient is currently stable.
[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. We have 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.

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.

*Anticipated Milestones*

On February 7, 2023, we announced that RP-A501 received RMAT designation from the FDA. We are very encouraged by the highly collaborative ongoing dialogue with the FDA for RP-A501 in DD and subject to the continued dialogue and agreement with the FDA anticipate initiating the initial component of the global study in the second quarter of 2023.

***Plakophilin-2 Arrhythmogenic Cardiomyopathy (PKP2-ACM)***

Arrhythmogenic cardiomyopathy ("ACM") 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 (ARVD/ARVC). 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 ("SCD").

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 ("ICD") for primary or secondary prevention of ventricular arrhythmias and SCD. Of note, ICDs are not curative, and breakthrough life-threatening arrythmias may persist with ongoing risk of death; ICDs furthermore 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 US and EU.

We currently have one adeno-associated viral vector 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 SCD associated with ACM, potentially impeding the development of irreversible cardiac structural changes. Prevention of syncopal episodes, life-threatening arrythmias, SCD, 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 nonclinical studies conducted by the Sponsor, RP-A601 has demonstrated efficacy in altering the natural history of PKP2-driven ACM. PKP2 cKO animals treated with the study drug have 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.

*Anticipated 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. We anticipate filing an IND in the second quarter of 2023.

***BAG3 Dilated Cardiomyopathy***

Dilated cardiomyopathy ("DCM") 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 United States 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-BAG3DCM patients.

In December 2022 we completed our acquisition of Renovacor which provided Rocket with Renovacor's most advanced program, a recombinant AAV9-based gene therapy 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.

*Anticipated 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 the first half of 2024.

**Hematology Programs**

***Fanconi Anemia Complementation Group A (FANCA)***

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

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

Each of our 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 acute myeloid leukemia ("AML"), a type of blood cancer, as well as bone marrow failure and congenital defects. The average lifespan of an FA patient is estimated to be 30 to 40 years. The prevalence of FA in the U.S. and EU is estimated to be approximately 4,000 patients in total. In light of the efficacy seen in non-conditioned patients, the addressable annual market opportunity is now believed to be 400 to 500 patients collectively in the U.S. and EU.

We currently have one ex-vivo LV-based program targeting FA, RP-L102. RP-L102 is our lead LV-based program that we in-licensed from Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas ("CIEMAT"), which is a leading research institute in Madrid, Spain. RP-L102 is currently being studied in our Phase 2 registrational enabling clinical trials treating FA patients at the Center for Definitive and Curative Medicine at Stanford University School of Medicine ("Stanford"), the University of Minnesota, Great Ormond Street Hospital ("GOSH") in London and Hospital Infantil de Nino Jesus ("HNJ") in Spain. The trial has enrolled a total of ten patients from the U.S. and EU. Two additional patients were treated in the US Phase 1 study at Stanford such that a total of 12 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 May 2022, we presented topline data for RP-L102 at ASGCT's 25th Annual Meeting. Five of nine evaluable patients as of the April 4, 2022 cut-off date had increased resistance to MMC in bone marrow-derived colony forming cells, ranging from 21% to 42% at 12 to 18 months, increasing to 51% to 94% at 18 – 21 months. The primary endpoint has been achieved, based on a trial protocol in which statistical and clinical significance requires a minimum of five patients to attain increased MMC resistance at least 10% above baseline at two or more timepoints, and concomitant evidence of genetic correction and clinical stabilization. A sixth patient has displayed evidence of progressively increasing genetic correction as evidenced by peripheral VCN. Three additional patients were less than 12 months post-treatment at the time of presentation. One patient had progressive bone marrow failure following therapy and underwent successful allogeneic transplant as previously disclosed. The tolerability profile of RP-L102 appears favorable with no signs of dysplasia, clonal dominance or oncogenic integrations; as previously reported, one patient experienced a Grade 2 transient infusion-related reaction, which resolved.

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

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.

*Anticipated Milestones*

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

***Leukocyte Adhesion Deficiency-I (LAD-I)***

LAD-I is a rare autosomal recessive disorder of white blood cell adhesion and migration, resulting from mutations in the ITGB2 gene encoding for the Beta-2 Integrin component, CD18. Deficiencies in CD18 result in an impaired ability for neutrophils (a subset of infection-fighting white blood cells) to leave blood vessels and enter tissues where these cells are needed to combat infections. As is the case with many rare diseases, accurate estimates of incidence are difficult to confirm; however, several hundred cases have been reported to date. Most LAD-I patients are believed to have the severe form of the disease. Severe LAD-I is notable for recurrent, life-threatening infections and substantial infant mortality in patients who do not receive an allogeneic HSCT. Mortality for severe LAD-I has been reported as 60 to 75% by age two in the absence of allogeneic HCST.

We currently have one *ex-vivo* program targeting LAD-I, RP-L201. RP-L201 is a clinical program that we in-licensed from CIEMAT. We have partnered with UCLA to lead U.S. clinical development efforts for the LAD-I program. UCLA and its Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research is serving as the lead U.S. clinical research center for the registrational clinical trial for LAD-I, and HNJ and GOSH serving as the lead clinical sites in Spain and London, respectively. This study has received a $6.6 million CLIN2 grant award from the California Institute for Regenerative Medicine ("CIRM") to support the clinical development of gene therapy for LAD-I.

The open-label, single-arm, Phase 1/2 registration-enabling clinical trial of RP-L201 has treated nine severe LAD-I patients to assess the safety and tolerability of RP-L201 to date. The first patient was treated at UCLA with RP-L201 in the third quarter 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 2021, we presented positive clinical data at the 63rd Annual Meeting of ASH. The ASH oral presentation included preliminary data from eight of nine severe LAD-I patients, as defined by CD18 expression of less than 2%, who received RP-L201 treatment as of the November 8, 2021, data cut-off date. Eight patients had follow-up data of at least three months, and four of the eight patients had been followed for 12 months or longer. All infusions of RP-L201 were well tolerated and no drug product-related serious adverse events were reported. Evidence of preliminary efficacy was observed in all eight evaluable patients. All eight patients demonstrated neutrophil CD18 expression that exceeded the 4-10% threshold associated with survival into adulthood and consistent with reversal of the severe LAD-I phenotype including six patients with at least 6 months of follow-up. Peripheral blood VCN levels have been stable and in the 0.54 – 2.94 copies per genome range. No patients had LAD-I related infections requiring hospitalization after hematopoietic reconstitution post-RP-L201. Additional updates presented in January 2022 included a ninth patient achieving CD18 expression of 61% at 3 months, with the preliminary observation that all nine of nine patients have demonstrated 26% to 87% CD18 expression at timepoints ranging from 3 to 24 months following RP-L102, with stable CD18 expression levels for each patient subsequent to month 3.

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

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. 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.

*Anticipated Milestones*

Based on the positive efficacy and safety data from the Phase 2 pivotal LAD-I trial, we have initiated discussions with the FDA on BLA filing plans for RP-L201 for the treatment of severe LAD-I and anticipate making such filing in the second quarter of 2023.

***Pyruvate Kinase Deficiency (PKD)***

Red blood cell PKD is a rare autosomal recessive disorder resulting from mutations in the pyruvate kinase L/R ("PKLR") gene encoding for a component of the red blood cell ("RBC") glycolytic pathway. PKD is characterized by chronic non-spherocytic hemolytic anemia, a disorder in which RBCs do not assume a normal spherical shape and are broken down, leading to decreased ability to carry oxygen to cells, with anemia severity that can range from mild (asymptomatic) to severe forms that may result in childhood mortality or a requirement for frequent, lifelong RBC transfusions. The pediatric population is the most commonly and severely affected subgroup of patients with PKD, and PKD often results in splenomegaly (abnormal enlargement of the spleen), jaundice and chronic iron overload which is likely the result of both chronic hemolysis and the RBC transfusions used to treat the disease. The variability in anemia severity is believed to arise in part from the large number of diverse mutations that may affect the PKLR gene. Estimates of disease incidence have ranged between 3.2 and 51 cases per million in the white U.S. and EU population. Industry estimates suggest at least 2,500 cases in the U.S. and EU have already been diagnosed despite the lack of FDA-approved molecularly targeted therapies. Market research indicates the application of gene therapy to broader populations could increase the market opportunity from approximately 250 to 500 patients per year.

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

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

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

In December 2022, we presented positive clinical data at the 64th Annual Meeting of ASH. The presentation included positive updated data from two adult patients with significant anemia. At 24 months post-infusion, both patients have robust and sustained efficacy demonstrated by normalized hemoglobin (from baseline levels in the 7.0-7.5 g/dL range), improved hemolysis parameters, independence from red blood cell transfusions and improved quality of life both reported anecdotally and as documented via formal quality of life assessments. The safety profile appears highly favorable, with no RP-L301-related serious adverse events through 24 months post-infusion in both adult patients. Insertion site analyses in peripheral blood and bone marrow in both adult patients up to 12 months post-RP-L301 demonstrated highly polyclonal patterns and there has been no evidence of insertional mutagenesis.

*Anticipated Milestones*

Enrollment in the PKD adult and pediatric cohort is completed in the Phase 1 study. Initiation of the phase 2 pivotal trial is anticipated in the fourth quarter of 2023.

***cGMP Manufacturing***

Our 103,720 square foot manufacturing facility in Cranbury, New Jersey has been scaled up to manufacture AAV drug product for a planned Phase 2 pivotal study in 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. To further strengthen our manufacturing and commercial capabilities during 2022, we appointed Mayo Pujols, one of the most seasoned cell and gene therapy technical operations and manufacturing leaders in the industry, as our Chief Technical Officer.

***Strategy***

We seek to bring hope and relief to patients with devastating, undertreated, rare pediatric diseases through the development and commercialization of potentially curative first-in-class gene therapies. To achieve these objectives, we intend to develop into a fully-integrated biotechnology company. In the near and medium-term, we intend to develop our first-in-class product candidates, which are targeting devastating diseases with substantial unmet need, develop proprietary in-house analytics and manufacturing capabilities and continue to commence registration trials for our currently planned programs. 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, 2023, we raised net cash proceeds of approximately $852.8 million from investors through both equity and convertible debt financing to fund operating activities.

***Revenue***

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

***Operating Expenses***

***Research and Development Expenses***

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

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

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

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

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

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

The following table presents R&D expenses tracked on a program-by-program basis as well as by type and nature of expense for the three months ended March 31, 2023 and 2022.

| | Three Months Ended March 31, | |
|---|---|---|
| | 2023 | 2022 |
| **Direct Expenses:** | | |
| Danon Disease (AAV) RP-A501 | $ 6,403 | $ 6,374 |
| Leukocyte Adhesion Deficiency (LV) RP-L201 | 5,841 | 3,051 |
| Fanconi Anemia (LV) RP-L102 | 6,548 | 4,530 |
| Pyruvate Kinase Deficiency (LV) RP-L301 | 299 | 854 |
| Infantile Malignant Osteopetrosis (LV) RP-L401(1) | - | 190 |
| Other product candidates | 3,439 | 3,254 |
| Total direct expenses | 22,530 | 18,253 |
| **Unallocated Expenses** | | |
| Employee compensation | $ 11,210 | $ 5,549 |
| Stock based compensation expense | 3,819 | 2,318 |
| Depreciation and amortization expense | 1,137 | 827 |
| Laboratory and related expenses | 5,102 | 1,226 |
| Professional Fees | 985 | 561 |
| Other expenses | 1,588 | 2,060 |
| Total other research and development expenses | 23,841 | 12,541 |
| Total research and development expense | $ 46,371 | $ 30,794 |

(1) Effective December 2021, a decision was made to no longer pursue Rocket-sponsored clinical evaluation of RP-L401; this program was returned to academic innovators. Costs to close out the study were incurred in 2022.

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

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

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

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

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

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

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

***General and Administrative Expenses***

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

***Interest Expense***

Interest expense for the three months ended March 31, 2023 and 2022, related to our financing lease obligation for the 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 condensed consolidated financial statements during the three months ended March 31, 2023, compared to those disclosed in our 2022 Form 10-K.

**Results of Operations**

***Comparison of the Three Months Ended March 31, 2023 and 2022***

| | Three Months Ended March 31, | | |
|---|---|---|---|
| | 2023 | 2022 | Change |
| Operating expenses: | | | |
| Research and development | $ 46,371 | $ 30,794 | $ 15,577 |
| General and administrative | 15,823 | 11,770 | 4,053 |
| Total operating expenses | 62,194 | 42,564 | 19,630 |
| Loss from operations | (62,194) | (42,564) | (19,630) |
| Interest expense | (468) | (464) | (4) |
| Interest and other income, net | 1,908 | 623 | 1,285 |
| Accretion of discount and amortization of premium on investments, net | 2,419 | (577) | 2,996 |
| Total other income (expense), net | 3,859 | (418) | 4,277 |
| Net loss | $ (58,335) | $ (42,982) | $ (15,353) |

*Research and Development Expenses*

R&D expenses increased $15.6 million to $46.4 million for the three months ended March 31, 2023 compared to the three months ended March 31, 2022. The increase in R&D expenses was primarily driven by increases in manufacturing and development costs of $2.8 million, compensation and benefits of $6.6 million due to increased R&D headcount, direct materials of $0.9 million, and laboratory supplies of $0.9 million.

*General and Administrative Expenses*

G&A expenses increased $4.1 million to $15.8 million for the three months ended March 31, 2023, compared to the three months ended March 31, 2022. The increase in G&A expenses was primarily driven by increases in commercial preparation related expenses of $1.1 million, compensation and benefits of $0.7 million due to increased G&A headcount and non-cash stock compensation expense of $1.1 million.

*Other Income (Expense), Net*

Other income increased $4.3 million to $3.9 million for the three months ended March 31, 2023, compared to the three months ended March 31, 2022. The increase in other income was primarily driven by an increase in interest and other income, net, of $1.3 million and an increase in accretion of discount and amortization of premium on investments, net, of $3.0 million. The increase in interest and other income, net, of $1.3 million was due to increased interest rates of $0.7 million and reduced fair value of warrant liability of $0.7 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, uncertainty of drug candidate development, technological uncertainty, uncertainty regarding patents and proprietary rights, having no commercial manufacturing experience, marketing or sales capability or experience, dependency on key personnel, compliance with government regulations and the need to obtain additional financing. Drug candidates currently under development will require significant additional R&D efforts, including extensive preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure, and extensive compliance-reporting capabilities.

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

Our consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities in the ordinary course of business. Rocket has incurred net losses and negative cash flows from its operations each year since inception. Rocket incurred net losses of $58.3 million for the three months ended March 31, 2023, and $221.9 million for the year ended December 31, 2022. We have experienced negative cash flows from operations and as of March 31, 2023 and December 31, 2022, we had an accumulated deficit of $772.1 million and $713.8 million, respectively. As of March 31, 2023, we had $360.0 million of cash, cash equivalents and investments. We expect such resources will be sufficient to fund our operating expenses and capital expenditure requirements into the first half of 2025. 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***

| | Three Months Ended March 31, | |
|---|---|---|
| | 2023 | 2022 |
| Net cash used in operating activities | $ (57,560) | $ (39,223) |
| Net cash used in investing activities | (36,721) | (62,995) |
| Net cash provided by financing activities | 18,343 | 76 |
| Net decrease in cash, cash equivalents and restricted cash | $ (75,938) | $ (102,142) |

***Operating Activities***

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, accretion of discount on investments of $2.3 million, and depreciation and amortization expense of $1.7 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.

During the three months ended March 31, 2022, operating activities used $39.2 million of cash, primarily resulting from our net loss of $43.0 million offset by net non-cash charges of $8.2 million, including non-cash stock-based compensation expense of $6.3 million, accretion of discount on investments of $0.6, and depreciation and amortization expense of $1.3 million. Changes in our operating assets and liabilities for the three months ended March 31, 2022, consisted of a decrease in accounts payable and accrued expenses of $0.5 million and a decrease in our prepaid expenses of $3.9 million.

***Investing Activities***

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.

During the three months ended March 31, 2022, investing activities used $63.0 million of cash, primarily resulting from proceeds of $82.0 million from the maturities of investments, offset by purchases of investments of $143.0 million, and purchases of property and equipment of $2.0 million.

***Financing Activities***

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.

During the three months ended March 31, 2022, net cash provided by financing activities was $0.1 million, consisting of the issuance of common stock, pursuant to exercises of stock options.

**Contractual Obligations and Commitments**

There were no material changes outside the ordinary course of our business to the contractual obligations specified in the table of contractual obligations included in “Management’s Discussion and Analysis of Financial Condition and Results of Operations” in our 2022 Form 10-K. Information regarding contractual obligations and commitments may be found in Note 12 of our unaudited consolidated financial statements in this Quarterly Report on Form 10-Q. We do not have any off-balance sheet arrangements that are material or reasonably likely to become material to our financial condition or results of operations.

**Recently Issued Accounting Pronouncements**

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

## Item 3 Quantitative and Qualitative Disclosures About Market Risk

Our exposure to market risk is principally confined to our cash, cash equivalents and marketable securities. We invest in U.S. treasury securities, commercial paper and corporate, government and agency bonds, which as of March 31, 2023, were classified as available-for-sale. 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+/Aa1 rated, thereby reducing credit risk exposure.

Based on a hypothetical 100 basis point decrease in market interest rates, the potential losses in future earnings and fair value of risk-sensitive financial instruments are immaterial, although the actual effects may differ materially from the hypothetical analysis. 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 and accounting officers, 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, 2023, our principal executive officer and interim principal financial and accounting officer concluded that our disclosure controls and procedures as of such date are effective at the reasonable assurance level. The term “disclosure controls and procedures,” as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act are recorded, processed, summarized, and reported within the time periods specified in the SEC’s rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and interim principal financial and accounting officer, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

***Inherent Limitations of Internal Controls***

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

***Changes in Internal Control over Financial Reporting***

There were no changes in our internal control over financial reporting 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, the Company may be subject to various legal proceedings and claims that arise in the ordinary course of its business activities. Although the results of litigation and claims cannot be predicted with certainty, the Company does not believe it is party to any other claim or litigation the outcome of which, if determined adversely to the Company, would individually or in the aggregate be reasonably expected to have a material adverse effect on its business. Regardless of the outcome, litigation can have an adverse impact on the Company because of defense and settlement costs, diversion of management resources and other factors.

### Item 1A. Risk Factors

Our material risk factors are disclosed in Item 1A of our 2022 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 and Use of Proceeds

None.

### Item 3. Defaults Upon Senior Securities

None.

### Item 4. Mine Safety Disclosures

Not applicable.

### Item 5. Other Information

None.

**Item 6. Exhibits**

| Exhibit Number | Description of Exhibit |
|---|---|
| 2.1 | Agreement and Plan of Merger and Reorganization, dated as of September 12, 2017, by and among Inotek Pharmaceuticals Corporation, Rocket Pharmaceuticals, Ltd., and Rome Merger Sub (incorporated by reference to Exhibit 2.1 to the Company's Current Report on Form 8- K (001-36829), filed with the SEC on September 13, 2017) |
| 2.2 | Agreement and Plan of Merger, dated September 19, 2022, by and among 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 to the Seventh Amended and Restated Certificate of Incorporation of the Registrant, effective as of June 25, 2018 (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on June 25, 2019 |
| 3.5 | Amended and Restated By-Laws of Rocket Pharmaceuticals, Inc., effective as of March 29, 2018 (incorporated by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on April 4, 2018) |
| 10.1 | Assignment, Assumption and Amended & Restated Warrant Agreement, dated January 16, 2023, by and among Rocket Pharmaceuticals, Inc., Zebrafish Merger Sub II, LLC, as successor to Renovacor, Inc,, and Continental Stock Transfer & Trust Company. (incorporated by reference to Exhibit 4.3 to the Company's Registration Statement on Form 8-A (001-36829), filed with the SEC on February 23, 2023. |
| 31.1* | Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002 |
| 31.2* | Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002 |
| 32.1** | Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002 |
| 101.INS | Inline XBRL Instance Document. |
| 101.SCH | Inline XBRL Taxonomy Extension Schema Document. |
| 101.CAL | Inline XBRL Taxonomy Extension Calculation Document. |
| 101.DEF | Inline XBRL Taxonomy Extension Definition Linkbase Document. |
| 101.LAB | Inline XBRL Taxonomy Extension Labels Linkbase Document. |
| 101.PRE | Inline XBRL Taxonomy Extension Presentation Link Document. |
| 104 | Cover Page Interactive Data File (the cover page XBRL tags are embedded within the Inline XBRL document) |

* Filed herewith.

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

**SIGNATURES**

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

ROCKET PHARMACEUTICALS, INC.

May 5, 2023 By: /s/ Gaurav Shah, MD

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

May 5, 2023 By: /s/ John Militello

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

**Exhibit 31.1**

## CERTIFICATIONS

I, Gaurav Shah, MD, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended March 31, 2023 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 5, 2023

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

**Exhibit 31.2**

## CERTIFICATIONS

I, John Militello, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended March 31, 2023 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 5, 2023

By: /s/ John Militello
John Militello
*VP of Finance, Senior Controller and Treasurer*
*(Interim Principal Financial Officer and Principal Accounting Officer)*

**Exhibit 32.1**

**CERTIFICATION PURSUANT TO**

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

**SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002**

In connection with the quarterly report on Form 10-Q of Rocket Pharmaceuticals, Inc. (the "Company") for the period ended March 31, 2023, 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 5, 2023

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

Date: May 5, 2023

By: /s/ John Militello
John Militello
*VP of Finance, Senior Controller and Treasurer*
*(Interim Principal Financial Officer and Principal Accounting Officer)*