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

# Form 10-Q

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

**For the quarterly period ended June 30, 2021**

**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)**

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

Indicate by check mark whether the registrant (1) has filed all reports required to be filed by Section 13 or 15(d) of the Securities Exchange Act of 1934 during the preceding 12 months (or for such shorter period that the registrant was required to file such reports), and (2) has been subject to such filing requirements for the past 90 days. Yes ☒ No ☐

Indicate by check mark whether the registrant has submitted electronically every Interactive Data File required to be submitted pursuant to Rule 405 of Regulation S-T (§ 232.405 of this chapter) during the preceding 12 months (or for such shorter period that the registrant was required to submit such files). Yes ☒ No ☐

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

| | |
|---|---|
| Large accelerated filer ☒ | Accelerated filer ☐ |
| Non-accelerated filer ☐ | Smaller reporting company ☐ |
| Emerging growth company ☐ | |

If an emerging growth company, indicate by check mark if the registrant has elected not to use the extended transition period for complying with any new or revised financial accounting standards provided pursuant to Section 13(a) of the Exchange Act. ☐

Indicate by check mark whether the registrant is a shell company (as defined in Rule 12b-2 of the Exchange Act). Yes ☐ No ☒

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

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

As of August 2, 2021, there were 63,615,685 shares of common stock, $0.01 par value per share, outstanding.

## Forward-looking statements

This Quarterly Report on Form 10-Q for the quarter ended June 30, 2021 (Form 10-Q) contains forward-looking statements that involve risks and uncertainties, as well as assumptions that, if they never materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q are forward-looking statements. In some cases, you can identify forward-looking statements by words such as “anticipate,” “believe,” “contemplate,” “continue,” “could,” “estimate,” “expect,” “intend,” “may,” “plan,” “potential,” “predict,” “project,” “seek,” “should,” “target,” “will,” “would,” or the negative of these words or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- federal, state, and non-U.S. regulatory requirements, including regulation of our current or any other future product candidates by the U.S. Food and Drug Administration (“FDA”);
- the timing of and our ability to submit regulatory filings with the FDA and to obtain and maintain FDA or other regulatory authority approval of, or other action with respect to, our product candidates;
- our competitors’ activities, including decisions as to the timing of competing product launches, pricing and discounting;
- whether safety and efficacy results of our clinical trials and other required tests for approval of our product candidates provide data to warrant progression of clinical trials, potential regulatory approval or further development of any of our product candidates;
- our ability to develop, acquire and advance product candidates into, enroll a sufficient number of patients into, and successfully complete, clinical studies, and our ability to apply for and obtain regulatory approval for such product candidates, within currently anticipated timeframes, or at all;
- our ability to establish key collaborations and vendor relationships for our product candidates and any other future product candidates;
- our ability to establish key collaborations and vendor relationships for our product candidates and any other future product candidates;
- our ability to acquire additional businesses, form strategic alliances or create joint ventures and our ability to realize the benefit of such acquisitions, alliances, or joint ventures;
- our ability to successfully develop and commercialize any technology that we may in-license or products we may acquire;
- unanticipated delays due to manufacturing difficulties, including the development of our direct manufacturing capabilities for our AAV programs, and any supply constraints or changes in the regulatory environment; our ability to successfully operate in non-U.S. jurisdictions in which we currently or in the future do business, including compliance with applicable regulatory requirements and laws;
- uncertainties associated with obtaining and enforcing patents to protect our product candidates, and our ability to successfully defend ourselves against unforeseen third-party infringement claims;
- anticipated trends and challenges in our business and the markets in which we operate;
- natural and manmade disasters, including pandemics such as COVID-19, and other force majeures, which could impact our operations, and those of our partners and other participants in the health care industry, and which could adversely impact our clinical studies, preclinical research activities, and drug supply;
- 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, 2020, 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.

## PART I — FINANCIAL INFORMATION

**Item 1. Financial Statements**

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

| | June 30, 2021 | December 31, 2020 |
|---|---|---|
| **Assets** | | |
| Current assets: | | |
| Cash and cash equivalents | $ 264,608 | $ 297,098 |
| Investments | 162,222 | 185,621 |
| Prepaid expenses and other current assets | 3,596 | 4,626 |
| Total current assets | 430,426 | 487,345 |
| Property and equipment, net | 20,989 | 19,206 |
| Goodwill | 30,815 | 30,815 |
| Restricted cash | 1,334 | 1,568 |
| Deposits | 455 | 455 |
| Operating lease right-of-use assets | 1,628 | 914 |
| Finance lease right-of-use asset | 49,507 | 50,521 |
| Total assets | $ 535,154 | $ 590,824 |
| **Liabilities and stockholders' equity** | | |
| Current liabilities: | | |
| Accounts payable and accrued expenses | $ 17,884 | $ 25,472 |
| Convertible notes, net of unamortized discount, current | 5,102 | 4,875 |
| Operating lease liabilities, current | 792 | 626 |
| Finance lease liability, current | 1,666 | 1,644 |
| Total current liabilities | 25,444 | 32,617 |
| Convertible notes, net of unamortized discount, non-current | - | 35,066 |
| Operating lease liabilities, non-current | 1,096 | 498 |
| Finance lease liability, non-current | 19,070 | 18,988 |
| Other liabilities | 107 | 136 |
| Total liabilities | 45,717 | 87,305 |
| Commitments and contingencies (Note 11) | | |
| | | |
| Stockholders' equity: | | |
| Preferred stock, $0.01 par value, authorized 5,000,000 shares: | | |
| Series A convertible preferred stock; 300,000 shares designated as Series A; 0 shares issued and outstanding | - | - |
| 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; 63,448,069 and 60,996,367 shares issued and outstanding at June 30, 2021 and December 31, 2020, respectively | 634 | 610 |
| Additional paid-in capital | 886,431 | 825,794 |
| Accumulated other comprehensive loss | (81) | (42) |
| Accumulated deficit | (397,547) | (322,843) |
| Total stockholders' equity | 489,437 | 503,519 |
| Total liabilities and stockholders' equity | $ 535,154 | $ 590,824 |

*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 June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| Revenue | $ - | $ - | $ - | $ - |
| Operating expenses: | | | | |
| Research and development | 24,798 | 16,731 | 53,340 | 33,687 |
| General and administrative | 9,250 | 6,828 | 19,930 | 13,990 |
| Total operating expenses | 34,048 | 23,559 | 73,270 | 47,677 |
| Loss from operations | (34,048) | (23,559) | (73,270) | (47,677) |
| Research and development incentives | - | - | 500 | - |
| Interest expense | (251) | (1,786) | (1,980) | (3,360) |
| Interest and other income - net | 501 | 429 | 1,412 | 1,395 |
| Amortization of premium on investments - net | (727) | (124) | (1,366) | (62) |
| Net loss | $ (34,525) | $ (25,040) | $ (74,704) | $ (49,704) |
| Net loss per share attributable to common stockholders - basic and diluted | $ (0.55) | $ (0.45) | $ (1.20) | $ (0.90) |
| Weighted-average common shares outstanding - basic and diluted | 63,061,232 | 55,158,459 | 62,321,926 | 55,020,789 |

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

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

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| Net loss | $ (34,525) | $ (25,040) | $ (74,704) | $ (49,704) |
| Other comprehensive loss | | | | |
| Net unrealized gain (loss) on investments | (6) | 304 | (39) | 209 |
| Total comprehensive loss | $ (34,531) | $ (24,736) | $ (74,743) | $ (49,495) |

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

**Rocket Pharmaceuticals, Inc.**
**Consolidated Statements of Stockholders' Equity**
**For the Three and Six Months Ended June 30, 2021 and 2020**
**(in thousands except share amounts)**
**(unaudited)**

| | Common Stock | | Treasury Stock | Additional Paid-In Capital | Accumulated Other Comprehensive Income/(Loss) | Accumulated Deficit | Total Stockholders' Equity |
|---|---|---|---|---|---|---|---|
| | Shares | Amount | | | | | |
| **Balance at December 31, 2020** | 60,996,367 | $ 610 | $ - | $ 825,794 | $ (42) | $ (322,843) | $ 503,519 |
| Issuance of common stock pursuant to exercise of stock options | 991,432 | 9 | - | 8,783 | - | - | 8,792 |
| Unrealized comprehensive loss on investments | - | - | - | - | (33) | - | (33) |
| Share-based compensation | - | - | - | 7,900 | - | - | 7,900 |
| Net loss | - | - | - | - | - | (40,179) | (40,179) |
| Balance at March 31, 2021 | 61,987,799 | 619 | - | 842,477 | (75) | (363,022) | 479,999 |
| Issuance of common stock pursuant to exercise of stock options | 133,838 | 2 | - | 1,113 | - | - | 1,115 |
| Issuance of common stock pursuant to conversion of notes | 1,326,432 | 13 | - | 35,530 | - | - | 35,543 |
| Unrealized comprehensive loss on investments | - | - | - | - | (6) | - | (6) |
| Share-based compensation | - | - | - | 7,311 | - | - | 7,311 |
| Net loss | - | - | - | - | - | (34,525) | (34,525) |
| **Balance at June 30, 2021** | 63,448,069 | $ 634 | $ - | $ 886,431 | $ (81) | $ (397,547) | $ 489,437 |
| | | | | | | | |
| **Balance at December 31, 2019** | 54,773,061 | $ 548 | $ (53) | $ 489,925 | $ 20 | $ (183,143) | $ 307,297 |
| Issuance of common stock pursuant to exercise of stock options | 386,974 | 3 | - | (3) | - | - | - |
| Share repurchase | (3,000) | - | - | (72) | - | - | (72) |
| Sale of treasury stock | - | - | 53 | - | - | - | 53 |
| Issuance of treasury stock pursuant to exercise of stock options | - | - | (429) | - | - | - | (429) |
| Unrealized comprehensive loss on investments | - | - | - | - | (95) | - | (95) |
| Share-based compensation | - | - | - | 3,961 | - | - | 3,961 |
| Net loss | - | - | - | - | - | (24,664) | (24,664) |
| Balance at March 31, 2020 | 55,157,035 | 551 | (429) | 493,811 | (75) | (207,807) | 286,051 |
| Issuance of common stock pursuant to exercise of stock options | 12,968 | - | - | 290 | - | - | 290 |
| Sale of treasury stock | - | - | 538 | - | - | - | 538 |
| Issuance of treasury stock pursuant to exercise of stock options | - | - | (109) | - | - | - | (109) |
| Unrealized comprehensive gain on | - | - | - | - | 304 | - | 304 |

| | | | | | | | |
|---|---|---|---|---|---|---|---|
| investments | | | | | | | |
| Share-based compensation | - | - | - | 4,489 | - | - | 4,489 |
| Net loss | - | - | - | - | - | (25,040) | (25,040) |
| **Balance at June 30, 2020** | 55,170,003 | $ 551 | $ - | $ 498,590 | $ 229 | $ (232,847) | $ 266,523 |

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

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

| | Six Months Ended June 30, 2021 | 2020 |
|---|---|---|
| **Operating Activities:** | | |
| Net loss | $ (74,704) | $ (49,704) |
| Adjustments to reconcile net loss to net cash used in operating activities: | | |
| Accretion of discount on convertible notes | 705 | 1,416 |
| Depreciation and amortization expense | 1,380 | 242 |
| Write down of property and equipment | - | 62 |
| Stock-based compensation | 15,211 | 8,450 |
| Accretion of discount on investments, net | 1,345 | 63 |
| Unrealized loss on marketable securities | 1 | - |
| **Changes in operating assets and liabilities:** | | |
| Prepaid expenses and other assets | 1,030 | (2,522) |
| Accounts payable and accrued expenses | (8,982) | (3,188) |
| Operating lease liabilities | 50 | (94) |
| Finance lease liability | 1,184 | 997 |
| Other long term liabilities | (29) | - |
| Net cash used in operating activities | (62,809) | (44,278) |
| **Investing activities:** | | |
| Purchases of investments | (158,571) | (56,436) |
| Proceeds from maturities of investments | 180,584 | 88,714 |
| Payments made to acquire right of use asset | (61) | (2,731) |
| Purchases of property and equipment | (1,449) | (7,115) |
| Purchases of internal use software | (325) | (368) |
| Net cash provided by investing activities | 20,178 | 22,064 |
| **Financing activities:** | | |
| Issuance of common stock, pursuant to exercise of stock options | 9,907 | 3 |
| Issuance of common stock, net of issuance costs | - | 287 |
| Common stock repurchase | - | (72) |
| Proceeds from sale of treasury stock | - | 591 |
| Payment of withholding tax on option exercise | - | (538) |
| Convertible notes refinancing costs to the lender | - | (237) |
| Net cash provided by financing activities | 9,907 | 34 |
| Net change in cash, cash equivalents and restricted cash | (32,724) | (22,180) |
| Cash, cash equivalents and restricted cash at beginning of period | 298,666 | 186,908 |
| Cash, cash equivalents and restricted cash at end of period | $ 265,942 | $ 164,728 |
| | | |
| **Supplemental disclosure of non-cash financing and investing activities:** | | |
| Accrued purchases of property and equipment | $ 1,272 | $ 3,958 |
| Accrued purchases of internal use software | $ 122 | $ 122 |
| Unrealized (loss) gain on investments | $ (39) | $ 209 |
| Conversion of convertible notes into common stock | $ 35,544 | $ - |
| Finance lease right of use asset and lease liability | $ - | $ 20,179 |
| Reclassification of construction in process (from) to finance right of use asset | $ (5) | $ 26,465 |
| **Supplemental cash flow information:** | | |
| Cash paid for interest | $ 1,347 | $ 1,495 |

*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. Rocket has four clinical-stage *ex vivo* lentiviral vector ("LVV") programs. These include programs for Fanconi Anemia ("FA"), a genetic defect in the bone marrow that reduces production of blood cells or promotes the production of faulty blood cells, Leukocyte Adhesion Deficiency-I ("LAD-I"), a genetic disorder that causes the immune system to malfunction, Pyruvate Kinase Deficiency ("PKD"), a rare red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia and Infantile Malignant Osteopetrosis ("IMO"), a genetic disorder characterized by increased bone density and bone mass secondary to impaired bone resorption. Of these, both the Phase 2 FA program and the Phase 1/2 LAD-I program are in registration-enabling studies in the United States ("U.S.") and Europe ("EU"). In addition, in the U.S., Rocket has a clinical stage *in vivo* adeno-associated virus ("AAV") program for Danon disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. Additional discovery efforts on a gene therapy program for the less common FA subtypes C and G is ongoing. The Company has global commercialization and development rights to all of these product candidates under royalty-bearing license agreements.

## 2. Risks and Liquidity

The Company has not generated any revenue and has incurred losses since inception. The Company's operations are subject to certain risks and uncertainties, including, among others, uncertainty of drug candidate development, technological uncertainty, uncertainty regarding patents and proprietary rights, the absence of 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 $397.5 million as of June 30, 2021. As of June 30, 2021, the Company had $426.8 million of cash, cash equivalents and investments. During the year ended December 31, 2020, the Company received net proceeds of $280.8 million from a public offering of 5,339,286 shares of common stock. The Company expects such resources will be sufficient to fund its operating expenses and capital expenditure requirements into the second half of 2023. In April 2021, the Company called for the redemption of the remaining $38.4 million principal balance of its 6.25%, 2022 Convertible Senior Notes due 2022 which were converted into common stock (see Note 7). On August 2, 2021, holders of $5.15 million of the 2021 Convertible Notes converted the $5.15 million remaining balance of the 2021 Convertible Notes into common stock. As of August 2, 2021, none of the 2021 Convertible Notes or 2022 Convertible Notes were outstanding.

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, 2020 included in the Annual Report on Form 10-K filed with the Securities and Exchange Commission ("SEC") on March 1, 2021 ("2020 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 June 30, 2021 and the results of its operations and its cash flows for the three and six months ended June 30, 2021 and 2020. The financial data and other information disclosed in these consolidated notes related to the three and six months ended June 30, 2021 and 2020 are unaudited. The results for the three and six months ended June 30, 2021 are not necessarily indicative of results to be expected for the year ending December 31, 2021 and any other interim periods or any future year or period.

*Principles of Consolidation*

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

*Use of Estimates*

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

*Cash, Cash Equivalents and Restricted Cash*

Cash, cash equivalents and restricted cash consists of bank deposits, certificates of deposit and money market accounts with financial institutions. Cash equivalents are carried at cost which approximates fair value due to their short-term nature and which the Company believes do not have a material exposure to credit risk. The Company considers all highly liquid investments with maturities of three months or less from the date of purchase to be cash equivalents. The Company's cash and cash equivalent accounts, at times, exceeds 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 finance and operating leases (see Note 11 "Commitments and Contingencies" for additional disclosures) and a deposit collateralizing a letter of credit issued by a bank supporting the Company's Corporate Credit Card. Cash, cash equivalents and restricted cash consist of the following:

| | June 30, 2021 | December 31, 2020 |
|---|---|---|
| Cash and cash equivalents | $ 264,608 | $ 297,098 |
| Restricted cash | 1,334 | 1,568 |
| | $ 265,942 | $ 298,666 |

*Significant Accounting Policies*

The significant accounting policies used in the preparation of these consolidated financial statements for the three and six months ended June 30, 2021 are consistent with those disclosed in Note 3 to the consolidated financial statements in the 2020 Form 10-K, except as noted below.

*NYS Life Sciences Research and Development Tax Credit*

New York State allows investors and owners of emerging technology companies focused on biotechnology to claim a tax credit against their New York State Tax return for certain expenditures incurred in New York State, including applicable R&D related expenditures. The credit is recognized as research and development incentives when the eligibility and amount has been approved by New York State. During the three and six months ended June 30, 2021, the Company recorded research and development incentive income of $0 and $0.5 million, respectively. During the three and six months ended June 30, 2020, the Company recorded research and development incentive income of $0.

*Recent Accounting Pronouncements*

In June 2016, the FASB issued ASU 2016-13, Financial Instruments – Credit Losses (Topic 362): Measurement of Credit Losses on Financial Statements ("ASU 2016-13"). The new standard requires that expected credit losses relating to financial assets measured on an amortized cost basis and available-for-sale debt securities be recorded through an allowance for credit losses. It also limits the amount of credit losses to be recognized for available-for-sale debt securities to the amount by which carrying value exceeds fair value and also requires the reversal of previously recognized credit losses if fair value increases. The targeted transition relief standard allows filers an option to irrevocably elect the fair value option of ASC 825-10, Financial Instruments-Overall, applied on an instrument-by-instrument basis for eligible instruments. The new standard was effective beginning January 1, 2021. The adoption of ASU 2016-13, and related updates, did not have a material impact on the Company's consolidated financial position and results of operations.

There were no other recent accounting pronouncements that impacted the Company or are expected to have 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 June 30, 2021 Using: | | | |
|---|---|---|---|---|
| | Level 1 | Level 2 | Level 3 | Total |
| Assets: | | | | |
| Cash and cash equivalents: | | | | |
| Money market mutual funds | $ 221,680 | $ - | $ - | $ 221,680 |
| | 221,680 | - | - | 221,680 |
| | | | | |
| Investments: | | | | |
| United States Treasury securities | 74,468 | - | - | 74,468 |
| Corporate Bonds | - | 72,435 | - | 72,435 |
| Municipal Bonds | - | 6,000 | - | 6,000 |
| Agency Bonds | - | 9,319 | - | 9,319 |
| | 74,468 | 87,754 | - | 162,222 |
| | | | | |
| | $ 296,148 | $ 87,754 | $ - | $ 383,902 |

| | Fair Value Measurements as of December 31, 2020 Using: | | | |
|---|---|---|---|---|
| | Level 1 | Level 2 | Level 3 | Total |
| Assets: | | | | |
| Cash and cash equivalents: | | | | |
| Money market mutual funds | $ 193,312 | $ - | $ - | $ 193,312 |
| United States Treasury securities | 62,497 | - | - | 62,497 |
| Corporate Bonds | - | 501 | - | 501 |
| Agency Bonds | - | 8,015 | - | 8,015 |
| | 255,809 | 8,516 | - | 264,325 |
| | | | | |
| Investments: | | | | |
| United States Treasury securities | 112,328 | - | - | 112,328 |
| Corporate Bonds | - | 63,710 | - | 63,710 |
| Municipal Bonds | - | 6,000 | - | 6,000 |
| Agency Bonds | - | 3,583 | - | 3,583 |
| | 112,328 | 73,293 | - | 185,621 |
| | | | | |
| | $ 368,137 | $ 81,809 | $ - | $ 449,946 |

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

The fair value of the 2021 Convertible Notes and 2022 Convertible Notes as of June 30, 2021 was $7.5 million and $0, respectively (see Note 7).

The fair value of the 2021 Convertible Notes and 2022 Convertible Notes as of December 31, 2020 was $9.5 million and $65.6 million, respectively (see Note 7).

## 5. Property and Equipment, Net

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

| | June 30, 2021 | December 31, 2020 |
|---|---|---|
| Laboratory equipment | $ 10,401 | $ 7,807 |
| Machinery and equipment | 10,012 | 9,933 |
| Computer equipment | 218 | 218 |
| Furniture and fixtures | 1,914 | 1,880 |
| Leasehold improvements | 39 | 29 |
| Internal use software | 1,832 | 1,385 |
| | 24,416 | 21,252 |
| Less: accumulated depreciation and amortization | (3,427) | (2,046) |
| | $ 20,989 | $ 19,206 |

During the three and six months ended June 30, 2021, the Company recognized $0.7 million and $1.4 million of depreciation and amortization expense, respectively. During the three and six months ended June 30, 2020, the Company recognized $0.1 million and $0.2 million of depreciation and amortization expense, respectively.

## 6. Accounts Payable and Accrued Expenses

At June 30, 2021 and December 31, 2020, the Company's accounts payable and accrued expenses consisted of the following:

| | June 30, 2021 | December 31, 2020 |
|---|---|---|
| Research and development | $ 11,251 | $ 14,962 |
| Property and equipment | 1,272 | 1,456 |
| Employee compensation | 3,115 | 4,875 |
| Accrued interest | 123 | 1,122 |
| Government grant payable | 597 | 590 |
| Professional fees | 735 | 1,332 |
| Internal use software | 122 | 300 |
| Other | 669 | 835 |
| | $ 17,884 | $ 25,472 |

## 7. Convertibles Notes

### 2021 Convertible Notes

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

The 2021 Convertible Notes are considered a hybrid financial instrument consisting of a fixed interest rate "host" and various embedded features that required evaluation as potential embedded derivatives under FASB ASC 815, *Derivatives and Hedging* ("ASC 815"). Based on the nature of the host instrument and the embedded features, management concluded that none of the conversion, put and redemption features required bifurcation and separate accounting from the host instrument. The Company determined that the Additional Interest was an embedded derivative that contains non-credit related events of default. As a result, the Additional Interest feature required bifurcation and separate accounting under ASC 815. Based on the amount of Additional Interest that would be owed and the likelihood of occurrence, the Company estimated the fair value of the Additional Interest feature to be insignificant upon issuance and as of June 30, 2021 and December 31, 2020. As of June 30, 2021, the stated interest rate was 5.75%, and the effective interest rate was 15.3%.

On August 2, 2021, holders of $5.15 million of the 2021 Convertible Notes converted the $5.15 million remaining balance of the 2021 Convertible Notes into 160,614 shares. As of August 2, 2021, none of the 2021 Convertible Notes were outstanding.

### *2022 Convertible Notes*

On February 20, 2020, and June 5, 2020, the Company entered into separate, privately negotiated exchange agreements (the "Exchange Agreements") with certain holders of the 2021 Convertible Notes. Pursuant to the Exchange Agreements, on February 20, 2020, the Company exchanged approximately $39.35 million aggregate principal amount of the 2021 Convertible Notes (representing approximately 76% of the aggregate outstanding principal amount of the 2021 Convertible Notes) for (a) approximately $39.35 million aggregate principal amount of 6.25% Convertible Senior Notes due August 2022 (the "2022 Convertible Notes") (an exchange ratio equal to 1.00 2022 Convertible Note per exchanged 2021 Convertible Note) and (b) $0.1 million to pay the accrued and unpaid interest on the exchanged 2021 Convertible Notes from February 1, 2020, to February 20, 2020, the closing date of the February 20, 2020 exchange transactions. Additionally, the Company repurchased 3,000 shares of its common stock that have been retired for an aggregate amount of $71,670 from certain holders of the 2021 Convertible Notes participating in the exchange transactions in privately negotiated, private transactions.

Also pursuant to the Exchange Agreements, on June 12, 2020, the Company exchanged $7.5 million aggregate principal amount of the 2021 Convertible Notes for (a) $7.5 million aggregate principal amount of its newly issued 6.25% Convertible Senior Notes due 2022 (an exchange ratio equal to 1.00 2022 Convertible Notes per exchanged 2021 Convertible Notes) and (b) approximately $11,000 to pay the accrued and unpaid interest on the exchanged 2021 Convertible Notes from, and including, February 1, 2020, to, but excluding, the closing date of the exchange transaction, adjusted to take into account the unearned accrued interest on the 2022 Convertible Notes from, and including, February 20, 2020, to, but excluding, the closing date of the exchange transaction.

The 2022 Convertible Notes were issued in a private placement exempt from registration in reliance on Section 4(a)(2) of the Securities Act of 1933, as amended (the "Securities Act"). The 2022 Convertible Notes issued in the exchange transaction were issued as additional notes pursuant to the Indenture, dated as of August 5, 2016, between the Company and Wilmington Trust, National Association, as trustee, as supplemented by the Second Supplemental Indenture, dated as of February 20, 2020, governing the 2022 Convertible Notes.

The conversion rate for the 2022 Convertible Notes was initially 31.1876 shares of the Company's common stock per 1.00 principal amount of 2022 Convertible Notes, which was equivalent to an initial conversion price of approximately $32.06 per share of common stock and was subject to adjustment under the terms of the 2022 Convertible Notes. The Company may have redeemed for cash all or any portion of the 2022 Convertible Notes, at its option, if the last reported sale price of its common stock was equal to or greater than 130% of the conversion price then in effect for at least 20 trading days (whether or not consecutive) during any 30 consecutive trading day period (including the last trading day of such period) ending within the five trading days immediately preceding the date on which the Company provided written notice of redemption. The 2022 Convertible Notes Indenture contained customary terms and covenants and events of default.

In December 2020, $8.5 million principal amount, representing a carrying value of $7.6 million of the 2022 Convertible Notes was converted into 298,562 shares of the Company's common stock.

On April 26, 2021, the Company called for the redemption of the remaining $38.4 million principal balance of the 2022 Convertible Notes as the Company's stock price traded above the Conversion Rate for at least 20 trading days during a 30-day consecutive trading period. On April 26, 2021, the Company redeemed in full the 2022 Convertible Notes prior to the redemption date. Holders of approximately $38.4 remaining million principal amount of the 2022 Convertible Notes converted such notes in accordance with the terms of the Exchange Agreements into approximately 1.3 million shares of the Company's common stock and cash in lieu of fractional shares. In accordance with ASC 470-*Debt*, the settlement of the 2022 Convertible Notes is accounted for as a conversion since the 2022 Convertible Notes did not include a beneficial conversion feature and the carrying amount of the 2022 Convertible Notes, including any unamortized premium or discount, was credited to additional paid in capital upon conversion to reflect the common stock issued and no gain or loss is recognized. The principal amount of $38.4 million and any unamortized discount was recorded to additional paid in capital upon redemption.

The table below summarizes the carrying value of the 2021 Convertible Notes as of June 30, 2021 and December 31, 2020:

| | 2021 Notes | |
|---|---|---|
| | **June 30, 2021** | **December 31, 2020** |
| Principal amount | $ 5,150 | $ 5,150 |
| Discount | (48) | (275) |
| Carrying value | $ 5,102 | $ 4,875 |

Accretion of the 2021 Convertible Notes discount was $0.1 million and $0.2 million for the three and six months ended June 30, 2021, respectively. Accretion of the 2021 Convertible Notes discount was $0.2 million and $0.8 million for the three and six months ended June 30, 2020, respectively. Accretion of the 2022 Convertible Notes discount was $0 and $0.5 million for the three and six months ended June 30, 2021, respectively. Accretion of the 2022 Convertible Notes discount was $0.5 million and $0.6 million for the three and six months ended June 30, 2020, respectively.

## 8. Stock Based Compensation

*Stock Option Valuation*

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

| | Six Months Ended June 30, 2021 | 2020 |
|---|---|---|
| Risk-free interest rate | 0.74% | 1.27% |
| Expected term (in years) | 5.84 | 5.84 |
| Expected volatility | 69.19% | 77.39% |
| Expected dividend yield | 0.00% | 0.00% |
| Exercise price | $ 57.18 | $ 21.56 |
| Fair value of common stock | $ 57.18 | $ 21.56 |

The following table summarizes stock option activity for the six months ended June 30, 2021 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, 2020 | 11,050,931 | $ 9.10 | 6.55 | $ 504,079 |
| Granted | 1,243,509 | 57.18 | 9.32 | |
| Exercised | (1,125,270) | 8.76 | | 53,345 |
| Cancelled | (128,473) | 34.15 | | |
| Outstanding as of June 30, 2021 | 11,040,697 | $ 14.27 | 6.40 | $ 346,476 |
| | | | | |
| Options vested and exercisable as of June 30, 2021 | 8,127,520 | $ 6.27 | 5.43 | $ 308,590 |
| Options unvested as of June 30, 2021 | 2,913,177 | $ 36.57 | 9.11 | |

The weighted average grant-date fair value per share of stock options granted during the six months ended June 30, 2021 and 2020 was $34.64 and $14.29, respectively.

The total fair value of options vested during the six months ended June 30, 2021 and 2020 was $13.2 million and $30.0 million, respectively.

*Stock-Based Compensation*

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

| | Three Months Ended June 30, 2021 | 2020 | Six Months Ended June 30, 2021 | 2020 |
|---|---|---|---|---|
| Stock options | $ 7,215 | $ 4,489 | $ 15,041 | $ 8,450 |
| Restricted stock units | 96 | - | 170 | - |
| Total share based compensation expense | $ 7,311 | $ 4,489 | $ 15,211 | $ 8,450 |

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

| | Three Months Ended June 30, 2021 | 2020 | Six Months Ended June 30, 2021 | 2020 |
|---|---|---|---|---|
| Research and development | $ 3,148 | $ 1,679 | $ 6,064 | $ 3,414 |
| General and administrative | 4,163 | 2,810 | 9,147 | 5,036 |
| Total share based compensation expense | $ 7,311 | $ 4,489 | $ 15,211 | $ 8,450 |

*Restricted Stock Units (“RSU”)*

The following table summarizes the Company's RSU activity for the six months ended June 30, 2021:

| | Number of Shares | Weighted Average Grant Date Fair Value |
|---|---|---|
| Unvested as of December 31, 2020 | 20,000 | $ 25.06 |
| Granted | 3,500 | 62.32 |
| Unvested as of June 30, 2021 | 23,500 | 30.61 |

As of June 30, 2021, the Company had an aggregate of $57.0 million of unrecognized stock-based compensation expense related to both stock options and Restricted Stock Unit grants, which is expected to be recognized over the weighted average period of 1.97 years.

## 9. Warrants

As of June 30, 2021 and December 31, 2020, the Company had warrants outstanding convertible into 7,051 and 603,386 shares of common stock at exercise price of $24.82 and $57.11 per share, respectively, which warrants will expire on June 28, 2023 and December 21, 2030, respectively.

## 10. Net Loss Per Share

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

| | Three Months Ended June 30, | | Six Months Ended June 30, | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| Numerator: | | | | |
| Net loss attributable to common stockholders | $ (34,525) | $ (25,040) | $ (74,704) | $ (49,704) |
| Denominator: | | | | |
| Weighted-average common shares outstanding - basic and diluted | 63,061,232 | 55,158,459 | 62,321,926 | 55,020,789 |
| Net loss per share attributable to common stockholders - basic and diluted | $ (0.55) | $ (0.45) | $ (1.20) | $ (0.90) |

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

| | Three and Six Months Ended June 30, | |
|---|---|---|
| | 2021 | 2020 |
| Shares issuable upon conversion of the 2021 Convertible Notes | 160,536 | 160,536 |
| Shares issuable upon conversion of the 2022 Convertible Notes | - | 1,460,412 |
| Warrants exercisable for common shares | 610,437 | 14,102 |
| Restricted stock units exercisable for common shares | 23,500 | - |
| Options to purchase common shares | 11,040,697 | 10,776,813 |
| | 11,835,170 | 12,411,863 |

## 11. 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). 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. A smaller area within this facility was originally leased in August 2018, and the lease was amended in June 2019 to include the full building (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 NJ Lease Agreement is estimated to be approximately $29.3 million over the 15-year term of the NJ Lease Agreement. 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 June 30, 2021 and December 31, 2020.

The Company determined the lease commencement date was reached on March 15, 2020 when the construction of all landlord owned improvements had been substantially completed and when the Company began including its leasehold improvements on the balance sheet and move equipment into the space. Upon commencement of the NJ Lease Agreement in March 2020, the Company recognized total right-of-use assets of $47.7 million, with a corresponding lease liability of $20.2 million. The Company reclassified $26.5 million of construction costs in progress and $1.1 million of prepaid rent as part of the right of use asset upon the lease commencement date of March 15, 2020. During the six months ended June 30, 2021, the Company reclassified an additional $0.1 million bringing the aggregate reclassification to $32.2 million of construction costs in progress as part of the right of use asset.

Interest associated with the financing lease was $0.5 million and $0.9 million for the three and six months ended June 30, 2021, respectively. Interest associated with the financing lease was $0.5 million and $0.6 million for the three and six months ended June 30, 2020, respectively. This is recorded as interest expense on the consolidated statements of operations.

*Operating Leases*

On June 7, 2018, the Company entered into a three-year lease agreement for office space in the Empire State Building in New York, NY (the "ESB Lease Agreement"). In connection with the ESB Lease Agreement, the Company established an irrevocable standby letter of credit (the "Empire LOC") for $0.9 million. On March 26, 2021, the Company entered in Amendment No. 1 to the ESB Lease Agreement ("ESB Lease Amendment") that extended the term of the lease agreement to June 30, 2024, reduced the rent payments going forward, and reduced the Empire LOC to $0.8 million. The Empire LOC

serves as the Company's security deposit on the lease in which the landlord is the beneficiary and expires August 29, 2024. The Company has accounted for the ESB Lease Amendment as a modification to the ESB Lease Agreement and remeasured the lease liability and adjusted the operating lease right of use asset by $1.1 million.

The Company has a certificate of deposit of $0.8 million and $0.9 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 June 30, 2021 and December 31, 2020, respectively.

On January 4, 2018, in connection with the Reverse Merger, the Company assumed an operating lease for Inotek's former headquarters in Lexington, Massachusetts, with a term ending in February 2023. In July 2018, the Company signed an agreement to sublease a portion of the Lexington, Massachusetts space and in September 2018, the Company signed an agreement to sublease the remaining portion of the Lexington, Massachusetts space. Rental income received under the sublease agreement totaled $0.1 million and $0.2 million for the three and six months ended June 30, 2021, respectively. Rental income received under the same agreement totaled $0.1 million and $0.2 million for the three and six months ended June 30, 2020, respectively. Rental income is netted against rent expense in the consolidated statement of operations.

Rent expense was $0.3 million and $0.6 million for the three and six months ended June 30, 2021, respectively. Rent expense was $0.3 million and $0.5 million for the three and six months ended June 30, 2020, respectively.

The total restricted cash balance for the Company's operating and finance leases at June 30, 2021 and December 31, 2020 was $1.0 million and $1.1 million, respectively.

| **Lease cost** | **June 30, 2021** |
|---|---|
| Operating lease cost | $ 275 |
| Finance lease cost | |
| Amortization of right of use assets | 1,071 |
| Interest on lease liablities | 920 |
| Total lease cost | $ 2,266 |

The following table summarizes the maturity of the Company's operating and finance lease liabilities on an undiscounted cash flow basis and a reconciliation to the operating and finance lease liabilities as of June 30, 2021:

| **Maturity of operating lease liabilities** | **June 30, 2021** |
|---|---|
| 2021 | $ 428 |
| 2022 | 860 |
| 2023 | 488 |
| 2024 | 207 |
| Total lease payments | $ 1,983 |
| Less: interest | (95) |
| Total operating lease liabilities | $ 1,888 |

| **Maturity of finance lease liability** | **June 30, 2021** |
|---|---|
| 2021 | $ 827 |
| 2022 | 1,689 |
| 2023 | 1,736 |
| 2024 | 1,791 |
| 2025 | 1,856 |
| Thereafter | 46,913 |
| Total lease payments | $ 54,812 |
| Less: interest | (34,076) |
| Total finance lease liability | $ 20,736 |

| Leases | June 30, 2021 |
|---|---|
| Operating right-of-use assets | $ 1,628 |
| Operating current lease liabilities | 792 |
| Operating noncurrent lease liabilities | 1,096 |
| Total operating lease liabilities | $ 1,888 |
| Finance right-of-use assets | $ 49,507 |
| Finance current lease liability | 1,666 |
| Finance noncurrent lease liability | 19,070 |
| Total finance lease liability | $ 20,736 |

**Other information**

| Cash paid for amounts included in the measurement of lease liabilities: | |
|---|---|
| Operating cash flows from operating leases | $ 225 |
| Cash flows from finance lease | $ 816 |
| Weighted-average remaining lease term - operating leases | 2.5 years |
| Weighted-average remaining lease term - finance lease | 23.2 years |
| Weighted-average discount rate - operating leases | 4.75% |
| Weighted-average discount rate - finance lease | 8.96% |

*Litigation*

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

*Indemnification Arrangements*

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

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

## 12. Agreements Related to Intellectual Property

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

## 13. CIRM Grants

*LAD-I CIRM Grant*

On April 30, 2019, the California Institute for Regenerative Medicine ("CIRM") awarded the Company up to $6.5 million under a CLIN2 grant award to support the clinical development of gene therapy for LAD-I. Proceeds from the grant will help fund clinical trial costs as well as manufactured drug product for Phase I/II patients enrolled at the U.S. clinical site, University of California, Los Angeles ("UCLA") Mattel Children's Hospital, led by principal investigator Donald Kohn, M.D., UCLA Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics (Hematology/Oncology), Molecular and Medical Pharmacology and member of the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at UCLA. In 2019, the Company received the first two grants from CIRM in the aggregate of $1.2 million, based on eligible costs incurred under the grant. The CIRM grant reimbursements are accrued as an offset against R&D expenses as reimbursable expenses are incurred. As of December 31, 2020, the Company met the next CIRM milestone and recorded a receivable, included in prepaid and other assets in the consolidated balance sheet, and a reduction of research and development expenses of $1.1 million. The Company received the $1.1 million milestone payment on January 4, 2021. As of March 31, 2021, the Company met the next CIRM milestone and recorded a receivable, included in prepaid and other assets in the consolidated balance sheet, and a reduction of research and development expenses of $1.0 million. The Company received the $1.0 million milestone payment on April 1, 2021.

*IMO CIRM Grant*

On November 12, 2020, the CIRM awarded the Company up to $3.7 million under a CLIN2 grant award to support the clinical development of its lentiviral vector (LVV)-based gene therapy, RP-L401, for the treatment of IMO. The Company received a $1.0 million pursuant to the grant on January 4, 2021 related to the CIRM IMO award and recorded a receivable, included in prepaid and other assets in the consolidated balance sheet, and a reduction of research and development expenses of $0.9 million as of December 31, 2020. The Company recorded a reduction of research and development expense of $0.1 million for the three and six months ended June 30, 2021.

**14. Related Party Transactions**

During April 2018, the Company entered into an agreement with a member of the Board of Directors for business development consulting services. Payments for the services under the agreement are $27.5 per quarter, and the Company may terminate the agreement with 14 days' notice. The Company incurred expenses of $55 and $27.5 during the three and six months ended June 30, 2021 and 2020, relating to services provided under this agreement.

In June 2020, the Company entered into a consulting agreement with a member of the Board of Directors for pipeline development, new asset evaluation, and corporate strategy. In lieu of cash for services to be provided under the consulting agreement during its one-year term, the Company granted the board member options to purchase 9,784 options with a fair value of $0.1 million.

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.

In December 2020, the Company entered into a consulting agreement with a related party. Pursuant to the consulting agreement, the related party provides certain business development and asset identification consulting services to the Company. The term of the consulting agreement is three years and may be terminated with 60 days' notice by either party. In exchange for the business development services to be provided under the agreement, the Company issued a warrant exercisable for 603,386 shares of common stock. Pursuant to the consulting agreement, the related party is entitled to receive additional warrants exercisable for common stock upon identification of new assets for the Company to in-license. There were no warrants issued for the six months ended June 30, 2021.

**15. 401(k) Savings Plan**

The Company has a defined contribution savings plan (the "Plan") under Section 401(k) of the Internal Revenue Code of 1986. This Plan covers substantially all employees who meet minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre-tax basis. Company contributions to the Plan may be made at the discretion of the Company's Board of Directors. The Company has elected to the safe harbor match of 4% of employee contributions to the Plan, subject to certain limitations. The Company's matching contribution for the three and six months ended June 30, 2021 was $0.1 million and $0.3 million, respectively. The Company's matching contribution for the three and six months ended June 30, 2020 was $0.1 million and $0.2 million, respectively.

**16. Subsequent Events**

On August 2, 2021, holders of $5.15 million of the 2021 Convertible Notes converted the $5.15 million remaining balance of the 2021 Convertible Notes into 160,614 common shares. As of August 2, 2021, none of the 2021 Convertible Notes were outstanding.

On August 9, 2021, the Company issued a warrant exercisable for 301,291 shares of common stock to a related party for business development and asset identification consulting services.

## 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 for the fiscal year ended December 31, 2020 filed with the U.S. Securities and Exchange Commission, or the SEC, on March 1, 2021 ("2020 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 2020 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 four clinical-stage *ex vivo* lentiviral vector ("LVV"). 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, Pyruvate Kinase Deficiency ("PKD"), a rare red blood cell autosomal recessive disorder that results in chronic non-spherocytic hemolytic anemia and Infantile Malignant Osteopetrosis ("IMO"), a genetic disorder characterized by increased bone density and bone mass secondary to impaired bone resorption. Of these, both the Phase 2 FA program and the Phase 1/2 LAD-I program are in registration-enabling studies in the United States ("U.S.") and Europe ("EU"). In addition, in the U.S., we have a clinical stage *in vivo* adeno-associated virus ("AAV") program for Danon disease, a multi-organ lysosomal-associated disorder leading to early death due to heart failure. Additional discovery efforts on a gene therapy program for the less common FA subtypes C and G is ongoing. We have global commercialization and development rights to all of these product candidates under royalty-bearing license agreements.

**Recent Developments**

On April 26, 2021, we redeemed in full our 2022 Convertible Notes prior to the redemption date. Holders of approximately $38.4 million remaining principal amount of the 2022 Convertible Notes converted such notes into approximately 1.3 million shares of the Company's common stock and cash in lieu of fractional shares. As of June 30, 2021, none of the 2022 Convertible Notes were outstanding.

On August 2, 2021, holders of $5.15 million of the 2021 Convertible Notes converted the $5.15 million remaining balance of the 2021 Convertible Notes into 160,614 shares. As of August 2, 2021, none of the 2021 Convertible Notes were outstanding.

On May 10, 2021, we announced that the RP-A501 Danon Disease program was placed on clinical hold by the FDA. No new drug-related safety events have been observed in the low- or high-dose adult cohorts of the Phase 1 trial. The FDA requested that we pause patient dosing and modify the protocol and other supporting documents with revised guidelines for patient selection and management. All follow-up study activities will continue. We have continued our dialogue with the FDA to ensure safety measures are updated and harmonized adequately across all protocol-related documents We believe we may be able to resume the trial in the third quarter of 2021. We anticipate reporting updated clinical data results in the fourth quarter of 2021.

***Gene Therapy Overview***

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

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

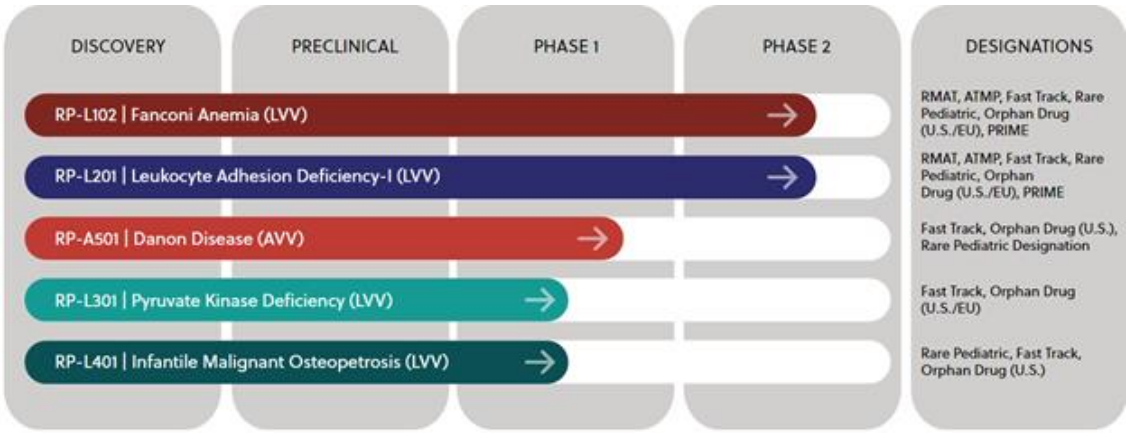
We are using modified non-pathogenic viruses for the development of our gene therapy treatments. Viruses are particularly well suited as delivery vehicles because they are adept at penetrating cells and delivering genetic material inside a cell. In creating our viral delivery vehicles, the viral (pathogenic)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

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

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

***Pipeline Overview***

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




***AAV Program***:

***Danon Disease:***

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

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

As of June 30, 2021, we have treated five patients in the RP-A501 Phase 1 clinical trial and completed the first cohort of the study evaluating an initial low-dose in male patients aged 15 or greater. The preliminary data announced in December 2020 for the low dose cohort included safety and clinical activity results from the three patients treated with the low dose of $6.7\times10^{13}$ genome copies (gc)/kilogram (kg) and early safety information from the two patients treated with the higher dose of $1.1\times10^{14}$ gc/kg as of the cutoff date of November 2020.

In the three patients treated in the low dose cohort, RP-A501 showed manageable safety results. No unexpected and serious drug product-related adverse events or severe adverse events were observed in this low dose cohort. The most common adverse events were predominantly mild, not associated with clinical symptoms and were related to elevated transaminases post treatment. Elevation in transaminases was observed in all three low-dose patients and returned to baseline levels within the first one to two months post-treatment. There was also a transient and reversible decline in platelets observed in these three patients. These changes were largely responsive to corticosteroids and other immunosuppressive therapies. All patients were given oral steroids to prevent or minimize potential immune-related events.

At the higher dose administered ($1.1\times10^{14}$ gc/kg), additional immunosuppressive therapies were stipulated and administered to mitigate the immune response associated with RP-A501. One of the two treated patients, who received the higher absolute AAV9 dose and had some degree of pre-existing anti-AAV9 immunity, experienced a non-persistent, immune-related event that was classified as a drug product-related serious adverse event. This thrombotic microangiopathy ("TMA") event (which was later reclassified as a Sudden Unexpected Serious Adverse Reaction ("SUSAR")) was believed to be likely due to immune-mediated complement activation, resulting in reversible thrombocytopenia and acute kidney injury requiring eculizumab and transient hemodialysis. This patient regained normal kidney function within three weeks.

From the perspective of gene expression results, all three low dose patients demonstrated evidence of cardiac *LAMP2B* expression by Western blot and/or immunohistochemistry. In two of the three patients in the low dose cohort who had closely monitored compliance with the immunosuppressive regimen, high levels of cardiac *LAMP2B* expression were observed along with clinical biomarker improvements. In cardiac biopsies of the low dose patients, *LAMP2B* gene expression was observed in 67.8% of cells compared to normal as determined by immunohistochemistry at 12 months in one patient, and at 92.4% of cells compared to normal at month 9 in the other patient. In this latter patient, Western blot assessment showed 61% of normal *LAMP2B* protein expression at month 9. The 12-month Western blot data was still pending for all three patients as of the data cutoff.

At least two of the three low dose patients demonstrated key clinical biomarker improvements consistent with improved cardiac function. Notably, photographic evidence for all three patients showed improvements (i.e., decrease) in autophagic vacuoles, a hallmark of Danon disease pathology, as assessed by electron microscopy of cardiac tissue via endomyocardial biopsy. Additionally, two of the three low dose patients with closely monitored immunosuppressive regimen compliance demonstrated improvement in cardiac output and brain natriuretic peptide ("BNP") and also upgraded from Class II to I in the New York Heart Association Functional Classification, which is the most commonly used heart failure classification system. Class II is where a patient exhibits a slight limitation of physical activity, are 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. In these two patients, we also observed substantial improvement of a key marker of heart failure, BNP, which decreased from a pretreatment baseline by 75 percent in one patient and 79 percent in the other as well as improvement in cardiac output by 35 percent in one patient and 62 percent in the other as measured by invasive hemodynamics. The third patient has demonstrated stabilization of NYHA class and BNP.

As disclosed in December 2020, one patient in the high dose cohort, who was the heaviest treated to date and had highly advanced disease developed complement-mediated TMA which resolved fully with transient hemodialysis (this event was later reclassified as a SUSAR). This patient continued to have progressive disease considered unrelated to gene therapy by the trial investigator as well as his transplant cardiologist and successfully received a heart transplant. The patient is currently doing well clinically and reports resolution of his baseline myopathy that was present prior to treatment. Analysis of the explanted heart demonstrated fibrosis that was consistent with end-stage Danon disease. Of note, this patient had advanced features of heart failure upon treatment; the updated trial protocol will exclude enrollment of Danon patients with end-stage disease. Data collection from this patient and the other high dose patient is ongoing with full presentation of the data from the high dose as well as low dose level expected to be presented in the fourth quarter.

Given the activity observed in the low dose cohort and to mitigate TMA and associated safety concerns observed in the high dose cohort, in agreement with the FDA, we will focus on the low dose moving forward (6.7e13) and will no longer administer the higher doses (1.1e14 or higher) in this trial. All follow-up study activities will continue, and additional safety measures have been implemented and will be reflected in the updated trial protocol once finalized with the FDA. We are working closely with the FDA and will provide an update when available.

***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.

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

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

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

We expect to report longer-term follow up on these patients in the fourth quarter of 2021.

***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 is serving as the lead clinical site in Spain. GOSH in London is also a site for the LAD-I trial. This study has received a $6.5 million CLIN2 grant award from the California Institute for Regenerative Medicine ("CIRM") to support the clinical development of gene therapy for LAD-I.

The ongoing open-label, single-arm, Phase 1/2 registration enabling clinical trial of RP-L201 has treated four 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 this study.

In April 2021, we presented positive clinical updates from RP-L201 at the Clinical Immunology Society (CIS) Annual Meeting. The Phase 1/2 data presented in a poster at CIS 2021 are from four pediatric patients with severe LAD-I. RP-L201 was well tolerated with no safety issues reported with treatment or post-treatment. All four patients achieved hematopoietic reconstitution within 5-weeks and demonstrated CD18 expression substantially exceeding the 4-10% threshold associated with survival into adulthood. The first patient with 18-months follow up demonstrated durable CD18 expression of ~40%, peripheral blood vector copy number (VCN) levels of 1.2 at 12-months post-treatment and resolution of skin lesions with no new lesions. The second patient with 9-months of follow up demonstrated CD18 expression of ~28% and peripheral blood VCN levels of 0.75 at 6-months post-treatment with kinetics consistent with those of the first patient. The third and fourth patients demonstrated high CD18 expression of ~70% and ~51%, respectively at 3-months post treatment, and peripheral blood VCN kinetics consistent with those of the first two patients.

The LAD-I program received Regenerative Medicine Advanced Therapy designation from the FDA and Priority Medicines designation from the European Medicines Agency ("EMA"), completing the full complement of all U.S. and EU accelerated regulatory designations for the program.

In May 2021, we presented clinical data at the 24$^{th}$ Annual Meeting of ASGCT. The phase 1/2 data presented in an oral presentation at ASGCT are from four pediatric patients with severe LAD-I. RP-L201 showed preliminary efficacy in all four patients, including one patient with 18-months of follow-up and one patient with 9-months of follow-up. All four patients demonstrated CD18 expression substantially exceeding the 4-10% threshold associated with survival into adulthood and consistent with the reversal of severe LAD-I phenotype. Most importantly, all four patients were able to leave the hospital in the weeks following RP-L201 therapy and all have been at home without any serious or severe infections following hospital discharge. RP-L201 was well tolerated with no safety issues reported with treatment or post-treatment.

We expect to announce initial Phase 2 data for our LAD-I program in the fourth quarter of 2021.

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

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

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

This global Phase 1 open-label, single-arm, clinical trial is expected to enroll six adult and pediatric transfusion-dependent PKD patients in the U.S. and Europe. The trial will be comprised of three cohorts to assess RP-L301 in young pediatric (age 8-11), older pediatric (age 12-17) 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.

The data presented at the 2020 ASH Annual Meeting were from two adult PKD patients with significant anemia and transfusion requirements. Patient L301-006-1001 was treated with RP-L301. Preliminary data from this first patient supported initial tolerability of RP-L301, hemoglobin improvement to a normal range at 3-months post treatment and additional normalization of hemolysis markers. The patient was 31-years of age at the time of enrollment and had been followed for 3-months post treatment as of the data cutoff date of October 2020.

Patient L301-006-1001 received a cell dose of $3.9x10^6$ cells/kilogram ("kg") with a drug product mean VCN of 2.73. For this patient, hematopoietic reconstitution was observed in less than two weeks. Furthermore, the patient attained peripheral blood VCN of 2.21 at 1-month and 1.55 at 3-months and normalized hemoglobin ("Hgb") and hemolysis markers at 3-months post-treatment. In particular, at baseline, the patient had Hb of approximately 7.4 grams ("g")/deciliter ("dL") to Hb of 14.3 g/dL at 3-months post treatment with RP-L301. In the two years prior to enrollment, the patient underwent approximately 14 transfusion episodes; subsequent to engraftment from RP-L301 treatment, the patient to date has not required any red blood cell transfusions. The patient also exhibited normalization of bilirubin, lactate dehydrogenase and erythropoietin levels at 3-months post treatment, each of which had been substantially elevated prior to study enrollment. The patient also had an increase in hepcidin and a decrease in reticulocytes at 3-months post treatment.

The data from Patient L301-006-1001 indicated that RP-L301 was generally well-tolerated and there were no serious safety issues or infusion-related complications observed 3-months post treatment. The patient experienced Grade 3 treatment-emergent adverse events of neutropenia, stomatitis, increased liver transaminase levels (AST and ALT) and a Grade 4 treatment-emergent adverse event of hypertriglyceridemia; the investigator did not consider these adverse events related to RP-L301.

Patient L301-006-1001 also experienced Grade 2 serious adverse events of chest pain, dyspnea, and nausea during the apheresis collection. The investigator considered these events related to apheresis, hyperleukocytosis and the mobilizing agents. They resolved with supportive care and without sequelae. Other events included Grade 2 bone pain and Grade 3 leukocytosis.

A second patient L301-006-1002, was 47 years old at the time of enrollment and had been recently treated with RP-L301, receiving a cell dose of $2.4x10^6$ cells/kg with a mean drug product VCN of 2.08.

In March 2021, we announced updated positive preliminary clinical data from Phase 1 trial of RP-L301 for the treatment of PKD. The updated preliminary Phase 1 RP-L301 data were from two patients that showed sustained safety and tolerability 6- and 3-months after treatment, respectively. The two patients demonstrated durable normalization of hemoglobin levels from an average baseline of ~7.4 to 13.9 g/dL at 6-months post treatment in the first patient and from a baseline of ~7.0 g/dL to 13.8 g/dL at 3-months post treatment in the second patient. Respectively, the two patients demonstrated significant improvements in bilirubin 6- and 3-months after treatment, which had been substantially elevated prior to study enrollment.

In May 2021, we presented positive clinical data at the $24^{th}$ Annual Meeting of ASGCT. Updated preliminary Phase 1 data presented in an oral presentation at ASGCT were from two patients with significant anemia and transfusion requirements that showed sustained safety and tolerability. Preliminary efficacy, measured by peripheral blood VCN levels, was evident in both patients during the initial 9-months and 3-months post-treatment, respectively. The two patients demonstrated durable normalization of hemoglobin levels, from an average baseline of ~7.4 grams (g)/deciliter (dL) to 13.1 g/dL at 9-months post treatment in the first patient and from a baseline of ~7.0 g/dL to 14.4 g/dL at 6-months post treatment in the second patient. The second cohort of this study will enroll older pediatric patients and is expected to be initiated in the second half of 2021.

The Phase 1 trial continues to enroll patients with longer-term data on track for the fourth quarter.

***Infantile Malignant Osteopetrosis (IMO):***

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

IMO represents the autosomal recessive, severe variants of a group of disorders characterized by increased bone density and bone mass secondary to impaired bone resorption. IMO typically presents in the first year of life and is associated with severe bone and hematologic manifestations leading to death within the first decade of life in the absence of allogeneic HSCT, although HSCT results have been limited to-date and notable for frequent graft failure, GVHD and other severe complications including pneumonitis and pulmonary hypertension.

Approximately 50% of IMO results from mutations in the TCIRG1 gene, resulting in cellular defects that prevent osteoclast bone resorption. As a result of this defect, bone growth is markedly abnormal. It is estimated that IMO occurs in 1 out of 250,000-300,000 within the general global population, although incidence is higher in specific geographic regions including Costa Rica, parts of the Middle East, the Chuvash Republic of Russia, and the Vasterbotten Province of Northern Sweden.

We currently have one LVV-based program targeting IMO, RP-L401. RP-L401 is a preclinical program that we in-licensed from Lund University, Sweden. This program has been granted ODD and Rare Pediatric Disease designation from the FDA. We have partnered with UCLA to lead U.S. clinical development efforts for the IMO program and UCLA will serve as the lead U.S. clinical site for IMO. The IND for RP-L401 to initiate a global Phase 1 study was cleared by the FDA in June 2020. The non-randomized, open-label Phase 1 clinical trial will enroll two pediatric patients, one month of age or older. The trial is designed to assess safety and tolerability of RP-L401, as well as preliminary efficacy, including potential improvements in bone abnormalities/density, hematologic status, and endocrine abnormalities.

In October 2020, we presented pre-clinical data from our LVV-based program targeting IMO, RP-L401, at the ESID 2020 Meeting. Preclinical data on IMO indicate that a modest level of engraftment can correct the disease phenotype *in vivo*, with increased long-term survival, tooth eruption, weight gain and normalized bone resorption. A comprehensive review of pre-clinical gene therapy investigations in TCIRG1-mediated osteopetrosis published in December 2020 supports acceleration into clinical development for RP-L401.

A clinical trial for RP-L401 was initiated in the fourth quarter of 2020.

The first patient treated with RP-L401 received investigational therapy during the second quarter of 2021. This was a 6-year-old child with severe IMO-related anemia and bone abnormalities. Although a drug product was successfully manufactured and infused without immediate complications, this patient died during the initial weeks after therapy of pulmonary complications, most likely pulmonary hemorrhage related to thrombocytopenia following conditioning therapy, and also related to underlying osteopetrosis. Corroborated by autopsy findings, this patient death was not considered to be RP-L401 related by the study investigators. Importantly, pulmonary hemorrhage is a rare but documented complication of HSCT, and pulmonary complications (including life threatening and fatal complications) have been observed to occur with high frequency in osteopetrosis patients undergoing allogenic HSCT procedures. Nonetheless in accordance with protocol-stipulated stopping rules, we have paused enrollment pending a comprehensive evaluation in collaboration with the Independent Data Monitoring Committee.

We expect to report clinical updates for our IMO program in the fourth quarter of 2021.

***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, we expect to submit our first biologics license applications (“BLAs”) and establish our gene therapy platform and expand our pipeline to target additional indications that we believe to be potentially compatible with our gene therapy technologies. In addition, during that time, we believe that our currently planned programs will become eligible for priority review vouchers from the FDA that provide for expedited review. We have assembled a leadership and research team with expertise in cell and gene therapy, rare disease drug development and 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 our resources to organizing and staffing, business planning, raising capital, acquiring, or discovering product candidates and securing related intellectual property rights, conducting discovery, research and development activities for the programs and planning for potential commercialization. We do not have any products approved for sale and have not generated revenue from product sales. From inception through June 30, 2021, we received net cash proceeds of approximately $654.1 million from investors through both equity and convertible debt financing to fund operating activities. As of June 30, 2021, we had cash, cash equivalents and investments of $426.8 million.

Since inception, we have incurred significant operating losses. Our ability to generate product revenue sufficient to achieve profitability will depend heavily on the successful development and eventual commercialization of one or more of the current or future product candidates and programs. We had net losses of $74.7 million for the six months ended June 30, 2021, and $139.7 million for the year ended December 31, 2020. As of June 30, 2021, we had an accumulated deficit of $397.5 million. We expect to continue to incur significant expenses and higher operating losses for the foreseeable future as we advance our current product candidates from discovery through preclinical development and clinical trials and seek regulatory approval of our product candidates. In addition, if we obtain marketing approval for any of our product candidates, we expect to incur significant commercialization expenses related to product manufacturing, marketing, sales and distribution. Furthermore, we expect to incur additional costs as a public company having transitioned out of emerging growth company status. Accordingly, we will need additional financing to support continuing operations and potential acquisitions of licensing or other rights for product candidates.

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

Because of the numerous risks and uncertainties associated with product development, we are unable to predict the timing or amount of increased expenses or when or if we will be able to achieve or maintain profitability. Even if we are able to generate product sales, we may not become profitable. If we fail to become profitable or are unable to sustain profitability on a continuing basis, then we may be unable to continue our operations at planned levels and be forced to reduce or terminate our operations.

***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 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;
- patent fees; 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 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 R&D activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development. As a result, we expect that R&D expenses will increase substantially over the next several years as we increase personnel costs, including stock-based compensation, support ongoing clinical studies, seek to achieve proof-of-concept in additional product candidates, advance preclinical programs to clinical programs, and prepare regulatory filings for product candidates.

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

- the scope, rate of progress, and expense of ongoing as well as any clinical studies and other 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.

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 as of June 30, 2021, is related to the 2021 Convertible Notes, which were converted into common stock on August 2, 2021, the 2022 Convertible Notes, which were redeemed and converted into common stock in April 2021, as well as the financing lease obligation for our Cranbury, NJ facility.

***Interest Income***

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

**Critical Accounting Policies and Significant Judgments and Estimates**

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

Our significant accounting policies are described in more detail in our 2020 Form 10-K.

**Results of Operations**

*Comparison of the Three Months Ended June 30, 2021 and 2020*

The following table summarizes the results of operations for the three months ended June 30, 2021 and 2020 ($ in thousands):

| | Three Months Ended June 30, | | |
|---|---|---|---|
| | **2021** | **2020** | **Change** |
| Operating expenses: | | | |
| Research and development | $ 24,798 | $ 16,731 | $ 8,067 |
| General and administrative | 9,250 | 6,828 | 2,422 |
| Total operating expenses | 34,048 | 23,559 | 10,489 |
| Loss from operations | (34,048) | (23,559) | (10,489) |
| Interest expense | (251) | (1,786) | 1,535 |
| Interest and other income net | 501 | 429 | 72 |
| Amortization of premium on investments - net | (727) | (124) | (603) |
| Total other expense, net | (477) | (1,481) | 1,004 |
| Net loss | $ (34,525) | $ (25,040) | $ (9,485) |

*Research and Development Expenses*

R&D expenses increased $8.1 million to $24.8 million for the three months ended June 30, 2021 compared to the three months ended June 30, 2020. The increase was primarily due to increases in compensation and benefits of $1.6 million due to increased R&D headcount, an increase in non-cash stock compensation expense of $1.7 million, an increase in manufacturing and development costs of $2.8 million, an increase in depreciation of $0.5 million, an increase in lab supplies and office expense of $0.5 million, an increase in research agreements for $0.5 million, and an increase in clinical trial expenses of $0.5 million.

*General and Administrative Expenses*

G&A expenses increased $2.4 million to $9.3 million for the three months ended June 30, 2021 compared to the three months ended June 30, 2020. The increases in G&A expenses were primarily driven by an increase in non-cash stock compensation expense of $1.1 million, an increase in compensation and benefits of $0.6 million due to increased G&A headcount, and an increase in office and administrative costs of $0.6 million.

*Other Expense, Net*

Other expense, net was $0.5 million for the three months ended June 30, 2021 compared to $1.5 million for the three months ended June 30, 2020. The change was primarily due to interest expense associated with the 2022 Convertible Notes which were redeemed in April 2021.

*Comparison of the Six Months Ended June 30, 2021 and 2020*

The following table summarizes the results of operations for the six months ended June 30, 2021 and 2020 ($ in thousands):

| | Six Months Ended June 30, | | |
|---|---|---|---|
| | 2021 | 2020 | Change |
| Operating expenses: | | | |
| Research and development | $ 53,340 | $ 33,687 | $ 19,653 |
| General and administrative | 19,930 | 13,990 | 5,940 |
| Total operating expenses | 73,270 | 47,677 | 25,593 |
| Loss from operations | (73,270) | (47,677) | (25,593) |
| Research and development incentives | 500 | - | 500 |
| Interest expense | (1,980) | (3,360) | 1,380 |
| Interest and other income net | 1,412 | 1,395 | 17 |
| Amortization of premium on investments - net | (1,366) | (62) | (1,304) |
| Total other expense, net | (1,434) | (2,027) | 593 |
| Net loss | $ (74,704) | $ (49,704) | $ (25,000) |

*Research and Development Expenses*

R&D expenses increased $19.7 million to $53.3 million for the six months ended June 30, 2021 compared to the six months ended June 30, 2020. The increase was primarily due to increases in compensation and benefits of $3.5 million due to increased R&D headcount, an increase in non-cash stock compensation expense of $2.9 million, an increase in manufacturing and development costs of $8.5 million, an increase in lab supplies and office expense of $1.3 million, an increase in depreciation and amortization of $1.5 million, an increase in research agreements of $0.2 million, an increase in building supplies and consumables related to the Cranbury, NJ facility for $0.3 million, and an increase in clinical trial expenses of $1.1 million.

*General and Administrative Expenses*

G&A expenses increased $6.0 million to $19.9 million for the six months ended June 30, 2021 compared to the six months ended June 30, 2020. The increases in G&A expenses were primarily driven by an increase in non-cash stock compensation expense of $3.9 million, an increase in compensation and benefits of $1.4 million due to increased G&A headcount, an increase in office and administrative costs of $1.6 million, an increase in accounting and consulting expenses of $0.7 million offset by a decrease in debt conversion expense recorded for the six months ended June 30, 2020 of $2.0 million due to the refinancing of the 2021 Convertible Notes in February 2020.

*Other Expense, Net*

Other expense, net was $1.4 million for the six months ended June 30, 2021 compared to $2.0 million for the six months ended June 30, 2020. The change was primarily due to interest expense associated with the 2022 Notes that were redeemed in April 2021 as well as a decrease of $1.3 million in accretion income related to our investments, due to lower interest rates.

**Liquidity, Capital Resources and Plan of Operations**

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

***Cash Flows***

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

| | Six Months Ended June 30, | |
|---|---|---|
| | 2021 | 2020 |
| Cash used in operating activities | $ (62,809) | $ (44,278) |
| Cash provided by investing activities | 20,178 | 22,064 |
| Cash provided provided by financing activities | 9,907 | 34 |
| Net change in cash, cash equivalents and restricted cash | $ (32,724) | $ (22,180) |

***Operating Activities***

During the six months ended June 30, 2021, operating activities used $62.8 million of cash, primarily resulting from our net loss of $74.7 million offset by net non-cash charges of $18.6 million, including non-cash stock-based compensation expense of $15.2 million and depreciation of $1.4 million. Changes in our operating assets and liabilities for the six months ended June 30, 2021 consisted of an decrease in accounts payable and accrued expenses for $9.0 million and an increase in our prepaid expenses of $1.0 million.

During the six months ended June 30, 2020, operating activities used $44.3 million of cash, primarily resulting from our net loss of $49.7 million offset by net non-cash charges of $10.2 million, including non-cash stock-based compensation expense of $8.5 million and accretion of the discount on convertible notes of $1.4 million. Changes in our operating assets and liabilities for the six months ended June 30, 2020 consisted of increases in prepaid expenses and other assets of $2.5 million, and accounts payable and accrued expenses of $3.2 million.

***Investing Activities***

During the six months ended June 30, 2021, net cash provided by investing activities was $20.2 million, consisting of proceeds of $180.6 million from the maturities of investments, offset by purchases of investments of $158.6 million, and purchases of property and equipment of $1.8 million.

During the six months ended June 30, 2020, net cash provided by investing activities was $22.1 million, consisting of proceeds of $88.7 million from the maturities of investments, offset by purchases of investments of $56.4 million, purchases of property and equipment of $7.1 million, payments made to acquire a right of use asset of $2.7 million, and purchases of internal use software of $0.4 million.

***Financing Activities***

During the six months ended June 30, 2021, net cash provided by financing activities was $9.9 million, consisting of issuance of common stock, pursuant to exercises of stock options.

During the six months ended June 30, 2020, there was a net zero movement on net cash provided by financing activities.

***Funding Requirements***

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

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

As of June 30, 2021, we had cash, cash equivalents and investments of $426.8 million. We expect such resources would be sufficient to fund our operating expenses and capital expenditure requirements into the second half of 2023.

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

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

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

If we raise funds through governmental funding, collaborations, strategic partnerships or marketing, distribution or licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies, future revenue streams, research programs or product candidates or grant licenses on terms that may not be favorable to us. If we are unable to raise additional funds through equity or debt financings when needed, we may be required to delay, reduce or eliminate our product development or future commercialization efforts or grant rights to develop and market product candidates that it would otherwise prefer to develop and market themselves.

**Contractual Obligations and Commitments**

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 2020 Form 10-K. Information regarding contractual obligations and commitments may be found in Note 11 of our Consolidated Unaudited Financial Statements in this Form 10-Q.

**Off-Balance Sheet Arrangements**

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

**Recently Issued Accounting Pronouncements**

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

## Item 3 Quantitative and Qualitative Disclosures About Market Risk

We are exposed to market risks in the ordinary course of our business. These market risks are principally limited to interest rate fluctuations. We had cash, cash equivalents and investments of $426.8 million at June 30, 2021, consisting primarily of funds in a money market account, corporate and municipal bonds and United States Treasury securities. The primary objective of our investment activities is to preserve principal and liquidity while maximizing income without significantly increasing risk. We do not enter into investments for trading or speculative purposes. Due to the short-term nature of our investment portfolio, we do not believe an immediate 1.0% increase in interest rates would have a material effect on the fair market value of our portfolio, and accordingly we do not expect a sudden change in market interest rates to affect materially our operating results or cash flows.

Our 2021 Convertible Notes bore interest at a fixed rate and therefore a change in interest rates would not have impacted the amount of interest we would have had to pay on this indebtedness, while it was outstanding.

## Item 4 Controls and Procedures

***Evaluation of Disclosure Controls and Procedures***

Our management, with the participation of our principal executive officer and our principal financial officer, evaluated, as of the end of the period covered by this Form 10-Q, the effectiveness of our disclosure controls and procedures. Based on that evaluation of our disclosure controls and procedures as of June 30, 2021, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures as of such date are effective at the reasonable assurance level. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act are recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms. Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

***Inherent Limitations of Internal Controls***

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

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

There were no changes in our internal control over financial reporting (as defined in Rules 13a-15(f) and 15d-15(f) under the Exchange Act) during the six months ended June 30, 2021, that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

## PART II – OTHER INFORMATION

### Item 1. Legal Proceedings

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

### Item 1A. Risk Factors

Our material risk factors are disclosed in Item 1A of our Annual Report on Form 10-K for the year ended December 31, 2020 filed with the Securities and Exchange Commission on March 1, 2021. 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) |
| 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) |
| 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.

**SIGNATURES**

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

ROCKET PHARMACEUTICALS, INC.

August 9, 2021 By: /s/ Gaurav Shah, MD

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

August 9, 2021 By: /s/ Carlos Garcia-Parada

Carlos Garcia-Parada
*Chief Financial Officer*
*(Principal Financial 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 June 30, 2021 of Rocket Pharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 9, 2021

/s/ Gaurav Shah, MD

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

**Exhibit 31.2**

## CERTIFICATIONS

I, Carlos Garcia-Parada, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended June 30, 2021 of Rocket Pharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
   a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
   b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
   c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
   d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
   a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
   b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: August 9, 2021

/s/ Carlos Garcia-Parada

Carlos Garcia-Parada
*Chief Financial Officer*
*(Principal Financial Officer)*

**Exhibit 32.1**

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

In connection with the quarterly report on Form 10-Q of Rocket Pharmaceuticals, Inc. (the "Company") for the period ended June 30, 2021, as filed with the United States Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers hereby certifies, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, 18 U.S.C. Section 1350, that to his knowledge:

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

Date: August 9, 2021

/s/ Gaurav Shah, MD

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

Date: August 9, 2021

/s/ Carlos Garcia-Parada

Carlos Garcia-Parada
*Chief Financial Officer*
*(Principal Financial Officer)*