

**UNITED STATES
SECURITIES AND EXCHANGE COMMISSION**

Washington, D.C. 20549

Form 10-Q

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

For the quarterly period ended March 31, 2026 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

(State or other jurisdiction of incorporation or organization)

04-3475813

(I.R.S. Employer Identification No.)

9 Cedarbrook Drive, Cranbury, NJ

(Address of principal executive office)

08512

(Zip Code)

(609) 659-8001

(Registrant's telephone number, including area code)

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

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

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

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

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

Large accelerated filer

Non-accelerated filer

Accelerated filer

Smaller reporting company

Emerging growth company

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

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

As of May 1, 2026, there were 109,193,549 shares of common stock, \$0.01 par value per share, outstanding.

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Summary of Abbreviated Terms

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

AAV	Adeno-associated virus	IV	Intravenous
AAV9	Adeno-associated serotype 9	IPR&D	In process research and development
ACM	Arrhythmogenic cardiomyopathy	KCCQ-12	Kansas City Cardiovascular Questionnaire-12
ASC	Accounting Standard Codification	LAD-1	Leukocyte Adhesion Deficiency-1
ASGCT	American Society of Gene & Cell Therapy	LAMP2	Lysosome-associated membrane protein 2
ATMP	Advanced Therapy Medical Product	LV	Lentiviral vector
BAG3	BCL2-associated athanogene 3	LVMI	Left ventricular mass index
BAG3-DCM	BCL2-associated athanogene 3 mutations associated with dilated cardiomyopathy	NYHA	New York Heart Association
BLA	Biologics License Application	PDUFA	Prescription Drug User Fee Act
BNP	Brain natriuretic peptide	PKD	Pyruvate Kinase Deficiency
Cantor	Cantor Fitzgerald & Co.	PKP2	Plakophilin-2
cGMP	Current Good Manufacturing Practice	PKP2-ACM	Plakophilin-2 Arrhythmogenic Cardiomyopathy
cKO	conditional knockout	PRV	Priority Review Voucher
CIRM	California Institute for Regenerative Medicine	PSU	Performance-Based Restricted Stock Unit
CMC	Chemistry Manufacturing Controls	PRIME	Priority Medicines
CODM	Chief Operating Decision Maker	R&D	Research and development
CRL	Complete Response Letter	Renovacor	Renovacor, Inc. acquired on December 1, 2022
CTIS	Clinical Trials Information Systems	RIF	Reduction in workforce
DCM	Dilated cardiomyopathy	RMAT	Regenerative Medicine Advanced Therapy
DD	Danon Disease	RSU	Restricted Stock Unit
DNA	Deoxyribonucleic acid	RTW	RTW Investments, L.P
EMA	European Medicines Agency	SAE	Serious Adverse Event
ESB Lease Agreement	Office Lease agreement for office space in the Empire State Building in New York City	SCD	Sudden cardiac death
EU	European Union	SEC	Securities and Exchange Commission
FA	Fanconi Anemia	Stanford	Center for Definitive and Curative Medicine at Stanford University School of Medicine
FDA	U.S. Food and Drug Administration	UCLA	University of California, Los Angeles
GMP	Good Manufacturing Practice	UCLB	UCL Business PLC
HF	Heart failure	UCSD	The Regents of the University of California, San Diego
HLA	Human Leukocyte Antigen	UPC	Unitary Patent Court
HNJ	Hospital Infantil de Niño Jesús	U.K.	United Kingdom
HSCT	Hematopoietic stem cell transplant	U.S.	United States
ICD	Internal Classification of Diseases	U.S. GAAP	U.S. Generally Accepted Accounting Principles
IND	Investigational New Drug application	USPTO	U.S. Patent and Trademark Office

Cautionary Statement Regarding Forward-Looking Statements

This Quarterly Report on Form 10-Q for the quarter ended March 31, 2026 contains forward-looking statements that involve risks and uncertainties, as well as assumptions that, if they do not materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q are forward-looking statements. In some cases, you can identify forward-looking statements by words such as “aim,” “anticipate,” “believe,” “can,” “contemplate,” “continue,” “could,” “design,” “develop,” “estimate,” “expect,” “expand,” “future,” “hope,” “intend,” “likely,” “may,” “plan,” “potential,” “predict,” “project,” “pursue,” “seek,” “should,” “strategy,” “target,” “will,” “would,” or the negative of these words or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- our ability to obtain additional funding to conduct our planned R&D efforts;
- our ability to meet our anticipated milestones for our various drug candidates with respect to the initiation and timing of clinical studies;
- federal, state, and non-U.S. regulatory requirements, including regulation of our current or any other future product candidates by the FDA;
- the timing of and our ability to submit regulatory filings with the FDA and to obtain and maintain FDA or other regulatory authority approval of, or other action with respect to, our product candidates;
- our competitors’ activities, including decisions as to the timing of competing product launches, pricing, and discounting;
- whether safety and efficacy results of our clinical trials and other required tests for approval of our product candidates provide data to warrant progression of clinical trials, potential regulatory approval, or further development of any of our product candidates;
- our ability to develop, acquire and advance product candidates into, enroll a sufficient number of patients into, and successfully complete, clinical studies, and our ability to apply for and obtain regulatory approval for such product candidates, within currently anticipated timeframes, or at all;
- our ability to establish key collaborations and vendor relationships for our product candidates and any other future product candidates;
- our ability to develop our sales and marketing capabilities or enter into agreements with third parties to sell and market any of our product candidates;
- our ability to acquire additional businesses, form strategic alliances or create joint ventures and our ability to realize the benefit of such acquisitions, alliances, or joint ventures;
- our ability to successfully develop and commercialize any technology that we may in-license or products we may acquire;
- the development of our direct manufacturing capabilities for our AAV programs;
- our ability to expand our pipeline to target additional indications that are compatible with our gene therapy technologies;
- our ability to achieve the expected benefits of our portfolio prioritization and strategic restructuring, including extending our cash runway, and our estimates related to the costs and timing of implementing such initiative;
- our ability to successfully operate in non-U.S. jurisdictions in which we currently or in the future do business, including compliance with applicable regulatory requirements and laws;
- our ability to obtain and enforce patents to protect our product candidates, and our ability to successfully defend ourselves against unforeseen third-party infringement claims;
- anticipated trends and challenges in our business and the markets in which we operate;
- our estimates regarding our capital requirements;
- our ability to close on the sale of our PRV pursuant to the asset purchase agreement we entered into in April 2026; 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, 2025, on Form 10-K, that could cause actual results or events to differ materially from the forward-looking statements that we make. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures, or investments we may make or enter into.

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

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

PART I — FINANCIAL INFORMATION
Item 1. Financial Statements

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

	March 31, 2026 (unaudited)	December 31, 2025
Assets		
Current assets:		
Cash and cash equivalents	\$ 49,610	\$ 77,558
Investments	94,769	111,371
Prepaid expenses and other current assets	5,448	3,805
Total current assets	149,827	192,734
Property and equipment, net	26,655	28,170
Goodwill	39,154	39,154
Intangible assets	25,150	25,150
Restricted cash	1,341	1,340
Deposits	482	482
Operating lease right-of-use assets, net	3,127	3,210
Finance lease right-of-use asset, net	39,671	40,209
Total assets	<u>\$ 285,407</u>	<u>\$ 330,449</u>
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	\$ 21,542	\$ 27,291
Operating lease liabilities, current	998	1,003
Finance lease liability, current	1,926	1,912
Total current liabilities	24,466	30,206
Operating lease liabilities, non-current	2,493	2,600
Finance lease liability, non-current	19,347	19,362
Other liabilities	1,062	1,060
Total liabilities	47,368	53,228
Commitments and contingencies (Note 13)		
Stockholders' equity:		
Preferred stock, \$0.01 par value, authorized 5,000,000 shares:		
Series A convertible preferred stock; 300,000 shares designated; 0 shares issued and outstanding	-	-
Series B convertible preferred stock; 300,000 shares designated; 0 shares issued and outstanding	-	-
Common stock, \$0.01 par value, 180,000,000 shares authorized; 109,123,671 and 108,319,783 shares issued and outstanding as of March 31, 2026 and December 31, 2025, respectively	1,091	1,083
Additional paid-in capital	1,725,893	1,717,408
Accumulated other comprehensive (loss) income	(112)	(31)
Accumulated deficit	(1,488,833)	(1,441,239)
Total stockholders' equity	238,039	277,221
Total liabilities and stockholders' equity	<u>\$ 285,407</u>	<u>\$ 330,449</u>

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

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

	Three Months Ended March 31,	
	2026	2025
Revenue	\$ -	\$ -
Operating expenses:		
Research and development	31,454	35,942
General and administrative	17,057	28,446
Total operating expenses	48,511	64,388
Loss from operations	(48,511)	(64,388)
Interest expense	(473)	(472)
Interest and other income, net	161	1,336
Accretion of discount on investments, net	1,229	2,190
Net loss	\$ (47,594)	\$ (61,334)
Net loss per share - basic and diluted	\$ (0.42)	\$ (0.56)
Weighted-average common shares outstanding - basic and diluted	112,134,059	110,093,461

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

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

	Three Months Ended March 31,	
	2026	2025
Net loss	\$ (47,594)	\$ (61,334)
Other comprehensive loss:		
Net unrealized loss on investments	(81)	(93)
Total comprehensive loss	<u>\$ (47,675)</u>	<u>\$ (61,427)</u>

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

Rocket Pharmaceuticals, Inc.
Consolidated Statements of Stockholders' Equity
For the Three Months Ended March 31, 2026 and 2025
(\$ in thousands except share amounts)
(unaudited)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income/(Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2025	108,319,783	\$ 1,083	\$ 1,717,408	\$ (31)	\$ (1,441,239)	\$ 277,221
Issuance of common stock pursuant to vesting of restricted stock units	803,888	8	(8)	-	-	-
Unrealized comprehensive loss on investments	-	-	-	(81)	-	(81)
Stock-based compensation	-	-	8,493	-	-	8,493
Net loss	-	-	-	-	(47,594)	(47,594)
Balance at March 31, 2026	<u>109,123,671</u>	<u>\$ 1,091</u>	<u>\$ 1,725,893</u>	<u>\$ (112)</u>	<u>\$ (1,488,833)</u>	<u>\$ 238,039</u>

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income/(Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2024	106,453,818	\$ 1,065	\$ 1,680,219	\$ 66	\$ (1,218,116)	\$ 463,234
Issuance of common stock pursuant to vesting of restricted stock units	300,068	3	(3)	-	-	-
Unrealized comprehensive loss on investments	-	-	-	(93)	-	(93)
Stock-based compensation	-	-	10,331	-	-	10,331
Net loss	-	-	-	-	(61,334)	(61,334)
Balance at March 31, 2025	<u>106,753,886</u>	<u>\$ 1,068</u>	<u>\$ 1,690,547</u>	<u>\$ (27)</u>	<u>\$ (1,279,450)</u>	<u>\$ 412,138</u>

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

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

	Three Months Ended March 31,	
	2026	2025
Operating activities:		
Net loss	\$ (47,594)	\$ (61,334)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization of property and equipment	1,608	2,451
Amortization of finance lease right of use asset	538	538
Stock-based compensation	8,493	10,331
Accretion of discount on investments, net	(1,020)	(2,075)
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(1,643)	(467)
Accounts payable and accrued expenses	(5,749)	(5,258)
Operating lease liabilities and right of use assets, net	(29)	12
Finance lease liability	-	12
Other liabilities	2	-
Net cash used in operating activities	<u>(45,394)</u>	<u>(55,790)</u>
Investing activities:		
Purchases of investments	(48,709)	(139,931)
Proceeds from maturities of investments	66,250	82,260
Purchases of property and equipment	(93)	(364)
Net cash provided by (used in) investing activities	<u>17,448</u>	<u>(58,035)</u>
Financing activities:		
Finance lease liability	(1)	-
Net cash used in financing activities	<u>(1)</u>	<u>-</u>
Net change in cash, cash equivalents and restricted cash	(27,947)	(113,825)
Cash, cash equivalents and restricted cash at beginning of period	78,898	164,997
Cash, cash equivalents and restricted cash at end of period	<u>\$ 50,951</u>	<u>\$ 51,172</u>
Supplemental disclosure of non-cash financing and investing activities:		
Accrued purchases of property and equipment, ending balance	\$ -	\$ 51

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

Rocket Pharmaceuticals, Inc.
Notes to Consolidated Financial Statements
(\$ in thousands, except shares and per share data) (Unaudited)

1. Nature of Business

Rocket Pharmaceuticals, Inc. (the “Company” or “Rocket”) is a fully integrated commercial-stage biotechnology company advancing a sustainable pipeline of genetic therapies, including one approved product and multiple investigational programs designed to correct the root cause of complex and rare disorders. Rocket’s innovative multi-platform approach allows the Company to design the optimal gene therapy for each indication, creating potential treatment options for people living with severe and rare diseases.

Rocket’s *in vivo* adeno-associated viral (AAV) vector-based cardiovascular portfolio includes a late-stage clinical program for Danon disease, a devastating heart failure condition resulting in thickening of the heart, and early-stage clinical programs for PKP2-arrhythmogenic cardiomyopathy (PKP2-ACM), a life-threatening heart failure disease causing ventricular arrhythmias and sudden cardiac death, and BAG3-associated dilated cardiomyopathy (BAG3-DCM), a heart failure condition that causes enlarged ventricles.

Rocket’s *ex vivo* lentiviral (LV) vector-based hematology portfolio includes KRESLADI™, which was granted FDA accelerated approval in March 2026 for severe Leukocyte Adhesion Deficiency-I (LAD-I), as well as additional programs for Fanconi Anemia (FA) and Pyruvate Kinase Deficiency (PKD).

2. Risks and Liquidity

The Company has not generated revenue from product sales to date and has incurred losses since inception. KRESLADI™ was approved by the FDA in March 2026 under the accelerated approval pathway; however, the Company has not yet generated product revenue. KRESLADI™ was approved under the FDA’s accelerated approval pathway based on an increase in neutrophil CD18 and CD11a surface expression. Continued approval may be contingent upon verification and description of clinical benefit through ongoing clinical follow-up and additional post-marketing data collection. Operations of the Company are subject to certain risks and uncertainties, including, among others, uncertainty of drug candidate development, technological uncertainty, uncertainty regarding patents and proprietary rights, having no commercial manufacturing experience, marketing or sales capability or experience, dependency on key personnel, compliance with government regulations and the need to obtain additional financing. Drug candidates currently under development will require significant additional R&D efforts, including extensive preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure, and extensive compliance-reporting capabilities. The commercialization of KRESLADI™ is subject to significant operational, logistical, and reimbursement-related risks. The delivery of autologous gene therapies requires complex coordination, including treatment center onboarding, manufacturing, and vein-to-vein logistics, which may impact the timing and scale of commercial uptake. In addition, given the ultra-rare patient population and anticipated phased commercial rollout, the Company does not expect KRESLADI™ to generate material revenue in the near term.

The Company’s product candidates are in the development and clinical stage. There can be no assurance that the Company’s R&D 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.

In May 2025, two patients participating in the Phase 2 pivotal study of RP-A501 each experienced an unexpected SAE. Rocket voluntarily paused further Phase 2 study dosing in the U.S. and E.U., and the FDA placed a clinical hold on the trial to allow for further evaluation, which was lifted on August 20, 2025, after the Company satisfactorily addressed issues outlined in the clinical hold.

In July 2025, the Company implemented a strategic corporate reorganization designed to align its resources with its highest-priority programs, namely, the AAV-based gene therapy platform focused on cardiovascular diseases. As part of this effort, the Company reduced its workforce by approximately 30%.

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 incurred recurring losses and negative cash flows from operations and had an accumulated deficit of \$1.49 billion as of March 31, 2026. As of March 31, 2026, the Company has \$144.4 million of cash, cash equivalents, and investments. In April 2026, the Company entered into an agreement to sell its Rare Pediatric Disease Priority Review Voucher (PRV) for \$180 million, which is expected to provide non-dilutive capital to support advancement of the Company's cardiovascular gene therapy pipeline and extend its operational runway. Based on the Company's current operating plan and the expected proceeds from the PRV sale, the Company believes its existing cash, cash equivalents, investments, and anticipated PRV proceeds will be sufficient to fund operations for at least twelve months from the filing date of this Quarterly Report on Form 10-Q.

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 expects to continue to generate operating losses for the foreseeable future and to finance its future cash needs through, but not limited to, one or a combination of equity offerings, debt financings, collaborations, strategic partnerships and alliances or licensing arrangements. If the Company is unable to obtain funding, the Company would be forced to delay, reduce or eliminate some or all of its R&D programs, preclinical and clinical testing or commercialization efforts, which could adversely affect its business prospects. 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.

The Company may also face challenges retaining key personnel and maintaining continuity across teams, which could impair its ability to advance clinical programs, meet regulatory milestones, or pursue long-term strategic objectives. Potential litigation or other employee-related claims arising from the workforce reduction could divert management attention and further increase costs. Any of these factors could have a material adverse effect on our business, operating results, and financial condition.

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, 2025 included in the Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 26, 2026. The unaudited interim consolidated financial statements have been prepared on the same basis as the audited annual financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for the fair statement of the Company's consolidated financial position as of March 31, 2026 and the results of its operations and its cash flows for the three months ended March 31, 2026. The financial data and other information disclosed in these consolidated notes related to the three months ended March 31, 2026 and 2025 are unaudited. The results for the three months ended March 31, 2026 are not necessarily indicative of results to be expected for the year ending December 31, 2026 and any other interim periods or any future year or period.

Significant Accounting Policies

The significant accounting policies used in the preparation of these consolidated financial statements for the three months ended March 31, 2026 are consistent with those disclosed in Note 3 to the consolidated financial statements in the 2025 Form 10-K with most significant policies also being listed here.

Principles of Consolidation

The consolidated financial statements represent the consolidation of the accounts of the Company and its subsidiaries in conformity with U.S. GAAP. All intercompany accounts have been eliminated in consolidation.

Use of Estimates

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

Cash, Cash Equivalents and Restricted Cash

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

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

	March 31, 2026	December 31, 2025
Cash and cash equivalents	\$ 49,610	\$ 77,558
Restricted cash	1,341	1,340
Total cash, cash equivalents and restricted cash	<u>\$ 50,951</u>	<u>\$ 78,898</u>

Concentrations of credit risk and off-balance sheet risk

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

Investments

Investments consist of U.S. Treasury Securities. Management determines the appropriate classification of these securities at the time they are acquired and evaluates the appropriateness of such classifications at each balance sheet date. The Company classifies its investments as available-for-sale pursuant to ASC 320, Investments-Debt and Equity Securities. Investments are recorded at fair value, with unrealized gains and losses included as a component of accumulated other comprehensive income (loss) in stockholders' equity and a component of total comprehensive loss in the consolidated statements of comprehensive loss, until realized. Realized gains and losses are included in investment income on a specific-identification basis. The Company estimates expected credit losses for investments when unrealized losses exist. Unrealized losses that are credit related are recognized in the Company's Consolidated Statement of Operations and unrealized losses that are not credit related are recognized in accumulated other comprehensive income (loss). For the three months ended March 31, 2026 and 2025, there were no unrealized losses that were credit related. For the three months ended March 31, 2026, and 2025 there were net unrealized loss on investments of \$0.1 and \$0.1 million, respectively.

Intangible Assets

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

Goodwill

Goodwill is tested for impairment annually as of December 31, or more frequently when events or changes in circumstances indicate that the asset might be impaired.

Property and Equipment, Net

Property and equipment are stated at cost less accumulated depreciation. Depreciation expense is recognized using the straight-line method over the estimated useful lives of the asset which are three to fifteen years. Expenditures for repairs and maintenance of assets are charged to expense as incurred. Upon retirement or sale, the cost and related accumulated depreciation of assets disposed of are removed from the accounts and any resulting gain or loss is included in loss from operations. Costs incurred in connection with development or purchase of internal use software and cloud computing arrangements, including in-substance software licenses, are capitalized as computer equipment and internal-use software. Amortization is computed on a straight-line basis over the estimated useful life of the asset, which is six years. Capitalized software is included in property and equipment in the Consolidated Balance Sheets.

Fair Value Measurements

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

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

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

Warrants

The Company accounts for stock warrants as either equity instruments, liabilities or derivative liabilities in accordance with ASC 480, Distinguishing Liabilities from Equity and/or ASC 815, Derivatives and Hedging, depending on the specific terms of the warrant agreement. Liability-classified warrants are recorded at their estimated fair values at each reporting period until they are exercised, terminated, reclassified or otherwise settled. Changes in the estimated fair value of liability-classified warrants are included in interest and other income in the Company's Consolidated Statement of Operations. Warrants classified as equity instruments are recorded within additional paid-in capital at the time of issuance and are not subject to remeasurement.

Stock-Based Compensation

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

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

The fair value of options on the date of grant is calculated using the Black-Scholes option pricing model based on key assumptions such as expected volatility and expected term.

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

Segment Reporting

Operating segments are defined as components of an enterprise about which separate discrete information is available for evaluation by the chief operating decision maker, or decision-making group, in deciding how to allocate resources and in assessing performance. The Company views its operations and manages its business in one operating segment. The Company's CODM is its Chief Executive Officer and the senior leadership team. The CODM manages the Company's operations on an integrated basis for the purpose of allocating resources. When evaluating the Company's financial performance, the CODM regularly reviews total expenses and expenses by significant areas to make decisions on a company wide basis. Included in these expenses are R&D expenses by program.

Recent Accounting Pronouncements

Accounting Pronouncements Not Adopted as of March 31, 2026

ASU 2024-03: Expense Disaggregation Disclosures. This update requires disaggregated disclosure of income statement expenses. This update is effective for fiscal years beginning after December 15, 2027. Early adoption is permitted. The Company is evaluating the effect that ASU 2024-03 will have on its financial statements and disclosures.

ASU 2025-10: Government Grants Topic 832. This update adds guidance on the recognition, measurement and presentation of government grants. This update is effective for fiscal years beginning after December 15, 2028. The Company is evaluating the effect that ASU 2025-10 will have on its financial statements and disclosures.

ASU 2025-11: Interim Reporting Topic 270. This update is intended to improve the navigability of guidance in ASC 270, Interim Reporting, and clarify when it applies. The amendments also provide additional guidance on what disclosures should be provided in interim reporting periods. This update is effective for fiscal years beginning after December 15, 2027. The Company is evaluating the effect that ASU 2025-11 will have on its disclosures.

4. Fair Value of Financial Instruments

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

	Amortized Cost	Fair Value Measurements as of March 31, 2026, Using:			
		Level 1	Level 2	Level 3	Total
Assets:					
Cash equivalents:					
Money market mutual funds	\$ 27,453	\$ 27,453	\$ -	\$ -	\$ 27,453
U.S. Treasury securities	4,481	-	4,480	-	4,480
	<u>31,934</u>	<u>27,453</u>	<u>4,480</u>	<u>-</u>	<u>31,933</u>
Investments:					
U.S. Treasury securities	94,782	-	94,769	-	94,769
	<u>94,782</u>	<u>-</u>	<u>94,769</u>	<u>-</u>	<u>94,769</u>
Total assets	<u>\$ 126,716</u>	<u>\$ 27,453</u>	<u>\$ 99,249</u>	<u>\$ -</u>	<u>\$ 126,702</u>

	Amortized Cost	Fair Value Measurements as of December 31, 2025, Using:			
		Level 1	Level 2	Level 3	Total
Assets:					
Cash equivalents:					
Money market mutual funds	\$ 22,090	\$ 22,090	\$ -	\$ -	\$ 22,090
U.S. Treasury securities	41,919	-	41,918	-	41,918
	<u>64,009</u>	<u>22,090</u>	<u>41,918</u>	<u>-</u>	<u>64,008</u>
Investments:					
U.S. Treasury securities	111,309	-	111,371	-	111,371
	<u>111,309</u>	<u>-</u>	<u>111,371</u>	<u>-</u>	<u>111,371</u>
Total assets	<u>\$ 175,318</u>	<u>\$ 22,090</u>	<u>\$ 153,289</u>	<u>\$ -</u>	<u>\$ 175,379</u>

The amortized cost for marketable debt securities approximates its fair value and these securities mature within one year as of March 31, 2026 and December 31, 2025,

The Company classifies its money market mutual funds as Level 1 assets under the fair value hierarchy, as these assets have been valued using quoted market prices in active markets without any valuation adjustment. The Company classifies its U.S. Treasury Securities 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 Company had a warrant liability, which expired on April 23, 2025. The warrant liability was recorded as part of other liabilities in the Consolidated Balance Sheets and measured at fair value on a recurring basis using unobservable inputs (Level 3). The warrant liability balance was approximately \$0 on March 31, 2025.

The Company utilized a Black-Scholes model to value the warrant liability with the estimated fair value of the warrant liability determined using an options pricing model and Level 3 inputs including expected share-price volatility of underlying company common stock, expected life of warrant, risk-free interest rate and dividend yield. The Company estimated the expected share-price volatility of its common stock based on historical volatility of its common stock, considering the expected remaining life of the warrant. The expected life of the warrant was assumed to be equivalent to their remaining contractual term. The risk-free interest rate was based on the U.S. Treasury zero-coupon yield curve on the valuation date for a maturity similar to the expected remaining life of the warrant. The dividend rate was based on the historical dividend rate, which the Company anticipated to remain at zero.

5. Property and Equipment, Net

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

	March 31, 2026	December 31, 2025
Laboratory equipment	\$ 32,231	\$ 32,138
Machinery and equipment	12,133	12,133
Computer equipment	1,015	1,015
Furniture and fixtures	2,777	2,777
Leasehold improvements	7,327	7,327
Internal use software	1,903	1,903
	<u>57,386</u>	<u>57,293</u>
Less: accumulated depreciation and amortization	(30,731)	(29,123)
Total property and equipment, net	<u>\$ 26,655</u>	<u>\$ 28,170</u>

During the three months ended March 31, 2026 and 2025, the Company recognized \$1.6 million and \$2.5 million of depreciation and amortization expense, respectively.

6. Intangible Assets and Goodwill

The Company's intangible assets consisted of an acquired IPR&D asset received in the acquisition of Renovacor with a carrying value of \$25.2 million as of March 31, 2026 and December 31, 2025.

The carrying value of Goodwill as of March 31, 2026 and December 31, 2025 was \$39.2 million.

7. Accounts Payable and Accrued Expenses

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

	March 31, 2026	December 31, 2025
Research and development	\$ 9,887	\$ 10,302
Employee compensation	5,235	12,294
Professional fees	2,197	2,318
Restructuring	-	46
Other	4,223	2,331
Total accounts payable and accrued expenses	<u>\$ 21,542</u>	<u>\$ 27,291</u>

8. Stockholders' Equity

At-the-Market Offering Program

On March 10, 2026, the Company entered into a sales agreement (the "Sales Agreement") with Cantor with respect to an at-the-market offering program pursuant to which the Company may offer and sell, from time to time at its sole discretion, shares having an aggregate price of up to \$100,000,000 through Cantor as its sales agent. The shares to be offered and sold under the Sales Agreement, if any, will be offered and sold pursuant to the Company's shelf registration statement on Form S-3. The Company filed a prospectus supplement with the SEC on March 10, 2026, in connection with the offer and sale of the shares pursuant to the Sales Agreement. The Company will pay Cantor a cash commission of up to 3.0% of gross proceeds from the sale of the shares pursuant to the Sales Agreement. Through March 31, 2026, the Company has not sold any shares under the at-the-market offering program.

9. Stock-Based Compensation

Stock Option Valuation

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

	Three Months Ended March 31,	
	2026	2025
Risk-free interest rate	3.65%	4.35%
Expected term (in years)	5.27	5.42
Expected volatility	91.00%	71.29%
Expected dividend yield	0.00%	0.00%
Exercise price	\$ 4.77	\$ 10.73
Fair value of common stock	\$ 4.77	\$ 10.73

The following table summarizes stock option activity for the three months ended March 31, 2026:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Contractual Term (Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2025	12,163,019	19.98	4.53	706
Granted	2,344,545	4.77	8.82	
Exercised	-	-		
Cancelled or forfeited	(346,516)	16.24		
Outstanding as of March 31, 2026	14,161,048	17.56	5.09	1,478
Options vested and exercisable as of March 31, 2026	10,018,923	\$ 22.18	3.44	\$ 336
Options unvested as of March 31, 2026	4,142,125	\$ 6.37	9.08	\$ 1,142

The weighted average grant-date fair value per share of stock options granted during the three months ended March 31, 2026, and 2025 was \$3.28 and \$6.05, respectively.

The total fair value of options vested during the three months ended March 31, 2026 and 2025 was \$4.4 million and \$10.7 million, respectively.

Restricted Stock Units

The following table summarizes the Company's RSU activity for the three months ended March 31, 2026:

	Number of Shares	Weighted Average Grant Date Fair Value
Unvested as of December 31, 2025	5,000,566	\$ 6.51
Granted	3,685,796	4.36
Vested ⁽¹⁾	(803,054)	9.30
Forfeited	(185,105)	7.04
Unvested as of March 31, 2026	7,698,203	\$ 5.18

(1) Common stock issued excludes 834 awards that were vested in 2025 but issued in 2026.

The total fair value of RSUs vested during the three months ended March 31, 2026 and 2025 was \$7.3 million and \$7.3 million, respectively.

Performance Stock Units

The Company granted PSU awards in 2024. PSU vesting and expense recognition is based on achievement of specific performance goals within certain time periods specified in the respective PSU award agreements. PSU awards that are not achieved within specific time periods are forfeited. As of December 31, 2025, the performance periods for all PSUs issued in 2024 had expired and all PSUs were forfeited.

Stock-Based Compensation Expense

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

	Three Months Ended March 31,	
	2026	2025
Stock options	\$ 3,548	\$ 5,907
Restricted stock units	4,945	4,424
Total stock-based compensation expense	<u>\$ 8,493</u>	<u>\$ 10,331</u>

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

	Three Months Ended March 31,	
	2026	2025
Research and development	\$ 4,307	\$ 4,388
General and administrative	4,186	5,943
Total stock-based compensation expense	<u>\$ 8,493</u>	<u>\$ 10,331</u>

As of March 31, 2026, the Company had an aggregate of \$51.5 million of unrecognized stock-based compensation expense related to stock options and RSU grants, which is expected to be recognized over a weighted average period of 2.16 years.

10. Warrants

A summary of the warrants outstanding as of March 31, 2026 is as follows:

Exercise Price	Outstanding	Grant/Assumption Date	Expiration Date
\$57.11	603,386	December 21, 2020	December 21, 2030
\$33.63	301,291	August 9, 2021	August 9, 2031
\$22.51	153,155	December 17, 2021	December 17, 2031
\$22.51	153,155	December 17, 2021	December 17, 2031
\$65.23	760,086	December 1, 2022	December 1, 2026
\$0.01	3,126,955	September 15, 2023	N/A
\$0.01	400,000	December 12, 2024	N/A
Total	<u>5,498,028</u>		

Warrants Issued in Public Offerings

In 2024 and 2023, the Company sold pre-funded warrants to purchase 400,000 and 3,126,955 shares of common stock, respectively at a price of \$0.01 per share. The pre-funded warrants were acquired by funds affiliated with RTW.

11. Net Loss Per Share

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

	Three Months Ended March 31,	
	2026	2025
Numerator:		
Net loss attributable to common stockholders	\$ (47,594)	\$ (61,334)
Denominator:		
Weighted-average common shares outstanding - basic and diluted	112,134,059	110,093,461
Net loss per share attributable to common stockholders - basic and diluted	\$ (0.42)	\$ (0.56)

For the three months ended March 31, 2026 and 2025, the Company included the 3,126,955 potential shares from pre-funded warrants acquired by RTW in 2023 and the 400,000 potential shares from pre-funded warrants acquired by RTW in 2024 in the basic weighted-average common shares outstanding as the warrants only require the holder to pay \$0.01 per share upon exercise.

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

	Three Months Ended March 31,	
	2026	2025
Warrants exercisable for common shares	1,971,073	2,588,123
Restricted stock units	7,698,203	4,048,463
Performance stock units	-	69,661
Options to purchase common shares	14,161,048	17,200,534
Total potential shares excluded from diluted net loss per share	23,830,324	23,906,781

12. Leases

Finance Lease

The Company has a lease for a facility in Cranbury, New Jersey, consisting of 103,720 square feet of space including areas for offices, process development, research, and development laboratories and 50,000 square feet dedicated to AAV cGMP manufacturing facilities to support the Company's pipeline (such lease, as amended, the "NJ Lease Agreement"). The NJ Lease Agreement has a 15-year term from September 1, 2019, with an option to renew for two consecutive five-year renewal terms. The renewal periods were included in the lease term as it was determined at commencement date that it was reasonably certain to exercise this option.

Estimated rent payments for the NJ Lease Agreement are \$1.2 million per annum, payable in monthly installments, and subject to annual base rent increases of 3%. The total commitment under the lease is estimated to be approximately \$29.3 million over the 15-year term of the lease. The Company paid a cash security deposit of \$0.3 million to the landlord in connection with the NJ Lease Agreement which has been reflected as part of deposits in the Consolidated Balance Sheets as of March 31, 2026 and December 31, 2025.

Operating Leases

On June 7, 2018, the Company entered into a three-year ESB Lease Agreement. In connection with the ESB Lease Agreement, the Company established an irrevocable standby letter of credit for \$0.8 million. On March 26, 2021, the Company entered into Amendment No. 1 to the ESB Lease Agreement that extended the term of the lease agreement to June 30, 2024. On March 29, 2024, the Company entered into Amendment No. 2 to the ESB Lease Agreement that extended the term of the lease agreement to July 31, 2027. The letter of credit serves as the Company's security deposit on the lease in which the landlord is the beneficiary and expires September 30, 2027.

During the year ended December 31, 2025, the Company began exploring a sublease of the ESB lease and entered negotiations with a sublease tenant. The Company reviewed the recoverability of the related right-of-use asset and determined that the changes in the intended use of the lease represented an impairment indicator as negotiations indicated that the carrying value of the right-of-use asset may not be recoverable. The Company compared the expected future undiscounted cash flows from the negotiations of a sublease to the carrying value of the right-of-use asset and determined that it was not recoverable. The Company calculated the fair value based on the present value of the expected cash flows from a sublease based on the negotiations with a sublease tenant and compared this estimated fair value to its carrying value. This resulted in a right-of-use asset impairment charge of approximately \$0.3 million. The fair value was estimated using level 3 inputs based on an income approach by converting estimated future cash inflows from a sublease to a single present value. Estimated cash flows were discounted at a rate of 8.6% commensurate with the inherent risks associated with the lease asset to arrive at an estimated fair value. The ESB sublease was completed in April 2026. In conjunction with the completion of the sublease, the Company received a security deposit of \$68,000.

The Company has a certificate of deposit of \$0.8 million with a bank as collateral for the ESB Lease Agreement letter of credit which is classified as part of restricted cash in the Consolidated Balance Sheets as of March 31, 2026 and December 31, 2025.

In connection with the acquisition of Renovacor, the Company added operating leases for space at facilities in Hopewell, New Jersey. The Company intends to sublease the facilities in Hopewell, New Jersey and signed the first agreement to sublease one of these facilities in January 2024. Rental income received under sublease agreements was less than \$0.1 million for the three months ended March 31, 2026 and 2025, respectively.

Rent expense excluding rental income was \$0.2 million and \$0.3 million for the three months ended March 31, 2026 and 2025, respectively.

The total restricted cash balance for the Company's operating and finance leases as of March 31, 2026 and December 31, 2025 was \$0.8 million.

The following table summarizes lease cost for the three months ended March 31, 2026 and 2025:

Lease cost	Three Months Ended March 31,	
	2026	2025
Operating lease cost	\$ 226	\$ 262
Finance lease cost:		
Amortization of right of use assets	538	538
Interest on lease liabilities	473	472
Total lease cost	\$ 1,237	\$ 1,272

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

Fiscal Year Ending December 31,	March 31, 2026
2026 (nine months)	\$ 756
2027	791
2028	554
2029	547
2030	557
Thereafter	1,324
Total lease payments	\$ 4,529
Less: interest	(1,038)
Total operating lease liabilities	\$ 3,491

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

Fiscal Year Ending December 31,	March 31, 2026	
2026 (nine months)	\$	1,437
2027		1,969
2028		2,028
2029		2,089
2030		2,152
Thereafter		36,763
Total lease payments	\$	46,438
Less: interest		(25,165)
Total finance lease liability	\$	21,273

The following table summarizes the operating and financing lease liabilities and right-of-use assets as of March 31, 2026 and December 31, 2025:

Leases	March 31, 2026		December 31, 2025	
Operating right-of-use assets	\$	3,127	\$	3,210
Operating current lease liabilities	\$	998	\$	1,003
Operating noncurrent lease liabilities		2,493		2,600
Total operating lease liabilities	\$	3,491	\$	3,603
Finance right-of-use assets	\$	39,671	\$	40,209
Finance current lease liability	\$	1,926	\$	1,912
Finance noncurrent lease liability		19,347		19,362
Total finance lease liability	\$	21,273	\$	21,274

Other Information	Three Months Ended March 31,	
	2026	2025
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases	\$ 255	\$ 250
Cash flows from finance lease	\$ 474	\$ 460

	As of March 31,	
	2026	2025
Weighted-average remaining lease term - operating leases	6.0 years	6.5 years
Weighted-average remaining lease term - finance lease	18.4 years	19.4 years
Weighted-average discount rate - operating leases	8.93%	8.82%
Weighted-average discount rate - finance lease	8.96%	8.96%

13. Commitments and Contingencies

Litigation

On June 11, 2025 and July 18, 2025, two stockholders filed putative securities class action lawsuits against the Company and certain of its executive officers in the United States District Court for the District of New Jersey, purportedly on behalf of classes of the Company's investors who purchased or otherwise acquired the Company's common stock between February 27, 2025 and May 26, 2025 (*Ho v. Rocket Pharmaceuticals, Inc., and Gaurav Shah*, Case No. 3:25-cv-10049) and between September 17, 2024 and May 26, 2025 (*Yankov v. Rocket Pharmaceuticals, Inc., Gaurav Shah and Aaron Ondrey*, 3:25-cv-13532), respectively. The complaints allege violations of Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 and Rule 10b-5 promulgated thereunder in connection with various public statements made by the Company regarding its Phase 2 clinical trial for RP-A501 for Danon disease. The actions seek unspecified damages, costs and expenses, including attorneys' fees. On September 9, 2025, the Court consolidated the two pending putative securities class action lawsuits, appointed two stockholders as co-lead plaintiffs, and approved their selection of co-lead counsel. Pursuant to a stipulation approved by the Court on September 22, 2025, the co-lead plaintiffs filed a consolidated amended complaint on November 18, 2025. The consolidated amended complaint, captioned in re Rocket Pharmaceuticals, Inc. Securities Litigation, 3:25-cv-10049, is brought on behalf of persons who purchased or otherwise acquired the Company's securities during the period of February 28, 2024 through August 25, 2025 (inclusive). Plaintiffs claim that the lawsuit arises from Defendants' public statements and purported omissions concerning Rocket's Phase 2 clinical trial for RP-A501 for DD. Among other things, Plaintiffs allege that Defendants failed to disclose certain SAEs that impacted patients during the Phase 2 clinical trial and the introduction of a C3 inhibitor to the trial's protocol and that Defendants purportedly lacked a viable trial design that could safely and effectively dose patients while managing the risk of serious adverse events. According to Plaintiffs, Rocket's stock price was inflated as a result of these purported misstatements and omissions. We intend to vigorously defend against the consolidated amended complaint's allegations. On January 30, 2026, the Company filed a motion to dismiss the consolidated amended complaint. The Plaintiffs response to the Company's motion was filed on April 1, 2026 and the Defendants reply thereto is due on May 16, 2026. Given the nature of the cases, including that the proceedings are in their early stages, the Company is unable to predict the ultimate outcome of the cases or estimate the range of potential loss, if any.

On October 22, 2025, a putative derivative action was filed in the District of New Jersey, naming as Defendants certain of the Company's officers and present or former directors of the Company. The Complaint (which names the Company as a nominal defendant) alleges that the Defendants engaged in wrongful conduct during the period from September 17, 2024 through May 26, 2025. The allegations in the complaint largely parallel the allegations made in the previously filed putative securities class action complaints, with some additional allegations regarding a supposed lack of internal controls and purported insider trading. The Complaint seeks declaratory relief, an award of damages to the Company, an order directing the Company and the individual defendants to institute certain requested corporate governance reforms, restitution from the individual defendants, and costs and disbursements related to the lawsuit. The parties agreed to stay all proceedings in the putative derivative action until any motions to dismiss the putative securities class action are resolved, and on December 22, 2025, the Court approved the parties' stipulation to that effect. The Company intends to vigorously defend the litigation. The Company will pay the legal fees related to the putative derivative action against the Company's officers and directors. Given the nature of the litigation, including the fact that the litigation is in its early stages, the Company is unable to predict the ultimate outcome of the litigation or estimate the range of potential loss, if any.

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

Indemnification Arrangements

Pursuant to its bylaws and as permitted under Delaware law, the Company has indemnification obligations to directors, officers, employees or agents of the Company or anyone serving in these capacities. Potential indemnification obligations include obligations from lawsuits. 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. Potential damages include damages from lawsuits. The Company has insurance that would allow it to recover a portion of any future amounts that could arise from these indemnifications. As a result, the Company believes that the estimated fair value of these indemnification commitments is minimal.

14. Agreements Related to Intellectual Property

The Company, directly and through its subsidiary Spacecraft Seven, LLC, has various license and research and collaboration arrangements. The transactions principally resulted in the acquisition of rights to intellectual property which is in the preclinical phase and has not been tested for safety or feasibility. In all cases, the Company did not acquire tangible assets, processes, protocols, or operating systems. The Company expenses the acquired intellectual property rights as of the acquisition date when the cost of intangible assets purchased from others has no alternative future uses. The Company incurred \$2.4 million, net, of payment obligations to licensors included in G&A expenses as a result of the approval of KRESLADITM in March 2026.

15. CIRM Grants

DD CIRM Grant

On August 18, 2024, CIRM awarded the Company up to \$5.8 million under a CLIN2 grant award to support the clinical development of its AAV-based gene therapy, RP-A501 for the treatment of DD. Proceeds from the grant would help fund clinical trial costs as well as manufactured drug product for Phase 1/2 patients. During the three months ended March 31, 2026 and 2025, the Company received grants of \$0 and \$2.7 million, respectively, which were recorded as a reduction of R&D expenses. Through March 31, 2026, the Company has received total RP-A501 grants of \$5.0 million from CIRM. No additional milestones were met during the three months ended March 31, 2026.

16. Related Party Transactions

In February 2025, the Company enter into a consulting agreement with one of the Company's board members, effective March 3, 2025, for services related to the Company's research and development activities. As compensation for services rendered during 2025, the consultant received \$125,000 and \$125,000 of RSU's valued as of the closing price on March 3, 2025 which cliff vested on December 31, 2025. The agreement ended on December 31, 2025. The board member was paid approximately \$12,500 for the period ended March 31, 2025 for services provided under the consulting agreement.

In February 2026, the Company entered into an agreement to receive services from a firm whose CEO and founder is the spouse of one the same board member. As compensation for services being rendered during 2026, the firm will receive a total of \$65,000 with \$32,500 paid in February 2026 at inception of the agreement and \$32,500 to be paid upon completion of services.

17. 401(k) Savings Plan

The Company has a defined contribution savings plan (the "Plan") under Section 401(k) of the Internal Revenue Code of 1986. This Plan covers substantially all employees who meet minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre-tax basis. Company contributions to the Plan may be made at the discretion of the Company's Board of Directors. The Company has elected the safe harbor match of 4% of employee contributions to the Plan, subject to certain limitations. The Company's matching contribution for the three months ended March 31, 2026 and 2025, was \$0.3 million and \$0.5 million, respectively.

18. Segment Reporting

The Company has one reportable segment related to R&D and commercial readiness of its gene therapies.

The Company's CODM is its Chief Executive Officer and the senior leadership team. The CODM manages the Company's operations on an integrated basis for the purpose of allocating resources. When evaluating the Company's financial performance, the CODM regularly reviews total expenses and expenses by significant areas to make decisions on a company-wide basis. Included in these expenses are R&D expenses by program.

The table below is a summary of the segment loss, including significant segment expenses:

	Three Months Ended March 31,	
	2026	2025
Revenue	\$ -	\$ -
Operating expenses:		
Research and development	31,454	35,942
Non-commercial general and administrative	11,375	20,615
Commercial general and administrative	5,682	7,831
Total operating expenses	<u>48,511</u>	<u>64,388</u>
Loss from operations	(48,511)	(64,388)
Interest expense	(473)	(472)
Interest and other income, net	161	1,336
Accretion of discount on investments, net	1,229	2,190
Net Segment loss and Net loss	<u>\$ (47,594)</u>	<u>\$ (61,334)</u>

The Company's CODM uses net loss to evaluate past spending and to guide decisions regarding future spending. Net loss is used to monitor budget versus actual results. The CODM also uses net loss in analysis of programs and along with the monitoring of budgeted versus actual results in assessing performance of the segment and in establishing manager's compensation. The measure of segment assets is reported on the balance sheet as total assets.

19. Restructuring

In June 2025, the Company's Board of Directors approved a restructuring plan to prioritize investments in its AAV platform and reduce overall cash spending, which was communicated to employees before the end of June 2025. The restructuring included a reduction of the Company's workforce by approximately 70 employees.

As a result of the restructuring, the Company incurred aggregate charges of approximately \$3.2 million in restructuring costs related to severance and employee termination costs that was paid out over multiple months.

The following table summarizes the accrued liabilities activity in connection with the restructuring plan for the three months ended March 31, 2026:

	For the Three Months Ended	
	March 31, 2026	
Beginning balance	\$ 46	46
Restructuring charges eliminated during the period		(46)
Remaining accrual at March 31, 2026	<u>\$ -</u>	<u>-</u>

20. Subsequent Events

On April 26, 2026, the Company entered into a definitive agreement to sell its PRV for \$180 million. The PRV was awarded by the FDA in connection with the accelerated approval of KRESLADI™ (marnetegrane autotemcel). The transaction remains subject to customary closing conditions, including the expiration or termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976.

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

You should read the following discussion and analysis of our financial condition and results of operations together with the consolidated financial statements and related notes that are included elsewhere in this Quarterly Report on Form 10-Q and our annual report on Form 10-K, filed on February 26, 2026, with the SEC.

Some of the statements contained in this discussion and analysis or set forth elsewhere in this quarterly report on Form 10-Q, including information with respect to our plans and strategy for our business, constitute forward looking statements within the meaning of Section 27A of the Securities Act of 1933, as amended (the "Securities Act"), and Section 21E of the Securities Exchange Act of 1934, as amended (the "Exchange Act"). We have based these forward-looking statements on our current expectations and projections about future events. The following information and any forward-looking statements should be considered in light of factors discussed elsewhere in this quarterly report on Form 10-Q particularly including those risks identified in Part II, Item 1A "Risk Factors" and our other filings with the Securities and Exchange Commission (the "SEC").

Our actual results and timing of certain events may differ materially from the results discussed, projected, anticipated, or indicated in any forward-looking statements. We caution you that forward-looking statements are not guarantees of future performance and that our actual results of operations, financial condition and liquidity, and the development of the industry in which we operate may differ materially from the forward-looking statements contained in this quarterly report on Form 10-Q. Statements made herein are made as of the date of the filing of this Form 10-Q with the SEC and should not be relied upon as of any subsequent date. Even if our results of operations, financial condition and liquidity, and the development of the industry in which we operate are consistent with the forward-looking statements contained in this quarterly report on Form 10-Q, they may not be predictive of results or developments in future periods. We disclaim any obligation, except as specifically required by law and the rules of the SEC, to publicly update or revise any such statements to reflect any change in our expectations or in events, conditions or circumstances on which any such statements may be based or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements.

We caution readers not to place undue reliance on any forward-looking statements made by us, which speak only as of the date they are made.

Overview

Rocket Pharmaceuticals is a fully integrated commercial-stage biotechnology company focused on the development, manufacturing, and commercialization of genetic therapies for rare diseases. Our multi-platform approach is designed to develop gene therapies that address the underlying genetic causes of disease, with a strategic focus on inherited cardiovascular conditions and select hematologic disorders. Our platform is supported by in-house research and development capabilities and cGMP manufacturing facilities that enable end-to-end control of clinical production, process development, and scale-up for commercialization.

The Company’s activities during the quarter reflect continued execution across its prioritized cardiovascular gene therapy programs, alongside the FDA accelerated approval of KRESLADI™ in March 2026 for the treatment of severe LAD-I in pediatric patients without a suitable human leukocyte antigen (HLA)-matched sibling donor. The Company is advancing commercial readiness activities, including treatment center onboarding, supply chain preparation, and coordination of vein-to-vein logistics, in advance of anticipated product availability. Given the ultra-rare patient population and anticipated phased commercial rollout, the Company does not expect KRESLADI™ to generate material revenue in the near term.

We aim to develop and commercialize genetic therapies for rare diseases with significant unmet medical need. As a fully integrated, commercial-stage biotechnology company, we have the resources and opportunity to generate a portfolio of highly differentiated and potentially first-in-class or best-in-class genetic medicines.

In July 2025, we announced a strategic corporate reorganization and pipeline prioritization initiative designed to maximize near-term value creation, extend our operational runway, and position the Company for sustainable long-term growth. The initiative focused resources on advancing our AAV cardiovascular gene therapy platform and supporting the submission of our response to the FDA’s CRL for KRESLADI™. As part of this strategic realignment, we de-prioritized further development activities related to our FA and PKD programs and implemented a workforce reduction of approximately 30%.

In March 2026, KRESLADI™ (marnetegragene autotemcel) received accelerated approval from the FDA for the treatment of pediatric patients with severe LAD-I who do not have an available HLA-matched sibling donor. In connection with the approval, the Company was awarded a PRV and, in April 2026, entered into a definitive agreement to sell the PRV for \$180 million. The Company intends to pursue a focused commercial strategy for KRESLADI™ that is appropriately scaled to the exceptionally small patient population affected by this ultra-rare disease.

Our strategy is built on several foundational pillars:

- **First-and-Best-in-Class Approach:** With our program selection, we apply a rigorous, disease-based selection approach to identify and prioritize programs: targeting complex genetic disorders with differentiated therapies that offer the potential to be first-, best-, or only-in-class, focusing on monogenic disease with on-target mechanisms of action to directly address the root cause of the disease to offer superior clinical profiles, and choosing indications with sizable market opportunities to enable broad patient impact and sustainable value creation.
- **Strategic Focus on Rare Cardiovascular Indications:** Our near-term research and platform investments are focused on leveraging our AAV capabilities in rare cardiovascular diseases. Collectively, our clinical cardiovascular gene therapy programs target the major genetically defined causes of hypertrophic, arrhythmogenic, and dilated cardiomyopathies which represent a significant portion of inherited heart disease and impact more than 100,000 patients in the U.S. and EU.
- **Late-Stage Science & Innovation with Robust Capabilities:** We are advancing promising clinical programs designed to support regulatory approvals in the U.S. and Europe, with potential expansion into Asia and beyond. To support our clinical and future commercial endeavors, we are currently operating a ~100,000 sq. ft. U.S.-based in-house AAV cGMP manufacturing facility in Cranbury, New Jersey.
- **Expertise & Collaboration:** Our leadership team brings a proven track record of over 20 successful U.S. and international drug approvals and launches with expertise in cell and gene therapies and rare diseases. We collaborate closely with scientific experts, healthcare providers, payors, and patient communities to ensure our therapies address real-world needs.

In the near- and medium-term, we are focused on:

- Advancing our first-in-class product candidates targeting monogenic cardiovascular diseases with substantial unmet need from pre-IND to registrational trials.
- Continuing to build and scale proprietary in-house analytics, process development, and manufacturing capabilities to support clinical and commercial supply.
- Evaluating potential strategic partnerships or other transactions for certain non-core programs to enable continued development, regulatory approval, and commercialization.

In the medium- and long-term, pending favorable data, we plan to:

- Submit BLAs for certain of our clinical programs.
- Expand our gene therapy platform to additional indications compatible with our technologies and core strategy.
- Pursue potential eligibility for FDA priority review voucher programs.

Gene Therapy Overview

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.

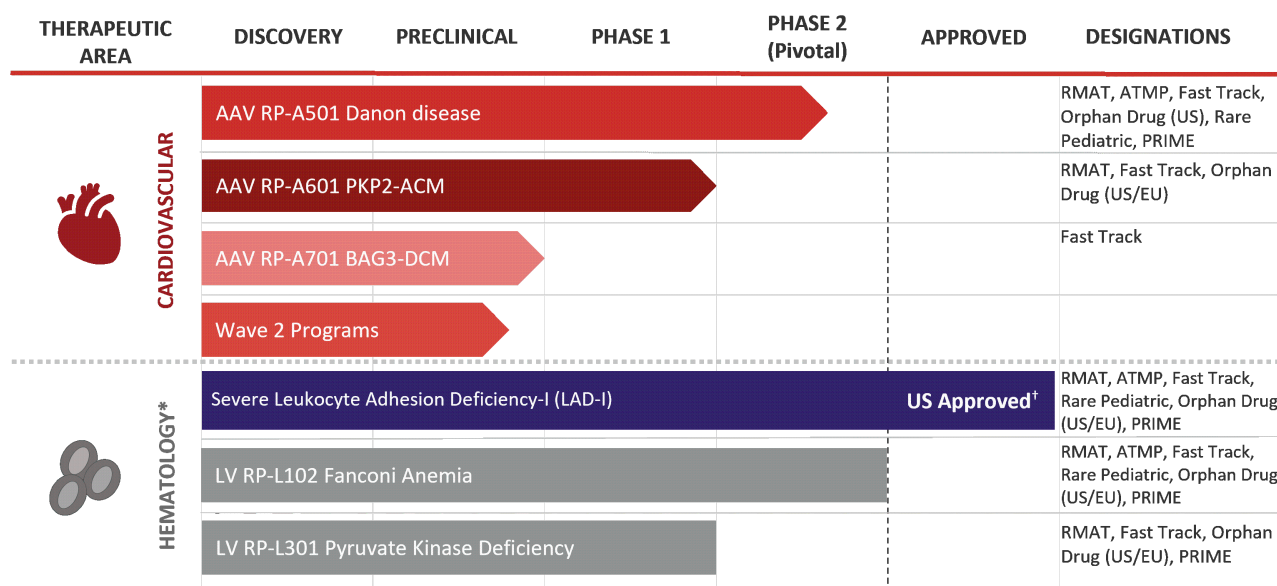
We are developing gene therapy product candidates utilizing modified, non-pathogenic viruses as delivery vehicles. Viruses are inherently effective for gene delivery due to their natural ability to enter cells and deliver genetic material. In engineering our viral vectors, the native viral genes are removed and replaced with a functional copy of the missing or mutated gene responsible for a patient's genetic disorder. This functional copy, known as the therapeutic gene or "transgene," is introduced through a process known as transduction. Once modified, the virus is termed a "viral vector," capable of delivering the transgene to targeted tissues or organs.

We are advancing gene therapy programs using two primary vector approaches: adeno-associated virus (AAV) vectors and lentiviral (LV) vectors. We believe our AAV- and LV-based programs have the potential to provide meaningful and durable therapeutic benefit by addressing the underlying genetic cause of disease. Our gene therapy product candidates are administered either (1) *in vivo*, in which an AAV vector is delivered directly to the patient, either systemically or through targeted tissue delivery, to enable *in situ* transduction of the desired cell populations, or (2) *ex vivo*, in which a patient's hematopoietic stem cells (HSCs) are collected, genetically modified with an LV vector in a controlled laboratory environment, and then reinfused into the patient.

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

Pipeline Overview

The chart below shows the current phases of development of our programs and product candidates:



*Rocket prioritized approval of the severe LAD-I program within its LV portfolio; external partnership opportunities for RP-L102 and RP-L301 are under evaluation.

[†]Accelerated Approval (AA) granted by FDA on March 26, 2026, for severe LAD-I.

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

Cardiovascular Programs

Danon disease

Danon disease (DD) is a rare X-linked inherited, multi-organ lysosomal-associated disorder with a devastating clinical course. The causative mutation has been identified in the gene encoding for lysosome-associated membrane protein, otherwise known as *LAMP2*, an important mediator of autophagy and primarily expressed in heart, skeletal muscle and brain tissue. This mutation results in the accumulation of autophagic vacuoles, predominantly in cardiac and skeletal muscles. Male patients typically die during adolescence or early adulthood from progressive heart failure in the absence of heart transplant. Along with severe cardiomyopathy, other DD-related manifestations can include skeletal muscle weakness and intellectual impairment. There are no specific therapies available for the treatment of DD and medications typically utilized for the treatment of HF are not believed to modify progression to end-stage HF. Patients with end-stage HF may undergo heart transplant, which currently is available to a minority of patients, is associated with significant short- and long-term complications and is not curative of the disorder in the long-term. It is estimated to have a prevalence of 15,000 to 30,000 patients in the U.S. and Europe.

RP-A501 is our investigational gene therapy for the treatment of DD and consists of a recombinant adeno-associated serotype 9 (AAV9) capsid containing a full-length, wild-type version of the human *LAMP2B* transgene which is administered as a single intravenous (IV) infusion. RP-A501 holds FDA RMAT, Fast Track, Rare Pediatric, and Orphan Drug designations in the U.S. along with ATMP and PRIME designations in the EU.

We treated seven patients in the single-arm, open-label, multi-center Phase 1 clinical trial assessing the safety and preliminary efficacy of RP-A501, which enrolled adult/older adolescent and pediatric male DD patients. This includes a first cohort evaluating a low-dose (6.7e13 genome copies/kilogram ([gc/kg]; n=3) in adult/older adolescent patients aged 15 or greater, a second cohort evaluating a higher dose (1.1e14 gc/kg; n=2) in adult/older adolescent patients aged 15 or greater, and a pediatric cohort at a low dose level (6.7e13 gc/kg; n=2).

We conducted a variety of efficacy assessments in the Phase 1 clinical study to measure the prospect of benefit for patients. These assessments included the following:

- *LAMP2* gene expression in endomyocardial biopsy samples is measured via both immunohistochemistry and Western blot and confirms the presence of *LAMP2* protein in DD cardiac tissue following RP-A501 treatment.
- Measurements of heart thickness, most notably, left ventricular mass and maximal left ventricular wall thickness, indicate the degree of hypertrophy present in the heart
- High sensitivity troponin I or hs-TnI and BNP are blood-based evaluations and a key marker of HF and cardiac injury. Both are frequently elevated in DD patients and have been shown to be markedly elevated in patients with advanced stage disease.
- KCCQ-12 is a patient-reported quality-of-life assessment that measures a patient's perception of their HF symptoms, impact of disease on physical and social function, and the impact of their HF on overall health status and quality of life. Assessment scores range from 0 (very poor health status) to 100 (excellent health status). Changes in KCCQ-12 score of +/- 5 points are considered meaningful and have been shown to correlate with HF outcomes.
- NYHA Functional Classification is the most commonly used HF classification system. NYHA Class I reflects the absence of clinical signs of HF, while NYHA Class II is where a patient exhibits a slight limitation of physical activity, is comfortable at rest, and ordinary physical activity results in fatigue, palpitation and/or dyspnea. NYHA Class III and IV are considered more severe or advanced HF.
- Histologic examination of endomyocardial biopsies via hematoxylin and eosin histology and electron microscopy is used to detect evidence of DD-associated tissue derangements, including the presence of autophagic vacuoles and disruption of myofibrillar architecture, each of which are characteristic of DD-related myocardial damage.

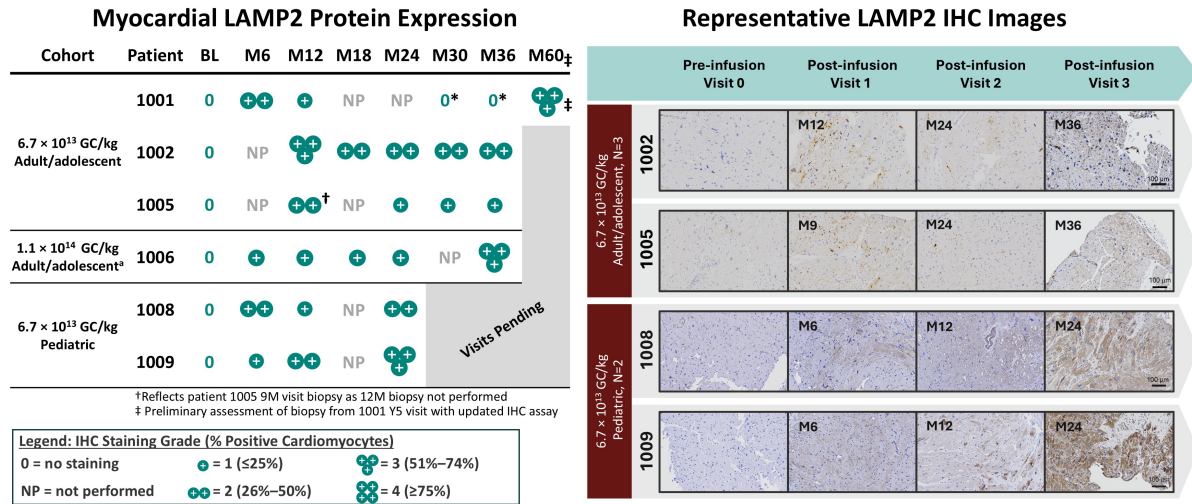
As previously announced, a patient receiving therapy on the high dose cohort (1.1e14 gc/kg dose) had progressive HF and underwent a heart transplant at month five following therapy. This patient had more advanced disease than the four other adult/older adolescent patients who received treatment in the low and high dose cohorts, as evidenced by diminished baseline left ventricle ejection fraction (32%) on echocardiogram and markedly elevated left ventricle filling pressure prior to treatment. The patient's clinical course was characteristic of DD progression. The patient is doing well post-transplant.

Based on the initial efficacy observed in the low dose cohort and to mitigate complement-mediated safety concerns observed in the high dose cohort (related to thrombotic microangiopathy or TMA) and in agreement with the FDA, the Phase 2 study was initiated at the low dose (6.7e13 gc/kg). Additional safety measures were implemented and are reflected in the updated trial protocol for Phase 1 and the protocol for our ongoing pivotal Phase 2 study. These measures include exclusion of patients with end-stage HF, and a refined immunomodulatory regimen involving transient B- and T-cell mediated inhibition, with emphasis on preventing complement activation, while also enabling lower steroid doses and earlier steroid taper, with all immunosuppressive therapy discontinued 2-3 months following administration of RP-A501.

In November 2024, we announced positive results and presented long-term safety and efficacy results of the Phase 1 study at the American Heart Association's 2024 Late-Breaking Science sessions and simultaneously published these data in the *New England Journal of Medicine*. The long-term safety and efficacy results from the Phase 1 RP-A501 study showed that RP-A501 was generally well tolerated and all evaluable DD patients demonstrated *LAMP2* protein expression at 12 months (sustained up to 60 months) and reduction of left ventricular mass index by $\geq 10\%$ at 12 months (sustained up to 54 months) after treatment. Results from the Phase 1 DD trial represent one of the first and most comprehensive investigational gene therapy datasets for any cardiac condition.

RP-A501 Phase I Study: Sustained LAMP2 Expression in Cardiomyocytes

Durable myocardial LAMP2 protein expression seen in all patients



Data up to month 36 timepoint published in *New England Journal of Medicine* [Greenberg et al 2024; DOI: 10.1056/NEJMoa2412392]; Month 60 data for patient 1001 confirmed after manuscript submission. Patient 1007 had LV systolic dysfunction (LVEF <40%) at enrollment and had progressive heart failure requiring transplantation 5m following RP-A501 treatment; this patient is currently stable 3 years post-transplant. Note: Grading of LAMP2 protein expression by IHC was done by a board-certified pathologist in a blinded fashion. The semi-quantitative grading reflects the extent of LAMP2 protein expressing cardiomyocytes in the entirety of biopsy sample according to the scale: Grade 0, negative staining; Grade 1 = < 25%; Grade 2 = 26%-50%; Grade 3 = 51%-74%; Grade 4 = >75%. IHC=immunohistochemistry; LAMP2=lysosome-associated membrane protein 2; M=month(s); VCN=vector copy number. *Patient 1001 demonstrated Grade 0 LAMP2 protein IHC staining at the 30- and 36- month assessments, however, patient 1001's LAMP2B vector RNA and DNA (VCN) levels have persisted through 36 months of follow-up.

Data from the Phase I study (cut-off April 19, 2024) showed that RP-A501 in conjunction with a transient immunomodulatory regimen was generally well tolerated. Evidence of sustained clinically meaningful improvement was observed in pediatric patients followed up to 24 months and adult/adolescent patients followed up to 60 months.

RP-A501 Phase I Study: Benefit Observed Across All Key Parameters

Early LAMP2, BNP, Tnl changes associated with sustained clinical improvement and guided Phase 2 endpoint selection

Cohort	Patient	Age at Most RV (y)	Most Recent Visit (mo)	LVEF BL to RV (%)	Δ LVMI,* BL to RV (g/m ²)	Δ IVSd, BL to RV (mm)	Δ LVPWd, BL to RV (mm)	Δ NT-proBNP, BL to RV (ng/L)	Δ cTnl,† BL to RV (ng/mL)	Δ NYHA Class	Δ KCCQ-12 OS, BL → RV
1: Low Dose Adult/Adolescent	1001	22.3	54	57 to 64	-33%, 85 to 56.9	-6%, 19.8 to 18.6	-20%, 18.8 to 15	-17%, 336 to 279	-99%, 0.6 to 0.01	II to I	+52, 44 to 96
	1002	24.9	54	55 to 66	-48%, 260.2 to 135.3	-52%, 60.1 to 28.6	-49%, 39.1 to 19.8	-93%, 5119 to 351	-96%, 1.46 to 0.06	II to I	+27, 64 to 91
	1005	21.8	42	65 to 59	-11%, 98.2 to 87.3	-10%, 30.9 to 27.8	-27%, 32.1 to 23.4	+16%, 841 to 975	-33%, 0.28 to 0.19	II to I	+7, 77 to 84
2: High Dose Adult/Adolescent	1006	23.9	36	62 to 51	-7%, 68.6 to 63.6	+5%, 18.0 to 19.0	-27%, 24.0 to 17.4	-65%, 720 to 249	-39%, 0.47 to 0.29	II to I	+9, 79 to 89
3: Low Dose Pediatric	1008	14.4	24	74 to 78	-38%, 141.5 to 87.8	-19%, 42.4 to 34.2	+1%, 22.8 to 23.1	-78%, 1629 [‡] to 360 [‡]	-85%, 1.78 to 0.27	II to I	+27, 50 to 77
	1009	13.7	24	77 to 77	-13%, 82.0 to 71.2	+12%, 18.5 to 20.8	-3%, 14.9 to 14.4	-48%, 1912 to 998	-82%, 1.08 to 0.20	II to I	+30, 52 to 82

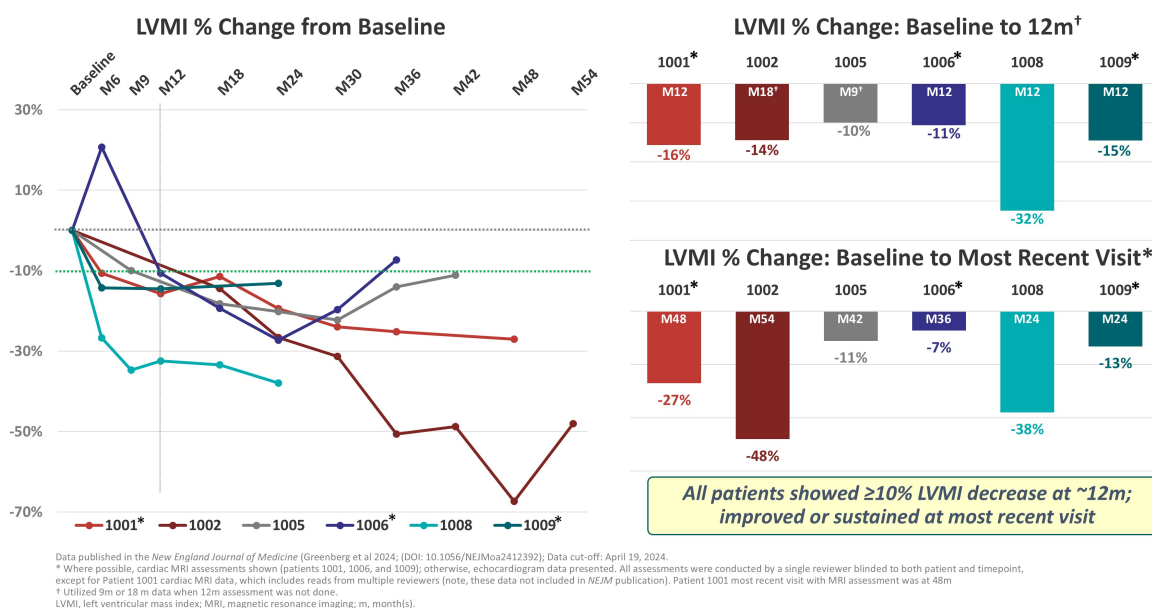
* Centrally evaluated (blinded) MRI data were utilized for LVMI when available. All other measurements of cardiac structure and function reflect centrally evaluated (blinded) echocardiogram data. † Central laboratory assessment of cTnl were performed on cryopreserved and non-cryopreserved samples. Values for cTnl from high-sensitivity and earlier tests. High-sensitivity and earlier assay are expressed in ng/mL. Improved Stabilized Worsened

Data published in the *New England Journal of Medicine* [Greenberg et al 2024; DOI: 10.1056/NEJMoa2412392]; Data cut-off: April 19, 2024. BL=Baseline; BNP=Brain Natriuretic Peptide; cTnl=cardiac troponin I; ICD=Implantable Cardioverter Defibrillator; IVSd=Intraventricular Septum in diastole; KCCQ=Kansas City Cardiomyopathy Questionnaire; NT-Pro-BNP=N-terminal pro-B-type natriuretic peptide; NYHA=New York Heart Association; LV=Left Ventricle; LVEF=Left Ventricular Ejection Fraction; LVMI=Left Ventricular Mass Index; LVPWd=Left Ventricular Posterior Wall in diastole; RV=(Most) Recent Visit.

In summary, all evaluable patients in the Phase 1 trial demonstrated:

- Cardiac *LAMP2* protein expression at 12 months and thereafter;
- Reduction or stabilization of left ventricular mass index (LVMI) – the median reduction from baseline to most recent visit of 24% (for the ongoing pivotal Phase 2 trial, a 10% reduction in LVMI and positive protein expression of Grade 1 or more are co-primary endpoints);
- Preservation of normal left ventricle ejection fraction (LVEF);
- Reduction or stabilization of cardiac biomarkers (median cardiac troponin I [cTnI] and BNP reductions of 84% and 57%, respectively);
- Improvement in NYHA class from Class II at baseline to Class I at most recent follow-up visit;
- Improvements in KCCQ-12 scores (median improvement of 27 points) that persisted up to 54 months of follow-up; and
- Preliminary long-term follow-up assessments for Patient 1001 were positive for immunohistochemical staining and appear to show Grade 3 expression in the heart at the five-year timepoint.

RP-A501 Phase 1 Study: Sustained Improvements in LV Mass Index



In September 2023, we announced that alignment was reached with the FDA on the global Phase 2 pivotal trial of RP-A501 for DD to support accelerated approval. The global, single-arm, multi-center Phase 2 pivotal trial is evaluating the efficacy and safety of RP-A501 in 12 patients with DD, including a pediatric safety run-in (n=2), with a natural history comparator and a dose level of 6.7 x 10¹³GC/kg. A global natural history study is also running concurrently to the Phase 2 pivotal trial and will serve as an external comparator.

To support accelerated approval, the study will assess the efficacy of RP-A501 as measured by the biomarker-based co-primary endpoint consisting of improvements in LAMP2 protein expression (≥ Grade 1, as measured by immunohistochemistry), and reductions in LVMI.

Secondary endpoints include the components of the primary endpoint (improvement in LAMP2 protein expression and reductions in LVMI), reductions in troponin and natriuretic peptide, KCCQ-12 and NYHA class, event free survival and treatment emergent safety events. These endpoints could support full approval with longer-term follow-up.

Drug product for the Phase 2 study is being produced in-house in our GMP manufacturing facility in Cranbury, NJ. We have successfully produced multiple commercial-grade Danon AAV cGMP batches since 2022. Furthermore, we have reached agreement with the FDA on the continued utilization of HEK-293 cell-based process through commercialization, our comparability approach and our potency assay.

In January 2024, we received CTIS approval to include clinical trial sites in certain EU Member States.

In September 2024, we announced completion of enrollment of 12 patients in the Phase 2 study across sites in the U.S. and EU.

In May 2025, two patients participating in the Phase 2 pivotal study of RP-A501 each experienced an unexpected SAE. The SAEs involved clinical complications related to a capillary leak syndrome resulting in multi-organ damage; one patient died as a result of these complications following an acute systemic infection. Rocket voluntarily paused further Phase 2 study dosing in the U.S. and EU, and the FDA subsequently placed the trial on clinical hold on May 23, 2025 to allow for further evaluation. In August 2025, the FDA lifted the clinical hold on the Phase 2 pivotal study following an investigation which concluded that the SAEs were likely the result of the combination of the C3 component inhibitor introduced into the immunomodulation regimen and RP-A501. The FDA authorized resumption of the Phase 2 pivotal study with a recalibrated dose of 3.8×10^{13} GC/kg of RP-A501 along with the first three patients to be treated sequentially with a minimum four-week interval between each treatment. This adjusted dose aligns with the lower range of administered doses that were associated with efficacy across multiple biomarkers, electrocardiogram and clinical endpoints in the Phase 1 study, and has been determined as most likely to confer the safety and efficacy identified in the low-dose Phase 1 cohorts.

Prior to the clinical hold, six patients with Danon disease were treated with RP-A501 in the Phase 2 study. Further updates on the Phase 2 study can be expected following review of data from the next three patients.

Plakophilin-2 Arrhythmogenic Cardiomyopathy

Plakophilin-2 related Arrhythmogenic cardiomyopathy, otherwise known as PKP2-ACM is an inheritable cardiac disorder caused by pathogenic variants in the *PKP2* gene that is characterized by a high propensity for arrhythmias and sudden cardiac death. Most commonly, the cardiomyopathy initially manifests in the right ventricular free wall, so the disease was originally termed arrhythmogenic right ventricular dysplasia/cardiomyopathy or ARVD/C. However, since left dominant and biventricular forms have also been observed, this has led more recently to the use of the term ACM. Mutations in the *PKP2* gene comprise the most frequent genetically identified etiology of familial ACM. Patients with mutations in *PKP2* are typically heterozygous and demonstrate reduced expression of the PKP2 protein in the myocardium. *PKP2* encodes for the protein Plakophilin-2, which is a component of the desmosome, an intercellular complex involved in cell-cell adhesion. The PKP2 protein is also involved in transcriptional regulation of calcium signaling between cardiomyocytes. PKP2-ACM is most commonly diagnosed in young adults with the mean age presentation at 35 years old, and patients have a very high lifetime risk of ventricular arrhythmias, structural ventricular abnormalities, and SCD.

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

RP-A601 is our investigational gene therapy for the treatment of PKP2-ACM and consists of a recombinant adeno-associated serotype rh74 capsid containing a functional version of the human *PKP2* transgene (AAVrh74.PKP2) which is administered as a single IV infusion. RP-A601 holds FDA RMAT and Fast Track and designations in the US and Orphan drug designations in both the U.S. and EU.

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

Enrollment in the U.S. Phase 1 study is ongoing, and the trial remains open and actively enrolling to further characterize biological activity across a broader range of disease severity. The ongoing single-arm, open-label, multi-center Phase 1 study is evaluating the safety and preliminary efficacy of RP-A601 in adult PKP2-ACM patients with ICDs and overall high risk for arrhythmias. To date, three patients have been treated in the study and is assessing the impact of RP-A601 on PKP2 myocardial protein expression, cardiac biomarkers, and clinical predictors of life-threatening ventricular arrhythmias and SCD. Patients in the Phase 1 study received a single dose of RP-A601 starting at 8×10^{13} GC/kg. We are continuing to work closely with the FDA to advance alignment on the design and potential endpoints of a pivotal Phase 2 trial of a pivotal Phase 2 trial intended to further evaluate the safety and efficacy of RP-A601 in this patient population.

In May 2025, we presented preliminary data from the Phase 1 study of RP-A601 for adult patients with PKP2-ACM at the ASGCT 28th Annual Meeting in the Late-Breaking Scientific Sessions. Initial data from the Phase 1 study (safety cut-off May 6, 2025; efficacy cut-off April 2025) showed that RP-A601 was generally well-tolerated with no dose-limiting toxicities observed in all patients followed for up to 12 months. Most treatment emergent adverse events were mild/moderate in severity and self-limited with only one patient experiencing an SAE which resolved without clinical sequelae within two months post-treatment, believed to be associated with the immunomodulatory regimen.

Cardiac biopsies showed RP-A601 increased PKP2 protein expression in all three patients. In the patients with low baseline PKP2 expression (n=2), improvements in PKP2 protein expression relative to total cell protein were approximately 110% and 398%, respectively, from baseline to six months follow-up. In all three patients, RP-A601 promoted desmosome localization of PKP2 and associated transmembrane interpolated disc proteins between 3 and 12 months after treatment. In addition, preliminary observations suggest potential improvement or stabilization in arrhythmia burden, cardiac function, and quality of life, although these findings are based on a limited number of patients and require further evaluation. Based on available data to date, we have selected 8×10^{13} GC/kg as the dose to be further evaluated in subsequent clinical development, and we do not currently plan to evaluate higher dose levels in this study.

BAG3 Dilated Cardiomyopathy

DCM is the most common form of cardiomyopathy and is characterized by progressive thinning of the walls of the heart resulting in enlarged heart chambers that are unable to pump blood. A familial association of DCM can be identified in 20-50% of DCM patients, with up to 40% of familial patients having an identifiable genetic cause. Mutations in the *BAG3* gene are among the more common pathogenic genetic variants observed in familial DCM and these variants are highly penetrant, with approximately 80% of individuals with disease-causing genetic variants in the *BAG3* gene developing DCM at > 40 years of age. Pathogenic variants in *BAG3* are estimated to cause from 2.3% to 6.7% of DCM cases in the U.S., Europe, and Japan. BAG3 protein is associated with a variety of cellular functions including cardiac contractility, protein quality control (as a co-chaperone), cardiomyocyte structural support and anti-apoptosis. BAG3-DCM leads to early onset, rapidly progressing heart failure and significant mortality and morbidity. The age of diagnosis in BAG3-DCM varies from adolescence to adulthood, with the mean age at clinical diagnosis in the mid-30s. The prevalence of BAG3-associated DCM in the U.S. is estimated to be as many as 30,000 individuals.

DCM represents a considerable unmet medical need and is the most common underlying diagnosis in patients undergoing heart transplantation. No currently approved therapies are specifically indicated to address BAG3-DCM. Medical management of patients with DCM follows the clinical guidelines for HF with reduced ejection fraction (HFrEF), including the use of beta-adrenergic receptor antagonists (beta-blockers), angiotensin converting enzyme (ACE) inhibitors, angiotensin receptor antagonists/nepriylisin inhibitors, mineralocorticoid antagonists, and inhibitors of the sodium-glucose cotransporter-2 (SGLT2), along with antiarrhythmic medications, implanted defibrillator, and/or ablation procedures as indicated. Heart transplantation is the only potentially definitive therapy; however, it is not considered curative and is associated with considerable morbidity and mortality. An effective and safe gene therapy to restore normal BAG3 cardiac protein levels may represent a viable therapeutic option which could substantially reduce morbidity/mortality in BAG3-DCM patients. Understanding the genetic mechanism of disease creates the opportunity to develop precision-based therapies potentially corrective of the underlying molecular defect.

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

In June 2025, the Company received FDA clearance of an IND application for RP-A701, an AAVrh74-based gene therapy candidate for the treatment of BAG3-DCM. In July 2025, the FDA granted Fast Track designation to RP-A701 for the treatment of BAG3-DCM. The first-in-human Phase 1 clinical trial will be a multi-center, dose-escalation study designed to evaluate the safety, biological activity, and preliminary efficacy of RP-A701 in adults with BAG3-DCM.

Hematology Programs

Leukocyte Adhesion Deficiency-I

LAD-I is a rare autosomal recessive disorder of white blood cell adhesion and migration caused by mutations in the *ITGB2* gene, which encodes the beta-2 integrin component, CD18. Deficiency of CD18 impairs the ability of neutrophils (a subset of infection-fighting white blood cells) to exit the bloodstream and migrate to sites of infection. As with many rare diseases, precise estimates of incidence are difficult to determine; however, several hundred cases across the spectrum of severity have been reported to date. Most patients are believed to have the severe form of the disease, which is characterized by recurrent, life-threatening infections and substantial infant mortality in the absence of allogeneic hematopoietic stem cell transplantation (HSCT). Mortality for severe LAD-I has been reported to be 60% to 75% by age two without allogeneic HSCT.

KRESLADI™, formerly known as RP-L201 (marnetegrane autotemcel), is our gene therapy consisting of autologous (patient-derived) hematopoietic stem cells genetically modified with a LV to deliver a functional copy of the *ITGB2* gene. The program has received RMAT, Rare Pediatric Disease, and Fast Track designations from the FDA, as well as PRIME and ATMP designations in the European Union, and Orphan Drug designations in both the U.S. and EU. KRESLADI™ was in-licensed from the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT), Centro de Investigación Biomédica en Red de Enfermedades Raras, and Instituto de Investigación Sanitaria Fundación Jiménez Díaz. The lentiviral vector was developed in collaboration with University College London and CIEMAT.

An open-label, single-arm, global Phase 1/2 registration-enabling clinical trial of RP-L201 in severe LAD-I treated nine patients. Updated follow-up data presented in May 2024 at the ASGCT 27th Annual Meeting (data cut-off July 24, 2023) included 18- to 45-month follow-up. Compared to pre-treatment history, treated patients demonstrated reductions in significant infections requiring hospitalization or intravenous antimicrobials, along with evidence of resolution of LAD-I-related skin and periodontal lesions and restoration of wound healing. RP-L201 was generally well tolerated, with no new treatment-related safety events reported. All treated patients were alive without the need for allogeneic transplant at last follow-up, including those enrolled at less than 12 months of age who surpassed 24 months without transplant. Clinical outcome data from the nine patients treated with KRESLADI™ were published in the *New England Journal of Medicine* in May 2025.

In September 2023, the FDA accepted a Biologics License Application (BLA) for RP-L201 and granted priority review, with an initial PDUFA date of March 31, 2024. In February 2024, the FDA extended the review period by three months to June 30, 2024 to allow additional time to review clarifying CMC information. In June 2024, the FDA issued a complete response letter (CRL) requesting limited additional CMC information. In October 2025, the FDA accepted the Company's resubmission of the BLA and assigned a PDUFA date of March 28, 2026.

On March 26, 2026, the FDA granted accelerated approval to KRESLADI™ for the treatment of severe LAD-I in pediatric patients with biallelic mutations in the *ITGB2* gene who do not have a suitable HLA-matched sibling donor. The approval was based on an increase in neutrophil CD18 and CD11a surface expression. Continued approval may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

On April 26, 2026, the Company entered into a definitive agreement to sell its PRV that was originally issued in connection with the FDA's approval of the BLA for KRESLADI™ for \$180 million. The transaction is subject to customary closing conditions, including the expiration or termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976.

Fanconi Anemia

FA is a rare and life-threatening DNA-repair disorder, characterized by bone marrow failure, cancer predisposition, and congenital malformations. Patients with FA have a genetic defect that prevents the normal repair of genes and chromosomes within blood cells in the bone marrow. The prevalence of FA in the U.S. and the EU is estimated to be approximately 5,500 to 7,000 patients.

Although improvements in allogeneic (donor-mediated) HSCT, currently the most frequently utilized therapy for FA, have resulted in frequent hematologic correction of the disorder, HSCT is associated with both acute and long-term risks, including transplant-related mortality, graft failure, and graft versus host disease, a sometimes fatal side effect of allogeneic transplant characterized by painful ulcers in the GI tract, liver toxicity and skin rashes, as well as increased risk of subsequent cancers. Our gene therapy program in FA is designed to enable a minimally toxic hematologic correction using a patient's own stem cells early in the disease course and administered without conditioning. We believe that the development of a broadly applicable autologous gene therapy can be transformative for these patients. In light of the efficacy seen in non-conditioned patients, the addressable annual market opportunity is now believed to be 400 to 500 patients collectively in the U.S. and EU.

RP-L102 is our investigational LV vector-based gene therapy for the treatment of FA. RP-L102's LV carries the *FANCA* gene as part of the PGK-FANCA-WPRE expression cassette which includes a phosphoglycerate kinase (PKG) promoter and an optimized woodchuck hepatitis virus post transcriptional regulatory element (WPRE). The Phase 2 study of RP-L102 for the treatment of FA type A without the use of myeloablative conditioning treated a total of 14 patients from the U.S. and EU. Patients received a single intravenous infusion of RP-L102 that utilizes fresh cells and an improved process which incorporates a modified stem cell enrichment process, transduction enhancers, as well as commercial-grade vector and final drug product. The Company holds FDA RMAT, Rare Pediatric, and Fast Track designations in the U.S., PRIME and ATMP designations in the EU, and Orphan Drug designations in both regions for the program.

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

In May 2024, we provided an incremental clinical update at the ASGCT 27th Annual Meeting (data cut-off September 11, 2023). RP-L102 continued to demonstrate sustained genetic correction, phenotypic correction, and hematologic stability in 8 of 12 patients with greater than 12 months of follow-up. RP-L102 continued to be well tolerated with no significant safety signals.

As of July 2025, the Company is no longer allocating additional internal resources towards regulatory filings and commercial activities for RP-L102 and subsequently is no longer pursuing BLA and EMA submissions for RP-L102. The Company is actively exploring external partnership options to provide a path forward for RP-L102 and the FA community. This decision was based solely on business and strategic considerations and does not reflect any concerns regarding the safety, efficacy, or quality of the therapy.

Pyruvate Kinase Deficiency

PKD is a rare, autosomal recessive, monogenic red blood cell disorder resulting from a mutation in the *PKLR* gene encoding for the pyruvate kinase enzyme, a key component of the red blood cell glycolytic pathway. Mutations in the *PKLR* gene result in increased red blood cell destruction and potentially life-threatening anemia with a significant impact on quality of life. PKD has an estimated prevalence of 4,000 to 8,000 patients in the U.S. and Europe.

RP-L301 is our investigational gene therapy that contains autologous hematopoietic stem cells that have been genetically modified with a lentiviral vector to contain a functional copy of the *PKLR* gene for the treatment of PKD. The Company holds FDA RMAT and Fast Track designations in the U.S., EMA PRIME designation in the EU, and Orphan Drug designation in both regions for the program. RP-L301 was in-licensed from CIEMAT, Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER) and Instituto de Investigación Sanitaria de la Fundación Jiménez Díaz (IIS-FJD).

A global Phase 1 open-label, single-arm, clinical study with 2 adult patients and 2 pediatric patients (age 8-17) in the U.S. and Europe assessed the safety, tolerability, and preliminary activity of RP-L301. Stanford served as the site in the U.S. for adult and pediatric patients, HNJ served as the lead site in Europe for pediatrics, and Hospital Universitario Fundación Jiménez Díaz served as the lead site in Europe for adult patients.

In February 2024, we presented further clinical updates at the ASGCT 27th Annual Meeting (data cut-off February 5, 2024), which included 36 months of follow-up on the two adult patients and 12 months of follow-up on the two pediatric patients. Sustained and clinically meaningful hemoglobin improvement was observed in all patients including hemoglobin normalization in three of four patients. No patients have required red blood cell transfusions following neutrophil engraftment. Improvements in hemoglobin supported by improved markers of hemolysis and quality of life have been observed. RP-L301 remains well-tolerated, with no drug-related serious adverse events. Insertion site analyses in the peripheral blood and bone marrow for both adult patients through 36 months post-RP-L301 continued to demonstrate highly polyclonal patterns with no clonal dominance or insertional mutagenesis.

Based on positive safety and efficacy data from the Phase 1 study, we have aligned with the FDA on the pivotal study design to support accelerated approval with a 10-patient, single-arm Phase 2 pivotal trial with a primary endpoint of ≥ 1.5 point hemoglobin Hgb improvement at 12 months. However, the Company is no longer allocating internal resources towards RP-L301 and does not plan to initiate enrollment in a Phase 2 RP-L301 study at this time. Similar to our FA program, we are actively exploring external partnership options to provide a path forward for RP-L301 and the PKD community.

Future Opportunities

In addition to the programs specified in this Quarterly Report, we are also conducting exploratory preclinical R&D. Research focus areas include the development of new candidates following our strategy outlined in "Overview" section.

cGMP Manufacturing

We have a 103,720 square foot manufacturing facility located in Cranbury, New Jersey. This facility supports clinical development of our pipeline of AAV gene therapy product candidates from discovery through pivotal trials, with space for potential future expansion and commercialization.

Financial Overview

Since our inception, we have devoted substantially all of our resources to organizing and staffing the Company, business planning, raising capital, acquiring or discovering product candidates and securing related intellectual property rights, conducting discovery and R&D activities for our product candidates, and preparing for commercialization.

KRESLADI™ was approved by the FDA in March 2026 under the accelerated approval pathway; however, we have not yet generated revenue from product sales. Given the ultra-rare patient population and anticipated phased commercial rollout, we do not expect KRESLADI™ to generate material revenue in the near term.

From inception through March 31, 2026, we have raised net cash proceeds of approximately \$1.2 billion from investors through equity and convertible debt financings to fund our operations.

In April 2026, the Company entered into an agreement to sell its PRV for \$180 million, providing non-dilutive capital to support advancement of its cardiovascular gene therapy pipeline.

Revenue

We have not generated revenue from product sales to date. KRESLADI™ was approved by the FDA in March 2026 under the accelerated approval pathway; however, given the ultra-rare patient population and anticipated phased commercial rollout, we do not expect KRESLADI™ to generate material revenue in the near term.

If our development efforts for additional product candidates are successful and result in regulatory approvals or commercialization through third-party collaborations, we may generate revenue in the future from product sales or other arrangements.

Research and Development Expenses

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

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

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

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

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

We allocate salary and benefit costs directly related to specific programs. We do not allocate personnel-related discretionary bonus or stock-based compensation costs, costs associated with our general discovery platform improvements, depreciation or other indirect costs that are deployed across multiple projects under development and, as such, the costs are separately classified as other R&D expenses.

The following table presents R&D expenses tracked on a program-by-program basis as well as by type and nature of expense for the three months ended March 31, 2026 and 2025:

	Three Months Ended March 31,	
	2026	2025
Direct Expenses:		
Danon Disease (AAV) RP-A501	\$ 5,072	\$ 886
Plakophilin-2 Arrhythmogenic Cardiomyopathy (AAV) RP-A601	1,710	1,811
Leukocyte Adhesion Deficiency (LV) RP-L201	1,684	4,003
Fanconi Anemia (LV) RP-L102	1,372	6,020
Pyruvate Kinase Deficiency (LV) RP-L301	417	1,090
Other product candidates	2,351	346
Total direct expenses	12,606	14,156
Unallocated Expenses:		
Employee compensation	10,324	12,239
Stock-based compensation expense	4,307	4,388
Depreciation and amortization expense	1,283	1,864
Laboratory and related expenses	732	914
Professional fees	1,143	1,060
Other expenses	1,059	1,321
Total other research and development expenses	18,848	21,786
Total research and development expense	\$ 31,454	\$ 35,942

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

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

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

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

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

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

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

General and Administrative Expenses

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

Restructuring Expense

In June 2025, the Company's Board of Directors approved a restructuring plan to reduce the Company's workforce and incurred aggregate charges of approximately \$3.2 million in restructuring expenses, consisting of employee severance payments and other termination benefits.

Interest Expense

Interest expense for the three months ended March 31, 2026 and 2025 was related to our financing lease obligation for our Cranbury, NJ facility.

Interest and Other Income

Interest and other income for the three months ended March 31, 2026 was related to interest earned from investments and cash equivalents. Interest and other income for the three months ended March 31, 2025 was related to interest earned from investments and cash equivalents and reduced fair value of warrant liability.

Critical Accounting Policies and Significant Judgments and Estimates

There have been no material changes in our critical accounting policies and estimates in the preparation of our consolidated financial statements during the three months ended March 31, 2026 compared to those disclosed in our 2025 Form 10-K.

Results of Operations

Comparison of the Three Months Ended March 31, 2026 and 2025

The following table summarizes our results of operations, in thousands, for each of the periods presented:

	Three Months Ended March 31,		
	2026	2025	Change
Operating expenses:			
Research and development	\$ 31,454	\$ 35,942	\$ (4,488)
General and administrative	17,057	28,446	(11,389)
Total operating expenses	<u>48,511</u>	<u>64,388</u>	<u>(15,877)</u>
Loss from operations	(48,511)	(64,388)	15,877
Interest expense	(473)	(472)	(1)
Interest and other income, net	161	1,336	(1,175)
Accretion of discount on investments, net	1,229	2,190	(961)
Total other income, net	<u>917</u>	<u>3,054</u>	<u>(2,137)</u>
Net loss	<u>\$ (47,594)</u>	<u>\$ (61,334)</u>	<u>\$ 13,740</u>

Research and Development Expenses

R&D expenses decreased \$4.5 million to \$31.5 million for the three months ended March 31, 2026, compared to the three months ended March 31, 2025. The decrease in R&D expenses was primarily driven by decreases in manufacturing and development and direct material costs of \$5.8 million, stock-based and other compensation and benefits expense of \$2.0 million due to decreased R&D headcount. The decrease was partially offset by increases in clinical trial expenses of \$2.8 million and professional fees of \$0.6 million.

General and Administrative Expenses

G&A expenses decreased \$11.4 million to \$17.1 million for the three months ended March 31, 2026, compared to the three months ended March 31, 2025. The decrease in G&A expenses was primarily driven by decreases in legal expenses of \$5.7 million as a result of legal litigation settlement in 2025, decrease in commercial preparation related expenses of \$4.6 million due to lower headcount and lower spending on commercial launch, and stock-based and other compensation and benefit expenses of \$2.8 million due to decreased G&A headcount. The decrease was partially offset by milestone related expenses of \$2.4 million.

Other Income, Net

Other income decreased \$2.1 million to \$0.9 million for the three months ended March 31, 2026, compared to the three months ended March 31, 2025. The decrease in other income was primarily driven by decreases in interest and other income, net, of \$1.2 million and accretion of discount on investments, net, of \$1.0 million due to lower investment balance and interest rates year over year.

Liquidity and Capital Resources

We have not generated any revenue and have incurred losses since inception. Operations of the Company are subject to certain risks and uncertainties, including, among others, those related to drug candidate development, technology and data security, patents and proprietary rights, our lack of commercial manufacturing marketing or sales experience, dependency on key personnel, compliance with government regulations and the need to obtain additional financing. Drug candidates currently under development will require significant additional R&D efforts, including extensive preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure, and extensive compliance-reporting capabilities.

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

Our consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities in the ordinary course of business. Rocket has incurred net losses and negative cash flows from its operations each year since inception. We incurred net losses of \$47.6 million for the three months ended March 31, 2026, and \$223.1 million for the year ended December 31, 2025. We have experienced negative cash flows from operations and as of March 31, 2026 and December 31, 2025, we had an accumulated deficit of \$1.49 billion and \$1.44 billion, respectively. As of March 31, 2026, we had \$144.4 million of cash, cash equivalents and investments. In April 2026, we entered into an agreement to sell our PRV for \$180 million, providing non-dilutive capital to support advancement of the Company's cardiovascular gene therapy pipeline and extend its operational runway. We believe that, based on our current operating plan, our existing cash, cash equivalents, and investments, together with the anticipated proceeds from the sale of the Company's PRV, will be sufficient to fund our operating expenses and capital expenditure requirements into the second quarter of 2028. Since inception, we have financed our operations primarily through the sale of equity securities and continue to manage our capital resources in a disciplined manner with a focus on operational execution, strategic prioritization, and long-term sustainability.

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

Cash Flows

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

	Three Months Ended March 31,	
	2026	2025
Net cash used in operating activities	\$ (45,394)	\$ (55,790)
Net cash provided by (used in) investing activities	17,448	(58,035)
Net cash used in financing activities	(1)	-
Net decrease in cash, cash equivalents and restricted cash	<u>\$ (27,947)</u>	<u>\$ (113,825)</u>

Operating Activities

During the three months ended March 31, 2026, operating activities used \$45.4 million of cash and cash equivalents, primarily resulting from our net loss of \$47.6 million offset by net non-cash charges of \$9.6 million, including non-cash stock-based compensation expense of \$8.5 million, depreciation and amortization expense of \$2.1 million, partially offset by accretion of discount on investments of \$1.0 million. Changes in our operating assets and liabilities for the three months ended March 31, 2026 included a decrease in accounts payable and accrued expenses of \$5.7 million and an increase in our prepaid expenses of \$1.6 million.

During the three months ended March 31, 2025, operating activities used \$55.8 million of cash and cash equivalents, primarily resulting from our net loss of \$61.3 million offset by net non-cash charges of \$11.2 million, including non-cash stock-based compensation expense of \$10.3 million, depreciation and amortization expense of \$3.0 million, partially offset by accretion of discount on investments of \$2.1 million. Changes in our operating assets and liabilities for the three months ended March 31, 2025 included a decrease in accounts payable and accrued expenses of \$5.3 million and an increase in our prepaid expenses of \$0.5 million.

Investing Activities

During the three months ended March 31, 2026, net cash provided by investing activities was \$17.4 million, primarily resulting from proceeds of \$66.3 million from the maturities of investments, offset by purchases of investments of \$48.7 million, and purchases of property and equipment of \$0.1 million.

During the three months ended March 31, 2025, net cash used by investing activities was \$58.0 million, primarily resulting from proceeds of \$82.3 million from the maturities of investments, offset by purchases of investments of \$139.9 million, and purchases of property and equipment of \$0.4 million.

Financing Activities

During the three months ended March 31, 2026, a minor amount of cash was used to pay down finance lease obligations.

During the three months ended March 31, 2025, no cash was provided or used by financing activities.

Contractual Obligations and Commitments

Information regarding contractual obligations and commitments may be found in Note 13 of our unaudited interim consolidated financial statements in this Quarterly Report on Form 10-Q. We do not have any off-balance sheet arrangements that are material or reasonably likely to become material to our financial condition or results of operations.

Recently Issued Accounting Pronouncements

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

Item 3. Quantitative and Qualitative Disclosures About Market Risk

We are exposed to market risk related to changes in interest rates. As of March 31, 2026 and December 31, 2025, we had cash, cash equivalents and investments of \$144.4 million and \$188.9 million, respectively. The Company's investments are primarily in U.S. Treasury Securities. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in U.S. interest rates and our investments that can decline in value if market interest rates increase. We do not utilize interest rate hedging agreements or other interest rate derivative instruments.

If market interest rates were to increase immediately and uniformly by 100 basis points, or one percentage point, from levels at March 31, 2026, the net effect on the net fair value of our investments would have resulted in a hypothetical decline of \$0.2 million. While we believe our cash, cash equivalents, and marketable securities do not contain excessive risk, we cannot provide absolute assurance that, in the future, our investments will not be subject to adverse changes in market value.

Item 4. Controls and Procedures

Evaluation of Disclosure Controls and Procedures

Our management, with the participation of our principal executive and our principal financial officer, evaluated, as of the end of the period covered by this Quarterly Report on Form 10-Q, the effectiveness of our disclosure controls and procedures. Based on that evaluation of our disclosure controls and procedures as of March 31, 2026, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures as of such date were effective at the reasonable assurance level. The term “disclosure controls and procedures,” as defined in Rules 13a-15(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 during the period covered by this report that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II – OTHER INFORMATION

Item 1. Legal Proceedings

On June 11, 2025 and July 18, 2025, two stockholders filed putative securities class action lawsuits against us and certain of our executive officers in the United States District Court for the District of New Jersey, purportedly on behalf of classes of the Company's investors who purchased or otherwise acquired the Company's common stock between February 27, 2025 and May 26, 2025 (Ho v. Rocket Pharmaceuticals, Inc., and Gaurav Shah, Case No. 3:35-cv-10049) and between September 17, 2024 and May 26, 2025 (Yankov v. Rocket Pharmaceuticals, Inc., Gaurav Shah and Aaron Ondrey, 3:25-cv-13532), respectively. The complaints allege violations of Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 and Rule 10b-5 promulgated thereunder in connection with various public statements made by the Company regarding its Phase 2 clinical trial for RP-A501 for Danon disease. The actions seek unspecified damages, costs and expenses, including attorneys' fees. On September 9, 2025, the Court consolidated the two pending putative securities class action lawsuits, appointed two stockholders as co-lead plaintiffs, and approved their selection of co-lead counsel. Pursuant to a stipulation approved by the Court on September 22, 2025, the co-lead plaintiffs filed a consolidated amended complaint on November 18, 2025. The consolidated amended complaint, captioned In re Rocket Pharmaceuticals, Inc. Securities Litigation, 3:25-cv-10049, is brought on behalf of persons who purchased or otherwise acquired the Company's securities during the period of February 28, 2024 through August 25, 2025 (inclusive). Plaintiffs claim that the lawsuit arises from Defendants' public statements and purported omissions concerning Rocket's Phase 2 clinical trial for RP-A501 for DD. Among other things, Plaintiffs allege that Defendants failed to disclose certain SAEs that impacted patients during the Phase 2 clinical trial and the introduction of a C3 inhibitor to the trial's protocol and that Defendants purportedly lacked a viable trial design that could safely and effectively dose patients while managing the risk of serious adverse events. According to Plaintiffs, Rocket's stock price was inflated as a result of these purported misstatements and omissions. We intend to vigorously defend against the consolidated amended complaint's allegations. On January 30, 2026, the Company filed a motion to dismiss the consolidated amended complaint. The Plaintiffs response to the Company's motion was filed on April 1, 2026 and the Defendants reply thereto is due on May 16, 2026. Given the nature of the cases, including that the proceedings are in their early stages, the Company is unable to predict the ultimate outcome of the cases or estimate the range of potential loss, if any.

On October 22, 2025, a putative derivative action was filed in the District of New Jersey, naming as Defendants certain of the Company's officers and present or former directors of the Company. The Complaint (which names the Company as a nominal defendant) alleges that the Defendants engaged in wrongful conduct during the period from September 17, 2024 through May 26, 2025. The allegations in the complaint largely parallel the allegations made in the previously filed putative securities class action complaints, with some additional allegations regarding a supposed lack of internal controls and purported insider trading. The Complaint seeks declaratory relief, an award of damages to the Company, an order directing the Company and the individual defendants to institute certain requested corporate governance reforms, restitution from the individual defendants, and costs and disbursements related to the lawsuit. The parties agreed to stay all proceedings in the putative derivative action until any motions to dismiss the putative securities class action are resolved, and on December 22, 2025, the Court approved the parties' stipulation to that effect. The Company intends to vigorously defend the litigation. The Company will pay the legal fees related to the putative derivative action against the Company's officers and directors. Given the nature of the litigation, including the fact that the litigation is in its early stages, the Company is unable to predict the ultimate outcome of the litigation or estimate the range of potential loss, if any.

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

Item 1A. Risk Factors

Our material risk factors are disclosed in Item 1A of our 2025 Form 10-K. There have been no material changes from the risk factors previously disclosed in such filing.

Item 2. Unregistered Sales of Equity Securities and Use of Proceeds

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

During the three months ended March 31, 2026, none of our directors or officers adopted or terminated a “Rule 10b5-1 trading arrangement” or “non-Rule 10b5-1 trading arrangement,” as each term is defined in Item 408(a) of Regulation S-K.

Item 6. Exhibits**Exhibit Number Description of Exhibit**

2.1	Agreement and Plan of Merger and Reorganization, dated as of September 12, 2017, by and among Inotek Pharmaceuticals Corporation, Rocket Pharmaceuticals, Ltd., and Rome Merger Sub (incorporated by reference to Exhibit 2.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on September 13, 2017)
2.2	Agreement and Plan of Merger, dated September 19, 2022, by and among Rocket Pharmaceuticals, Renovacor, Inc., Zebrafish Merger Sub, Inc. and Zebrafish Merger Sub II, LLC (incorporated by reference to Exhibit 2.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on September 20, 2022)
3.1	Seventh Amended and Restated Certificate of Incorporation of Rocket Pharmaceuticals, Inc., effective as of February 23, 2015 (incorporated by reference to Exhibit 3.1 to the Company's Annual Report on Form 10-K (001-36829), filed with the SEC on March 31, 2015)
3.2	Certificate of Amendment (Reverse Stock Split) to the Seventh Amended and Restated Certificate of Incorporation of the Registrant, effective as of January 4, 2018 (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on January 5, 2018)
3.3	Certificate of Amendment (Name Change) to the Seventh Amended and Restated Certificate of Incorporation of the Registrant, effective January 4, 2018 (incorporated by reference to Exhibit 3.2 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on January 5, 2018)
3.4	Certificate of Amendment (Declassify Board of Directors) to the Seventh Amended and Restated Certificate of Incorporation of the Registrant, effective as of June 25, 2018 (incorporated by reference to Exhibit 3.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on June 25, 2019)
3.5	Certificate of Amendment (Authorized Shares Increase) to the Seventh Amended and Restated Certificate of Incorporation of the Registrant (incorporated by reference to Exhibit 3.1 of the Registrant's Current Report on Form 8-K filed with the Commission on June 20, 2024)
3.6	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 With Embedded Linkbase Documents.
104	Cover Page Interactive Data File (the cover page XBRL tags are embedded within the Inline XBRL document)

* Filed herewith.

Indicates management contract or compensatory plan.

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

SIGNATURES

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

ROCKET PHARMACEUTICALS, INC.

May 7, 2026

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

May 7, 2026

By: /s/ Martin Wilson
Martin Wilson
General Counsel and Chief Corporate Officer
(Principal Financial Officer)

CERTIFICATIONS

I, Gaurav Shah, MD, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended March 31, 2026 of Rocket Pharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer(s) and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 7, 2026

By: /s/ Gaurav Shah, MD

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

CERTIFICATIONS

I, Martin Wilson, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended March 31, 2026 of Rocket Pharmaceuticals, Inc.;
2. Based on my knowledge, this report does not contain any untrue statement of a material fact or omit to state a material fact necessary to make the statements made, in light of the circumstances under which such statements were made, not misleading with respect to the period covered by this report;
3. Based on my knowledge, the financial statements, and other financial information included in this report, fairly present in all material respects the financial condition, results of operations and cash flows of the registrant as of, and for, the periods presented in this report;
4. The registrant's other certifying officer and I are responsible for establishing and maintaining disclosure controls and procedures (as defined in Exchange Act Rules 13a-15(e) and 15d-15(e)) and internal control over financial reporting (as defined in Exchange Act Rules 13a-15(f) and 15d-15(f)) for the registrant and have:
 - a. Designed such disclosure controls and procedures, or caused such disclosure controls and procedures to be designed under our supervision, to ensure that material information relating to the registrant, including its consolidated subsidiaries, is made known to us by others within those entities, particularly during the period in which this report is being prepared;
 - b. Designed such internal control over financial reporting, or caused such internal control over financial reporting to be designed under our supervision, to provide reasonable assurance regarding the reliability of financial reporting and the preparation of financial statements for external purposes in accordance with generally accepted accounting principles;
 - c. Evaluated the effectiveness of the registrant's disclosure controls and procedures and presented in this report our conclusions about the effectiveness of the disclosure controls and procedures, as of the end of the period covered by this report based on such evaluation; and
 - d. Disclosed in this report any change in the registrant's internal control over financial reporting that occurred during the registrant's most recent fiscal quarter (the registrant's fourth fiscal quarter in the case of an annual report) that has materially affected, or is reasonably likely to materially affect, the registrant's internal control over financial reporting; and
5. The registrant's other certifying officer(s) and I have disclosed, based on our most recent evaluation of internal control over financial reporting, to the registrant's auditors and the audit committee of the registrant's board of directors (or persons performing the equivalent functions):
 - a. All significant deficiencies and material weaknesses in the design or operation of internal control over financial reporting which are reasonably likely to adversely affect the registrant's ability to record, process, summarize and report financial information; and
 - b. Any fraud, whether or not material, that involves management or other employees who have a significant role in the registrant's internal control over financial reporting.

Date: May 7, 2026

By: /s/ Martin Wilson

Martin Wilson
General Counsel and Chief Corporate Officer
(Principal Financial Officer)

CERTIFICATION PURSUANT TO

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

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the quarterly report on Form 10-Q of Rocket Pharmaceuticals, Inc. (the “Company”) for the period ended March 31, 2026, as filed with the United States Securities and Exchange Commission on the date hereof (the “Report”), each of the undersigned officers hereby certifies, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, 18 U.S.C. Section 1350, that to his knowledge:

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

Date: May 7, 2026

By: /s/ Gaurav Shah, MD

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

Date: May 7, 2026

By: /s/ Martin Wilson

Martin Wilson
General Counsel and Chief Corporate Officer
(Principal Financial Officer)
