UNITED STATES
SECURITIES AND EXCHANGE COMMISSION

Washington, D.C. 20549

Form 10-Q

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

For the quarterly period ended September 30, 2025 or

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

For the transition period from_____to

Commission File Number: 001-36829

Rocket Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**04-3475813**
(State or other jurisdiction of incorporation or organization)	(I.R.S. Employer Identification No.)
9 Cedarbrook Drive, Cranbury, NJ	**08512**
(Address of principal executive office)	(Zip Code)

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

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

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

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

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

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, 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 ☒	Accelerated filer ☐
Non-accelerated filer ☐	Smaller reporting company ☐
	Emerging growth company ☐

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

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

As of October 31, 2025, there were 108,222,228 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	IND	Investigational New Drug application
ACM	Arrhythmogenic cardiomyopathy	IPR&D	In process research and development
ASC	Accounting Standard Codification	KCCQ	Kansas City Cardiovascular Questionnaire
ASGCT	American Society of Gene & Cell Therapy	LAD-I	Leukocyte Adhesion Deficiency-I
ATMP	Advanced Therapy Medical Product	LAMP2	Lysosome-associated membrane protein 2
BAG3	BCL2-associated athanogene 3	LV	Lentiviral vector
BAG3-DCM	BCL2-associated athanogene 3 mutations associated with dilated cardiomyopathy	MAA	Marketing Authorization Application
BLA	Biologics License Application	NYHA	New York Heart Association
BNP	Brain natriuretic peptide	Offering	Equity offering of shares of common stock, which closed December 12, 2024
cGMP	Current Good Manufacturing Practice	PDUFA	Prescription Drug User Fee Act
CIRM	California Institute for Regenerative Medicine	PKD	Pyruvate Kinase Deficiency
CMC	Chemistry Manufacturing Controls	PKP2	Plakophilin-2
CODM	Chief Operating Decision Maker	PKP2-ACM	Plakophilin-2 Arrhythmogenic Cardiomyopathy
CRL	Complete Response Letter	PSU	Performance Stock Unit
Cowen	Cowen and Company, LLC	PRIME	Priority Medicines
CTIS	Clinical Trials Information Systems	Private Placement	Issuance of pre-funded warrants on December 12, 2024
DCM	Dilated cardiomyopathy	R&D	Research and development
DD	Danon Disease	RBC	Red blood cell
DNA	Deoxyribonucleic acid	Renovacor	Renovacor, Inc. acquired on December 1, 2022
ECG	Electrocardiogram	RMAT	Regenerative Medicine Advanced Therapy
EMA	European Medicines Agency	RSU	Restricted stock unit
ESB Lease Agreement	Office Lease agreement for office space in the Empire State Building in New York City	RTW	RTW Investments, L.P
EU	European Union	SAE	Serious Adverse Event
FA	Fanconi Anemia	SCD	Sudden cardiac death
FDA	U.S. Food and Drug Administration	SEC	Securities and Exchange Commission
GMP	Good Manufacturing Practice	Stanford	Center for Definitive and Curative Medicine at Stanford University School of Medicine
HF	Heart Failure	UCLA	University of California, Los Angeles
HNJ	Hospital Infantil de Niño Jesús	U.S.	United States
HSCT	Hematopoietic stem cell transplant	U.S. GAAP	U.S. Generally Accepted Accounting Principles
ICD	Implantable cardiac defibrillator		

Cautionary Statement Regarding Forward-Looking Statements

This Quarterly Report on Form 10-Q for the quarter ended September 30, 2025 contains forward-looking statements that involve risks and uncertainties, as well as assumptions that, if they do not materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q are forward-looking statements. In some cases, you can identify forward-looking statements by words such as "aim," "anticipate," "believe," "can," "contemplate," "continue," "could," "design," "develop," "estimate," "expect," "expand," "future," "hope," "intend," "likely," "may," "plan," "potential," "predict," "project," "pursue," "seek," "should," "strategy," "target," "will," "would," or the negative of these words or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- our ability to meet our anticipated milestones for our various drug candidates with respect to the initiation and timing of clinical studies;
- federal, state, and non-U.S. regulatory requirements, including regulation of our current or any other future product candidates by the FDA;
- the timing of and our ability to submit regulatory filings with the FDA and to obtain and maintain FDA or other regulatory authority approval of, or other action with respect to, our product candidates;
- our competitors' activities, including decisions as to the timing of competing product launches, pricing, and discounting;
- whether safety and efficacy results of our clinical trials and other required tests for approval of our product candidates provide data to warrant progression of clinical trials, potential regulatory approval, or further development of any of our product candidates;
- our ability to develop, acquire and advance product candidates into, enroll a sufficient number of patients into, and successfully complete, clinical studies, and our ability to apply for and obtain regulatory approval for such product candidates, within currently anticipated timeframes, or at all;
- our ability to establish key collaborations and vendor relationships for our product candidates and any other future product candidates;
- our ability to develop our sales and marketing capabilities or enter into agreements with third parties to sell and market any of our product candidates;
- our ability to obtain additional funding to conduct our planned research and development efforts;
- our ability to acquire additional businesses, form strategic alliances or create joint ventures and our ability to realize the benefit of such acquisitions, alliances, or joint ventures;
- our ability to successfully develop and commercialize any technology that we may in-license or products we may acquire;
- the development of our direct manufacturing capabilities for our AAV programs;
- our ability to 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 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; 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, 2024, 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)

	September 30, 2025	December 31, 2024
	(unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	$ 75,948	$ 163,635
Investments	146,810	208,701
Prepaid expenses and other current assets	4,922	5,847
Total current assets	227,680	378,183
Property and equipment, net	29,768	36,786
Goodwill	39,154	39,154
Intangible assets	25,150	25,150
Restricted cash	1,365	1,362
Deposits	515	529
Operating lease right-of-use assets, net	3,653	4,173
Finance lease right-of-use asset, net	40,748	42,363
Total assets	$ 368,033	$ 527,700
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 28,258	$ 37,827
Operating lease liabilities, current	1,015	1,001
Finance lease liability, current	1,897	1,856
Total current liabilities	31,170	40,684
Operating lease liabilities, non-current	2,758	3,258
Finance lease liability, non-current	19,375	19,383
Other liabilities	1,061	1,141
Total liabilities	54,364	64,466
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; 108,208,643 and 106,453,818 shares issued and outstanding as of September 30, 2025, and December 31, 2024, respectively	1,082	1,065
Additional paid-in capital	1,711,297	1,680,219
Accumulated other comprehensive (loss) income	(9)	66
Accumulated deficit	(1,398,701)	(1,218,116)
Total stockholders’ equity	313,669	463,234
Total liabilities and stockholders’ equity	$ 368,033	$ 527,700

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	34,068	42,315	112,668	133,887
General and administrative	18,351	27,109	71,817	76,624
Restructuring	(172)	-	3,299	-
Total operating expenses	52,247	69,424	187,784	210,511
Loss from operations	(52,247)	(69,424)	(187,784)	(210,511)
Interest expense	(473)	(471)	(1,418)	(1,413)
Interest and other income, net	709	1,327	2,528	6,650
Accretion of discount on investments, net	1,679	1,849	6,089	6,855
Net loss	$ (50,332)	$ (66,719)	$ (180,585)	$ (198,419)
Net loss per share - basic and diluted	$ (0.45)	$ (0.71)	$ (1.63)	$ (2.11)
Weighted-average common shares outstanding - basic and diluted	111,571,136	94,158,491	110,900,161	93,893,729

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

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Net loss	$ (50,332)	$ (66,719)	$ (180,585)	$ (198,419)
Other comprehensive loss:				
Net unrealized gain (loss) on investments	98	431	(75)	(106)
Total comprehensive loss	$ (50,234)	$ (66,288)	$ (180,660)	$ (198,525)

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

Rocket Pharmaceuticals, Inc.
Consolidated Statements of Stockholders' Equity
($ in thousands except share amounts)
(unaudited)

	Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Income/(Loss)	Deficit	Equity
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	106,753,886	1,068	1,690,547	(27)	(1,279,450)	412,138
Issuance of common stock pursuant to exercise of stock options	952,313	9	(9)	-	-	-
Issuance of common stock pursuant to vesting of restricted stock units	178,221	2	(2)	-	-	-
Unrealized comprehensive loss on investments	-	-	-	(80)	-	(80)
Stock-based compensation	-	-	10,857	-	-	10,857
Return of related party short-swing profits	-	-	215	-	-	215
Net loss	-	-	-	-	(68,919)	(68,919)
Balance at June 30, 2025	107,884,420	1,079	1,701,608	(107)	(1,348,369)	354,211
Vesting of restricted stock units	347,031	3	(3)	-	-	-
Repurchase of restricted stock units to satisfy tax withholding	(22,808)	-	(67)	-	-	(67)
Unrealized comprehensive gain on investments	-	-	-	98	-	98
Stock-based compensation	-	-	9,759	-	-	9,759
Net loss	-	-	-	-	(50,332)	(50,332)
Balance at September 30, 2025	108,208,643	$ 1,082	$ 1,711,297	$ (9)	$ (1,398,701)	$ 313,669

	Common Stock		Additional Paid-In	Accumulated Other Comprehensive	Accumulated	Total Stockholders'
	Shares	Amount	Capital	Income/(Loss)	Deficit	Equity
Balance at December 31, 2023	90,282,267	$ 903	$ 1,450,722	$ 319	$ (959,370)	$ 492,574
Issuance of common stock pursuant to exercise of stock options	73,745	-	1,184	-	-	1,184
Issuance of common stock pursuant to vesting of restricted stock units	290,578	3	(3)	-	-	-
Unrealized comprehensive loss on investments	-	-	-	(454)	-	(454)
Stock-based compensation	-	-	10,252	-	-	10,252
Net loss	-	-	-	-	(62,054)	(62,054)
Balance at March 31, 2024	90,646,590	906	1,462,155	(135)	(1,021,424)	441,502
Issuance of common stock pursuant to exercise of stock options	159,355	2	1,528	-	-	1,530
Issuance of common stock pursuant to vesting of restricted stock units	150,668	2	(2)	-	-	-
Unrealized comprehensive loss on investments	-	-	-	(83)	-	(83)
Stock-based compensation	-	-	11,332	-	-	11,332
Net loss	-	-	-	-	(69,646)	(69,646)
Balance at June 30, 2024	90,956,613	910	1,475,013	(218)	(1,091,070)	384,635
Issuance of common stock pursuant to exercise of stock options	13,108		176			176
Issuance of common stock pursuant to vesting of restricted stock units	146,971	1	(1)			-
Unrealized comprehensive gain on investments				431		431
Stock-based compensation			11,248			11,248
Net loss					(66,719)	(66,719)
Balance at September 30, 2024	91,116,692	$ 911	$ 1,486,436	$ 213	$ (1,157,789)	$ 329,771

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

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

	Nine Months Ended September 30, 2025	2024
Operating activities:		
Net loss	$ (180,585)	$ (198,419)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation and amortization of property and equipment	7,193	5,399
Amortization of finance lease right of use asset	1,615	1,615
Stock-based compensation	30,947	32,832
Accretion of discount on investments, net	(5,886)	(6,845)
Change in fair value of warrant liabilities	-	(1,875)
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	939	(1,015)
Accounts payable and accrued expenses	(9,384)	5,293
Operating lease liabilities and right of use assets, net	34	72
Finance lease liability	33	80
Other liabilities	(80)	81
Net cash used in operating activities	(155,174)	(162,782)
Investing activities:		
Purchases of investments	(222,058)	(121,841)
Proceeds from maturities of investments	289,760	296,971
Purchases of property and equipment	(360)	(5,553)
Net cash provided by investing activities	67,342	169,577
Financing activities:		
Issuance of common stock pursuant to exercise of stock options	-	2,890
Return of related party short-swing profits	215	-
Repurchase of restricted stock units to satisfy tax withholding	(67)	-
Net cash provided by financing activities	148	2,890
Net change in cash, cash equivalents and restricted cash	(87,684)	9,685
Cash, cash equivalents and restricted cash at beginning of period	164,997	57,276
Cash, cash equivalents and restricted cash at end of period	$ 77,313	$ 66,961
Supplemental disclosure of non-cash financing and investing activities:		
Accrued purchases of property and equipment, ending balance	$ -	$ 265
Operating lease liabilities	$ -	$ 1,134
Operating lease right of use assets	$ -	$ 1,134
Net unrealized loss on investments	$ (75)	$ (106)

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") is a fully integrated, late-stage biotechnology company advancing a sustainable pipeline of investigational genetic therapies 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 potentially transformative options that enable people living with devastating rare diseases to experience long and full lives.

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 consists of late-stage programs for Leukocyte Adhesion Deficiency-I (LAD-I), a severe pediatric genetic disorder that causes recurrent and life-threatening infections which are frequently fatal; Fanconi Anemia (FA), a difficult-to-treat genetic disease that leads to bone marrow failure and potentially cancer; and Pyruvate Kinase Deficiency (PKD), a monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia.

2. Risks and Liquidity

The Company has not generated any revenue and has incurred losses since inception. Operations of the Company are subject to certain risks and uncertainties, including, among others, uncertainty of drug candidate development, technological uncertainty, uncertainty regarding patents and proprietary rights, having no commercial manufacturing experience, marketing or sales capability or experience, dependency on key personnel, compliance with government regulations and the need to obtain additional financing. Drug candidates currently under development will require significant additional research and development efforts, including extensive preclinical and clinical testing and regulatory approval, prior to commercialization. These efforts require significant amounts of additional capital, adequate personnel infrastructure, and extensive compliance-reporting capabilities.

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

The Company's consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities in the ordinary course of business. The Company has experienced negative cash flows from operations and had an accumulated deficit of $1.40 billion as of September 30, 2025. As of September 30, 2025, the Company had $222.8 million of cash, cash equivalents and investments. The Company expects that in accordance with the current operating plan, which reflects a strategic corporate reorganization and reprioritization announced in July 2025, such resources will be sufficient to fund the Company's operating expenses and capital expenditure requirements into the second quarter of 2027.

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 research and development 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.

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. On August 20, 2025, the Company announced that the FDA lifted the clinical hold on the trial 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%.

While the reorganization is intended to streamline operations, improve capital efficiency, and extend the Company's cash runway, there can be no assurance that the anticipated benefits such as cost savings, improved focus, or operational efficiency will be realized to the extent or within the timeframe currently expected. The reduction in force and related organizational changes may also result in near-term disruption to the Company's operations, loss of institutional knowledge, or diminished employee morale, which could adversely impact productivity and the Company's ability to execute on key initiatives. In addition, the Company may incur additional or unforeseen costs associated with the reorganization, including severance, outplacement, legal, and consulting expenses.

The Company may also face challenges retaining key personnel and maintaining continuity across teams, which could impair our 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, 2024 included in the Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 27, 2025. The unaudited interim consolidated financial statements have been prepared on the same basis as the audited annual financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for the fair statement of the Company's consolidated financial position as of September 30, 2025 and the results of its operations and its cash flows for the nine months ended September 30, 2025. The financial data and other information disclosed in these consolidated notes related to the three and nine months ended September 30, 2025 and 2024 are unaudited. The results for the three and nine months ended September 30, 2025 are not necessarily indicative of results to be expected for the year ending December 31, 2025 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 and nine months ended September 30, 2025 are consistent with those disclosed in Note 3 to the consolidated financial statements in the 2024 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:

	September 30, 2025	December 31, 2024
Cash and cash equivalents	$ 75,948	$ 163,635
Restricted cash	1,365	1,362
Total cash, cash equivalents and restricted cash	$ 77,313	$ 164,997

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 and nine months ended September 30, 2025 and 2024, there were no unrealized losses that were credit related. For the three and nine months ended September 30, 2025, there were net unrealized gain on investments of $0.1 million and net unrealized loss on investment of $0.1 million, respectively. For the three and nine months ended September 30, 2024, there were net unrealized gain on investments of $0.4 million and net unrealized loss on investment of $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 the 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.

In May 2025, two patients participating in the Phase 2 pivotal study of RP-A501 each experienced an unexpected SAE after which the FDA placed a clinical hold on the trial to allow for further evaluation. In response to the unexpected SAEs, the Company performed a quantitative assessment of its goodwill and determined that it is more likely than not that the fair value of the Company exceeds the carrying value. As a result, the Company has determined that there was no goodwill impairment as of the date of the event or for the three and nine months ended September 30, 2025. The clinical hold on the trial was subsequently lifted by the FDA after the Company satisfactorily addressed issues outlined in the clinical hold.

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.

Income Taxes

On July 4, 2025, the One Big Beautiful Bill Act was enacted into law with changes to U.S. tax law that will be applicable to the Company beginning in 2025. These changes include provisions allowing accelerated tax deductions for qualified property and research expenditures. We are in the process of evaluating the impact of the One Big Beautiful Bill Act on our Consolidated Financial Statements.

Recent Accounting Pronouncements

Accounting Pronouncements Not Adopted as of September 30, 2025

ASU 2023-09: Income Taxes Topic 740 - Improvements to Income Tax Disclosures. This update standardizes categories for the effective tax rate reconciliation, requires disaggregation of income taxes and additional income tax-related disclosures. This update is required to be effective for the Company for fiscal periods beginning after December 15, 2024. The Company is evaluating the effect that ASU 2023-09 will have on its financial statements and disclosures.

ASU 2024-03: Expense Disaggregation Disclosures. This update requires disaggregated disclosure of income statement expenses. This update will be effective for the Company 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.

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:

	Fair Value Measurements as of September 30, 2025, Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market mutual funds	$ 50,286	$ -	$ -	$ 50,286
U.S. Treasury securities	-	4,999	-	4,999
	50,286	4,999	-	55,285
Investments:				
U.S. Treasury securities	-	146,810	-	146,810
	-	146,810	-	146,810
Total assets	$ 50,286	$ 151,809	$ -	$ 202,095

	Fair Value Measurements as of December 31, 2024, Using:			
	Level 1	Level 2	Level 3	Total
Assets:				
Cash equivalents:				
Money market mutual funds	$ 139,948	$ -	$ -	$ 139,948
U.S. Treasury securities	-	7,453	-	7,453
	139,948	7,453	-	147,401
Investments:				
U.S. Treasury securities	-	208,701	-	208,701
	-	208,701	-	208,701
Total assets	$ 139,948	$ 216,154	$ -	$ 356,102

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 December 31, 2024.

5. Property and Equipment, Net

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

	September 30, 2025	December 31, 2024
Laboratory equipment	$ 32,058	$ 32,205
Machinery and equipment	12,133	12,857
Computer equipment	1,015	1,015
Furniture and fixtures	2,777	2,777
Leasehold improvements	7,327	7,282
Internal use software	1,903	1,903
	57,213	58,039
Less: accumulated depreciation and amortization	(27,445)	(21,253)
Total property and equipment, net	$ 29,768	$ 36,786

During the three and nine months ended September 30, 2025, the Company recognized $2.7 million and $7.2 million of depreciation and amortization expense, respectively. During the three and nine months ended September 30, 2024, the Company recognized $1.9 million and $5.4 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. Intangible assets as of September 30, 2025, and December 31, 2024, are summarized as follows:

	September 30, 2025	December 31, 2024
Gross carrying value	$ 25,150	$ 25,150
Accumulated amortization	-	-
Total intangible assets	$ 25,150	$ 25,150

The carrying value of Goodwill as of September 30, 2025, and December 31, 2024, was $39.2 million.

7. Accounts Payable and Accrued Expenses

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

	September 30, 2025	December 31, 2024
Research and development	$ 9,577	$ 16,768
Employee compensation	11,429	11,944
Property and equipment	-	185
Professional fees	1,819	7,305
Restructuring	2,760	-
Other	2,673	1,625
Total accounts payable and accrued expenses	$ 28,258	$ 37,827

8. Stockholders' Equity

Public Offerings and Private Placements

On December 12, 2024, the Company completed the Offering of 15,180,000 shares of its common stock at a public offering price of $12.50 per share and a Private Placement of pre-funded warrants to purchase 400,000 shares of common stock at a price of $12.49 per pre-funded warrant. The gross proceeds from the Offering and Private Placement were approximately $194.7 million. The net proceeds of the Offering and Private Placement were approximately $182.5 million after reduction for offering costs, underwriting discounts and commissions, legal and other expenses.

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:

	Nine Months Ended September 30,	
	2025	2024
Risk-free interest rate	4.19%	4.85%
Expected term (in years)	5.51	5.82
Expected volatility	75.33%	73.70%
Expected dividend yield	0.00%	0.00%
Exercise price	$ 8.62	$ 27.24
Fair value of common stock	$ 8.62	$ 27.24

The following table summarizes stock option activity for the nine months ended September 30, 2025:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Contractual Term (Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2024	16,044,686	$ 16.19	4.67	$ 63,295
Granted	2,243,352	8.62	8.26	
Exercised	(952,313)	0.00		-
Cancelled or forfeited	(664,722)	20.17		
Outstanding as of September 30, 2025	16,671,003	$ 15.94	4.18	$ 9,935
Options vested and exercisable as of September 30, 2025	13,776,919	$ 16.51	3.25	$ 9,823
Options unvested as of September 30, 2025	2,894,084	$ 13.20	8.61	$ 112

The weighted average grant-date fair value per share of stock options granted during the nine months ended September 30, 2025, and 2024 was $5.15 and $18.34, respectively.

The total fair value of options vested during the nine months ended September 30, 2025, and 2024 was $24.0 million and $23.6 million, respectively.

Restricted Stock Units

The following table summarizes the Company's RSU activity for the nine months ended September 30, 2025:

	Number of Shares	Weighted Average Grant Date Fair Value
Unvested as of December 31, 2024	1,414,210	$ 23.02
Granted	5,622,352	6.60
Vested[(1)]	(825,320)	21.16
Forfeited	(709,618)	12.32
Unvested as of September 30, 2025	5,501,624	$ 7.90

[(1)] Common stock issued is net of 22,808 RSUs related to taxes.

The total fair value of RSUs vested during the nine months ended September 30, 2025, and 2024 was $17.5 million and $11.0 million, respectively.

Performance Stock Units

The following table summarizes the Company's PSU activity for the nine months ended September 30, 2025:

	Number of Shares	Weighted Average Grant Date Fair Value
Unvested as of December 31, 2024	69,661	$ 28.71
Granted	-	-
Vested	-	-
Forfeited	(52,246)	28.71
Unvested as of September 30, 2025	17,415	$ 28.71

PSU vesting and expense recognition is based on achievement of specific performance goals within certain time periods. PSU awards that are not achieved within specific time periods are forfeited. No performance goals were probable of achievement as of September 30, 2025, and the time periods for two of three performance goals at the beginning of the year expired during the nine months ended September 30, 2025.

Stock-Based Compensation Expense

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Stock options	$ 4,419	$ 7,005	$ 15,780	$ 21,105
Restricted stock units	5,340	4,243	15,167	11,727
Total stock-based compensation expense	$ 9,759	$ 11,248	$ 30,947	$ 32,832

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Research and development	$ 4,752	$ 4,789	$ 13,961	$ 14,311
General and administrative	5,007	6,459	16,986	18,521
Total stock-based compensation expense	$ 9,759	$ 11,248	$ 30,947	$ 32,832

As of September 30, 2025, the Company had an aggregate of $58.7 million of unrecognized stock-based compensation expense related to stock options, RSU and PSU grants. The stock options and RSU grants had an aggregate of $58.2 million of unrecognized stock-based compensation expense, which is expected to be recognized over a weighted average period of 1.87 years.

10. Warrants

A summary of the warrants outstanding as of September 30, 2025, 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	5,498,028		

Warrants Issued in Public Offerings

The Company issued warrants to a related party during the years ended December 31, 2024, and 2023. For the years ended December 31, 2024, and 2023, the Company sold pre-funded warrants to purchase 400,000 and 3,126,955 shares of common stock, respectively with an exercise price of $0.01 per share (see Note 8 “Stockholders Equity”). The pre-funded warrants were acquired by funds affiliated with RTW (see Note 16 “Related Party Transactions”).

Assumed Renovacor Warrants

In conjunction with the acquisition of Renovacor, the Company assumed pre-acquisition public warrants that were converted into warrants to purchase 760,086 shares of Rocket Pharmaceuticals, Inc. common stock at an exercise price of $65.23 per share.

In conjunction with the acquisition of Renovacor, the Company assumed pre-acquisition private warrants that were converted into warrants to purchase 617,050 shares of Rocket Pharmaceuticals, Inc. common stock at an exercise price of $65.23 per share. The Company determined that the private warrants did not meet all of the criteria for equity classification. Accordingly, the Company classified these as a derivative liability in other liabilities in the Consolidated Balance Sheets. The Company measured the fair value of these warrants at the end of each reporting period and recognized changes in the fair value from the prior period in the Company’s operating results for the current period. These warrants expired on April 23, 2025.

11. Net Loss Per Share

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Numerator:				
Net loss attributable to common stockholders	$ (50,332)	$ (66,719)	$ (180,585)	$ (198,419)
Denominator:				
Weighted-average common shares outstanding - basic and diluted	111,571,136	94,158,491	110,900,161	93,893,729
Net loss per share attributable to common stockholders - basic and diluted	$ (0.45)	$ (0.71)	$ (1.63)	$ (2.11)

For the three and nine months ended September 30, 2025, the Company included the 3,126,955 potential shares from the pre-funded warrants acquired by RTW in 2023 and the 400,000 potential shares from the 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.

For the three and nine months ended September 30, 2024, the Company included the 3,126,955 potential shares from the pre-funded warrants acquired by RTW in 2023 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 and Nine Months Ended September 30,	
	2025	2024
Warrants exercisable for common shares	1,971,073	2,588,123
Restricted stock units	5,501,624	1,565,444
Performance stock units	17,415	121,907
Options to purchase common shares	16,671,003	16,148,020
Total potential shares excluded from diluted net loss per share	24,161,115	20,423,494

12. Leases

Finance Lease

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

Estimated rent payments for the NJ Lease Agreement are $1.2 million per annum, payable in monthly installments, 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 September 30, 2025, and December 31, 2024.

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 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 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.

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 September 30, 2025, and December 31, 2024.

On December 1, 2022, in connection with the acquisition of Renovacor, the Company added operating leases for space at facilities in Hopewell, New Jersey and Cambridge, Massachusetts with remaining lease terms of approximately 10.3 and 1.3 years, respectively. The Company recognized total right-of-use assets of $3.8 million with corresponding total lease liabilities of $3.6 million at lease commencement dates. The Company 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 a sublease agreement was less than $0.1 million for the three and nine months ended September 30, 2025. Rental income received under a sublease agreement totaled $0.1 million and $0.3 million for the three and nine months ended September 30, 2024, respectively.

Rent expense excluding rental income was $0.3 million and $0.9 million for the three and nine months ended September 30, 2025. Rent expenses excluding rental income were $0.3 million and $1.2 million for the three and nine months ended September 30, 2024, respectively.

The total restricted cash balance for the Company's operating and finance leases as of September 30, 2025, and December 31, 2024, was $0.8 million.

The following table summarizes lease cost for the nine months ended September 30, 2025, and 2024:

	Nine Months Ended September 30,	
Lease cost	**2025**	**2024**
Operating lease cost	$ 785	$ 956
Finance lease cost:		
Amortization of right of use assets	1,615	1,615
Interest on lease liabilities	1,418	1,413
Total lease cost	$ 3,818	$ 3,984

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,	September 30, 2025
2025 (three months)	$ 251
2026	1,005
2027	759
2028	522
2029	539
Thereafter	1,881
Total lease payments	$ 4,957
Less: interest	(1,184)
Total operating lease liabilities	$ 3,773

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

Fiscal Year Ending December 31,	September 30, 2025
2025 (three months)	$ 471
2026	1,911
2027	1,969
2028	2,028
2029	2,089
Thereafter	38,915
Total lease payments	$ 47,383
Less: interest	(26,111)
Total finance lease liability	$ 21,272

The following table summarizes the operating and financing lease liabilities and right-of-use assets as of September 30, 2025, and December 31, 2024:

Leases	September 30, 2025	December 31, 2024
Operating right-of-use assets	$ 3,653	$ 4,173
Operating current lease liabilities	$ 1,015	$ 1,001
Operating noncurrent lease liabilities	2,758	3,258
Total operating lease liabilities	$ 3,773	$ 4,259
Finance right-of-use assets	$ 40,748	$ 42,363
Finance current lease liability	$ 1,897	$ 1,856
Finance noncurrent lease liability	19,375	19,383
Total finance lease liability	$ 21,272	$ 21,239

Other Information	Nine Months Ended September 30, 2025	2024
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases	$ 750	$ 829
Cash flows from finance lease	$ 1,385	$ 1,333
Weighted-average remaining lease term - operating leases	6.2 years	6.7 years
Weighted-average remaining lease term - finance lease	18.9 years	19.9 years
Weighted-average discount rate - operating leases	8.83%	8.82%
Weighted-average discount rate - finance lease	8.96%	8.96%

13. Commitments and Contingencies

Litigation

In June 2025, the Company entered into a settlement agreement with Lexeo Therapeutics, Inc. ("Lexeo") to resolve all claims in ongoing litigation between the parties in the United States District Court for the Southern District of New York. The litigation involved allegations by the Company of trade secret misappropriation and tortious interference, and counterclaims by Lexeo including correction of inventorship, breach of contract, and trade secret misappropriation. As part of a comprehensive resolution, Rocket also entered into separate settlement agreements on the same day with certain individuals formerly affiliated with the Company, who were also named in the litigation.

Under the terms of the settlement agreement between the Company and Lexeo, the litigation has been resolved amicably and fully, and without admission of liability by any party. For the three and nine months ended September 30, 2025, the gain or loss in connection with this settlement agreement was not material.

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 and between September 17, 2024 and May 26, 2025, 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 will have until November 18, 2025 to file a consolidated amended complaint. The Company intends to vigorously defend against such allegations. 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 our 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 Company intends to vigorously defend the litigation. 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. The maximum potential number of future payments the Company could be required to pay is unlimited. The Company has insurance that reduces its monetary exposure and would enable it to recover a portion of any future amounts paid. As a result, the Company believes that the estimated fair value of these indemnification commitments is minimal.

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

Contingent Consulting Fee

The Company has an agreement with a consultant that requires payment of a success fee of $0.3 million upon FDA approval of KRESLADI™ before January 31, 2026.

14. Agreements Related to Intellectual Property

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

15. CIRM Grants

LAD-I CIRM Grant

On April 30, 2019, CIRM awarded the Company up to $7.5 million under a CLIN2 grant to support the clinical development of its LV-based gene therapy, RP-L201. Proceeds from the grant helped fund clinical trial costs as well as manufactured drug product for Phase 1/2 patients enrolled at the U.S. clinical site, University of California, Los Angeles Mattel Children's Hospital, led by principal investigator Donald Kohn, M.D., UCLA Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics (Hematology/Oncology), Molecular and Medical Pharmacology and member of the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at UCLA. As of September 30, 2025, the Company has received $5.9 million in total RP-L201 grants from CIRM. The Company received a final milestone grant of less than $0.1 million on January 2, 2024, and no additional payments are available under the grant awards program.

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 nine months ended September 30, 2025, the Company received grants of $2.7 million, which were recorded as a reduction of R&D expenses. Through September 30, 2025, the Company has received RP-A501 grants of $5.0 million from CIRM.

16. Related Party Transactions

In June 2023, the Company entered into a consulting agreement with the spouse of one of the Company's former executive officers for information technology advisory services. The Company incurred expenses of approximately $0 and $2,000 for the nine months ended September 30, 2025 and 2024, respectively, for services provided under this agreement.

In December 2024, in connection with a public offering, the Company sold 400,000 pre-funded warrants to purchase shares of the Company's common stock to funds affiliated with RTW, the Company's largest shareholder (see Note 8 "Stockholders' Equity").

In February 2025, the Company entered into a consulting agreement with one of the Company's board members, effective March 3, 2025, for services related to the Company's R&D activities. As compensation for services rendered during 2025, the consultant will receive $125,000 to be paid in equal monthly installments and $125,000 of RSU's valued as of the closing price on March 3, 2025, which will cliff vest on December 31, 2025. The agreement will terminate on December 31, 2025, unless terminated earlier by the Company for cause or voluntarily by the consultant. The board member was paid approximately $87,500 for the nine months ended September 30, 2025, for services provided under the consulting agreement.

In June 2025, the Company received a total of approximately $215,000, representing return of short-swing profits from the sale of common stock by beneficial owners under Section 16(b) of the Securities and Exchange Act of 1934, as amended. The Company recognized these proceeds as a capital contribution and reflected a corresponding increase to additional paid-in capital.

In August 2025, the Company paid a total of approximately $67,000, representing payment of taxes upon vesting of RSUs by beneficial owners under Section 16(b) of the Securities and Exchange Act of 1934, as amended. The Company recognized these payments as capital payments and reflected a corresponding decrease to additional paid-in capital.

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 the minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre-tax basis. Company contributions to the Plan may be made at the discretion of the Company's Board of Directors. The Company has elected the safe harbor match of 4% of employee contributions to the Plan, subject to certain limitations. The Company's matching contribution for the three and nine months ended September 30, 2025, was $0.4 million and $1.5 million, respectively. The Company's matching contribution for the three and nine months ended September 30, 2024, was $0.5 million and $1.3 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 September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	34,068	42,315	112,668	133,887
Non-commercial general and administrative	13,350	15,249	51,007	47,802
Commercial general and administrative	5,001	11,860	20,810	28,822
Restructuring	(172)	-	3,299	-
Total operating expenses	52,247	69,424	187,784	210,511
Loss from operations	(52,247)	(69,424)	(187,784)	(210,511)
Interest expense	(473)	(471)	(1,418)	(1,413)
Interest and other income, net	709	1,327	2,528	6,650
Accretion of discount and amortization of premium on investments, net	1,679	1,849	6,089	6,855
Net Segment loss and Net loss	$ (50,332)	$ (66,719)	$ (180,585)	$ (198,419)

The Company's CODM uses net loss to evaluate past spending and to guide decisions about future spending. Net loss is used to monitor the 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 $3.3 million in restructuring costs related to severance and employee termination costs to be paid out over multiple months through the second half of 2025.

The following table summarizes the accrued liabilities activity in connection with the restructuring plan for the nine months ended months ended September 30, 2025:

	Nine Months Ended September 30, 2025
Beginning balance	$ -
Restructuring charges incurred during the period	3,299
Restructuring charges paid during the period	(539)
Remaining accrual at September 30, 2025	$ 2,760

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

You should read the following discussion and analysis of our financial condition and results of operations together with the consolidated financial statements and related notes that are included elsewhere in this Quarterly Report on Form 10-Q and our annual report on Form 10-K, filed on February 27, 2025, 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, late-stage biotechnology company focused on the development, manufacturing, and potential commercialization of genetic therapies for rare and often fatal diseases with a high unmet medical need. Rocket's innovative multi-platform approach allows for the creation of best-in-class gene therapy product candidates aimed at correcting the root cause of complex genetic disorders, spanning across cardiac and hematologic indications, offering the potential for transformative and durable clinical benefits. Rocket's platform is supported by in-house R&D capabilities and current cGMP facilities that enable end-to-end control over clinical production and scale-up for commercialization.

Our Strategy

We seek to bring hope and relief to patients with devastating, undertreated and rare diseases through the development and commercialization of potentially curative first-in-class gene therapies. As a fully integrated, late-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 designed to maximize near-term value, extend our operational runway, and position the Company for sustained long-term growth. This initiative focuses our resources on advancing our AAV cardiovascular gene therapy platform and supporting the submission of our responses to the CRL for KRESLADI™. The program contemplates a scaled commercial effort tailored to the exceptionally small patient population affected by this ultra-rare indication. As part of this strategic realignment, we are also de-prioritizing further development activities related to our FA and PKD programs. As part of the restructuring, the Company implemented a reduction in the workforce of approximately 30%, which, along with other planned cost-saving initiatives, is expected to reduce Rocket's 12-month operating expenses by nearly 25%.

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 diseases with substantial unmet need.
- Building proprietary in-house analytics and manufacturing capabilities.
- Conducting registration trials for our lead programs.

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.
- Pursue potential eligibility for FDA priority review vouchers, pending program renewal by Congress.

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.

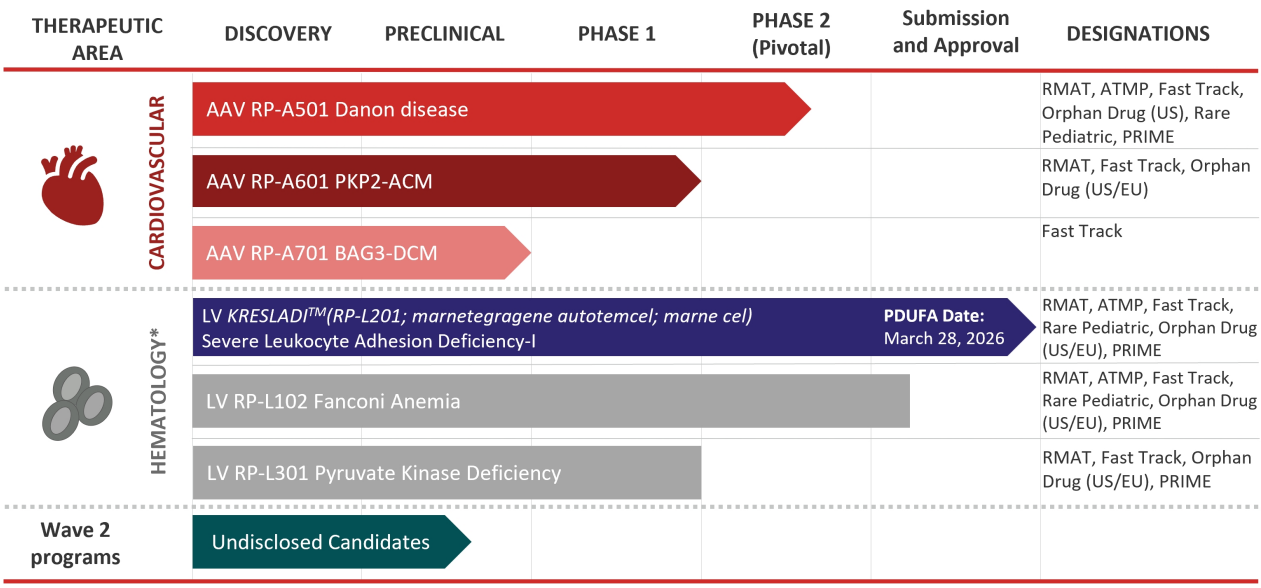
We are developing gene therapy product candidates utilizing modified, non-pathogenic viruses as delivery vehicles. Viruses are inherently effective for gene delivery due to their natural ability to enter cells and deliver genetic material. In engineering our viral vectors, the native viral genes are 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 two categories of viral vectors: AAV vectors and LV vectors. We believe that our AAV- and LV-based programs have the potential to confer significant and durable therapeutic benefits. Our gene therapy product candidates are administered either (1) *ex vivo*, whereby a patient's cells are collected, transduced with the viral vector in a controlled laboratory environment, and subsequently reinfused into the patient, or (2) *in vivo*, whereby the viral vector is delivered directly into the patient, either intravenously or through targeted tissue injection, to enable in situ transduction of the desired cell populations.

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:



*In its LV portfolio, Rocket is prioritizing KRESLADI and seeking external partnership opportunities for the RP-L102 and RP-L301 programs.

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

Cardiovascular Programs

Danon disease

Danon disease, otherwise known as 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 often require heart transplantation and typically die during adolescence or early adulthood from progressive heart failure. Along with severe cardiomyopathy, other DD-related manifestations can include skeletal muscle weakness and intellectual impairment. There are no specific therapies available for the treatment of DD and medications typically utilized for the treatment of 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.
- ECG 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 has been shown to be markedly elevated in patients with advanced stage disease.
- KCCQ is a validated, 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 score of +/- 5 points are considered meaningful and have been shown to correlate with heart failure 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 disclosed, 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 (35%) on echocardiogram and markedly elevated left ventricle filling pressure prior to treatment. The patient's clinical course was characteristic of DD progression. The patient is doing well post-transplant.

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

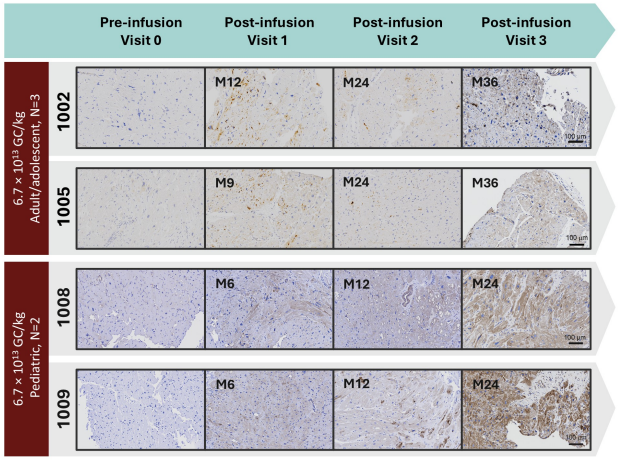
Myocardial LAMP2 Protein Expression

Cohort	Patient	BL	M6	M12	M18	M24	M30	M36	M60‡
6.7 × 10^{13} GC/kg Adult/adolescent	1001	0	++	+	NP	NP	0*	0*	++
	1002	0	NP	++	++	++	++	++	
	1005	0	NP	++†	NP	+	+	+	
1.1 × 10^{14} GC/kg Adult/adolescent[a]	1006	0	+	+	+	+	NP	++	
6.7 × 10^{13} GC/kg Pediatric	1008	0	++	+	NP	++	Visits Pending		
	1009	0	+	++	NP	++			

†Reflects patient 1005 9M visit biopsy as 12M biopsy not performed
‡ Preliminary assessment of biopsy from 1001 Y5 visit with updated IHC assay

Legend: IHC Staining Grade (% Positive Cardiomyocytes)
0 = no staining; + = 1 (≤25%); ++ = 3 (51%–74%)
NP = not performed; ++ = 2 (26%–50%); ++ = 4 (≥75%)

Representative LAMP2 IHC Images



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
a. 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 1 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, TnI 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$^{2.7}$)	Δ IVSd, BL to RV (mm)	Δ LVPWd, BL to RV (mm)	Δ NT-proBNP, BL to RV (ng/L)	Δ cTnI,† 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

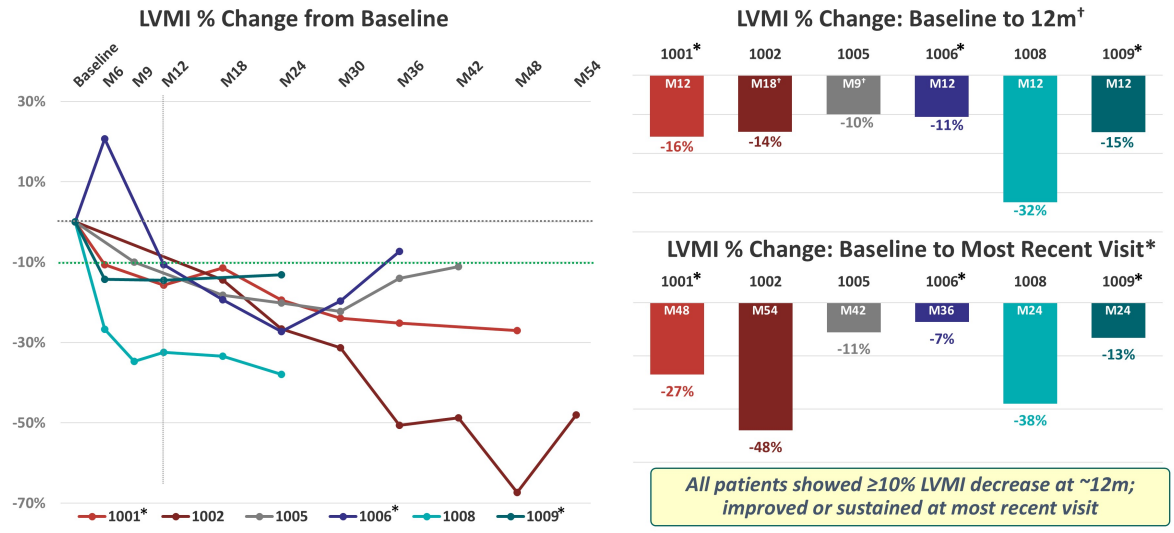
* Centrally evaluated (blinded) MRI data were utilized for LVMI when available. All other measurements of cardiac structure and function reflect centrally evaluated (blinded) echocardiogram data.
† Central laboratory assessment of cTnI were performed on cryopreserved and non-cryopreserved samples. Values for cTnI 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; cTnI=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 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. These are preliminary results with a formal update anticipated to be presented at an upcoming medical conference.

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



Data published in the *New England Journal of Medicine* (Greenberg et al 2024; (DOI: 10.1056/NEJMoa2412392); Data cut-off: April 19, 2024.
* Where possible, cardiac MRI assessments shown (patients 1001, 1006, and 1009); otherwise, echocardiogram data presented. All assessments were conducted by a single reviewer blinded to both patient and timepoint, except for Patient 1001 cardiac MRI data, which includes reads from multiple reviewers (note, these data not included in *NEJM* publication). Patient 1001 most recent visit with MRI assessment was at 48m
† Utilized 9m or 18 m data when 12m assessment was not done.
LVMI, left ventricular mass index; MRI, magnetic resonance imaging; m, month(s).

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 10^{13} GC/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 left ventricular mass.

Key secondary endpoint is change in troponin. Additional secondary endpoints will include natriuretic peptide, KCCQ, NYHA class, event free survival to 24 months and treatment emergent safety events. These endpoints could support full approval with longer-term follow-up.

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. These batches have superior specifications to Phase 1 material in both titer and full versus empty particles. We believe the improved quality of our in-house manufactured product will allow for full dosing with lower total viral particles. 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 and were followed by a systemic infection and multi-organ damage, ultimately leading to one of the patient's death. 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 after the investigation traced the unexpected SAEs to a protocol amendment that added a C3 inhibitor to the immunomodulatory regimen. 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. In addition, Rocket will collaborate with investigators to implement an immunomodulatory regimen more closely reflecting that administered in the Phase 1 pediatric cohort. The revised regimen also discontinues prophylactic use of a C3 complement inhibitor.

To date, six patients with Danon disease have been treated in the Phase 2 study with RP-A501. Further updates about 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 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 arrythmias 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 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 US 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*.

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. The study 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 x 10^{13} GC/kg. Enrollment in the U.S. Phase 1 study has completed. We are currently in ongoing discussions with the FDA regarding the design and potential endpoints 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 (n=3) followed for up to 12 months. ·Most treatment emergent adverse events were mild/moderate in severity and self-limited with only one patient experiencing SAEs 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 indications of improvement or stabilization in arrhythmia burden, heart function, and quality of life were observed. At this time, we believe that we have reached both the safe and efficacious dose and based on the overall risk benefit seen so far, we are no longer dose escalating in this trial and will move 8 x 10^{13} GC/kg into the next phase of development.

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 heart failure with reduced ejection fraction (HFrEF), including the use of beta-adrenergic receptor antagonists (beta-blockers), angiotensin converting enzyme (ACE) inhibitors, angiotensin receptor antagonists/neprilysin 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. The understanding of the genetic mechanism of disease affords 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 AAV.rh74-based gene therapy candidate for the treatment of BAG3-DCM. Following, the FDA granted Fast Track designation to RP-A701 for the treatment of BAG3-DCM in July 2025. 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, resulting from mutations in the *ITGB2* gene encoding for the Beta-2 Integrin component, CD18. Deficiencies in CD18 result in an impaired ability for neutrophils (a subset of infection-fighting white blood cells) to leave blood vessels and enter tissues where these cells are needed to combat infections. As is the case with many rare diseases, accurate estimates of incidence are difficult to confirm; however, several hundred cases across the spectrum of severity have been reported to date. Most LAD-I patients are believed to have the severe form of the disease. Severe LAD-I is notable for recurrent, life-threatening infections and substantial infant mortality in patients who do not receive an allogeneic HSCT. Mortality for severe LAD-I has been reported as 60 to 75% by age two in the absence of allogeneic HCST.

KRESLADI™, formally known as RP-L201 or marnetegragene autotemcel, is our investigational gene therapy that contains autologous (patient-derived) hematopoietic stem cells that have been genetically modified with a lentiviral vector to deliver a functional copy of the *ITGB2* gene. Rocket 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. 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 between University College London and CIEMAT.

The open-label, single-arm, global Phase 1/2 registration-enabling clinical trial of RP-L201 for severe LAD-I treated nine patients. In May 2024, we presented updated follow-up data at the ASGCT 27th Annual Meeting, including 18- to 45-month follow-up data (data cut-off July 24, 2023). Compared to pre-treatment history, patients demonstrated substantial 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 capabilities. RP-L201 remained well tolerated, with no new safety events related to the treatment. We continued to observe 100% survival without the need for allogeneic transplant, with all patients enrolled at less than 12 months of age surpassing 24 months without transplant. The clinical outcomes data from the nine severe LAD-I patients treated with KRESLADI™ was published in the *New England Journal of Medicine (NEJM)* in May 2025.

In September 2023, A BLA filing for RP-L201 was accepted by the FDA with priority review with an initial PDUFA date of March 31, 2024. In February 2024, the review time was extended by three months, to June 30, 2024, to allow additional time to review clarifying CMC information submitted by us in response to FDA information requests. In June 2024, we announced that the FDA issued a CRL in response to the BLA wherein the FDA requested limited additional CMC information to complete its review. In October 2025, the Company announced that the FDA accepted its resubmission of the BLA for RP-L201 and was given a PDUFA date of March 28, 2026.

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. An estimated 60% to 70% of cases arise from mutations in the *FANCA* gene. FA frequently results in bone marrow failure, developmental abnormalities, acute myeloid leukemia, and other myelodysplastic syndrome types of blood cancers, often during the early years and decades of life. Bone marrow aplasia, which is bone marrow that no longer produces any or very few red and white blood cells and platelets leading to infections and bleeding, is the most frequent cause of early morbidity and mortality in FA, with a median onset before 10 years of age. Leukemia is the next most common cause of mortality, ultimately occurring in about 20% of patients later in life. Solid organ malignancies, such as head and neck cancers, can also occur, although at lower rates during the first two to three decades of life. The average lifespan of an FA patient is estimated to be 30 to 40 years. The prevalence of FA in the U.S. and EU is estimated to be approximately 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 lentiviral vector 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. Rocket 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 2023, we presented updated clinical data for RP-L102 at the ASGCT 26th Annual Meeting. As of the data cut-off (April 17, 2023), RP-L102 conferred sustained genetic correction in 8 of 12 evaluable patients and comprehensive phenotypic correction in 7 of 12 evaluable patients with ≥12 months of follow-up as demonstrated by increased resistance to mitomycin-C in bone marrow-derived colony forming cells and hematologic stabilization. The safety profile of RP-L102 has been highly favorable, and the treatment, administered without any cytotoxic conditioning, has been well tolerated. No signs of bone marrow dysplasia, clonal dominance or insertional mutagenesis related to RP-L102 have been observed. Polyclonal integration patterns have been observed in each of the seven patients with phenotypic, genetic, and hematologic evidence of engraftment.

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. Children are the most commonly and severely affected subgroup of patients. Patients with PKD have a high unmet medical need, as currently available treatments include splenectomy and red blood cell transfusions, which are associated with immune defects and chronic iron overload. Mitapivat, an oral enzyme activator, is approved for use in adult patients, however its efficacy is limited in more severely-afflicted patients, most notably in those who are splenectomized, transfusion-dependent, or whose disease results from deleterious mutations.

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

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 transfusion 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 the Phase 2 RP-L301 study at this time. Like 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 research and development. Research focus areas include the development of new candidates following our strategy outlined in “Our Strategy” 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, R&D activities for our product candidates and planning for potential commercialization. We do not have any products approved for sale and have not generated any revenue from product sales. From inception through September 30, 2025, we raised net cash proceeds of approximately $1.2 billion from investors through both equity and convertible debt financing to fund operating activities.

Revenue

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

Research and Development Expenses

Our R&D program expenses consist 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 and nine months ended September 30, 2025, and 2024:

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2025	2024	2025	2024
Direct Expenses:				
Danon Disease (AAV) RP-A501	$ 5,593	$ 6,442	$ 12,934	$ 18,912
Plakophilin-2 Arrhythmogenic Cardiomyopathy (AAV) RP-A601	1,488	2,217	5,623	4,724
Leukocyte Adhesion Deficiency (LV) RP-L201	1,051	3,490	8,381	11,557
Fanconi Anemia (LV) RP-L102	2,113	3,421	13,409	12,856
Pyruvate Kinase Deficiency (LV) RP-L301	548	1,664	2,432	8,750
Other product candidates	939	2,435	2,439	7,728
Total direct expenses	11,732	19,669	45,218	64,527
Unallocated Expenses:				
Employee compensation	12,754	12,297	37,486	38,311
Stock-based compensation expense	4,752	4,789	13,961	14,311
Depreciation and amortization expense	1,515	1,522	4,993	4,542
Laboratory and related expenses	1,167	890	3,986	3,134
Professional fees	518	705	3,107	3,971
Other expenses	1,630	2,443	3,917	5,091
Total other research and development expenses	22,336	22,646	67,450	69,360
Total research and development expense	$ 34,068	$ 42,315	$ 112,668	$ 133,887

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 remain 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 in 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 remain significant for the foreseeable future due to the 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 significant 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 $3.3 million in restructuring expenses, consisting of employee severance payments and other termination benefits.

Interest Expense

Interest expense for the three and nine months ended September 30, 2025, and 2024 was related to our financing lease obligation for our Cranbury, NJ facility.

Interest and Other Income

Interest and other income for the three and nine months ended September 30, 2025, was related to interest earned from investments and cash equivalents. Interest and other income for the three and nine months ended September 30, 2024, 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 nine months ended September 30, 2025, compared to those disclosed in our 2024 Form 10-K.

Results of Operations

Comparison of the Three Months Ended September 30, 2025 and 2024

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

	Three Months Ended September 30,		
	2025	2024	Change
Operating expenses:			
Research and development	$ 34,068	$ 42,315	$ (8,247)
General and administrative	18,351	27,109	(8,758)
Restructuring	(172)	-	(172)
Total operating expenses	52,247	69,424	(17,177)
Loss from operations	(52,247)	(69,424)	17,177
Interest expense	(473)	(471)	(2)
Interest and other income, net	709	1,327	(618)
Accretion of discount on investments, net	1,679	1,849	(170)
Total other income, net	1,915	2,705	(790)
Net loss	$ (50,332)	$ (66,719)	$ 16,387

Research and Development Expenses

R&D expenses decreased $8.2 million to $34.1 million for the three months ended September 30, 2025, compared to the three months ended September 30, 2024. The decrease in R&D expenses was primarily driven by decreases in manufacturing and development and direct costs of $3.6 million, clinical trial expenses of $2.4 million, and professional fees of $2.0 million.

General and Administrative Expenses

G&A expenses decreased $8.8 million to $18.4 million for the three months ended September 30, 2025, compared to the three months ended September 30, 2024. The decrease in G&A expenses was primarily driven by decreases in commercial preparation related expenses of $6.6 million and stock based compensation expenses of $1.5 million.

Restructuring Expense

Restructuring expenses decreased $0.2 million for the three months ended September 30, 2025. The decrease in restructuring expenses was driven by a decrease in the planned reduction of the Company’s workforce during the three months ended September 30, 2025.

Other Income, Net

Other income decreased $0.8 million to $1.9 million for the three months ended September 30, 2025, compared to the three months ended September 30, 2024. The decrease in other income was primarily driven by a decrease in interest and other income, net, of $0.6 million due to a decrease in interest earned on investments due to lower interest rates year over year and a decrease in fair value of warrant liabilities in 2024 and a decrease in accretion of discount on investments, net, of $0.2 million.

Comparison of the Nine Months Ended September 30, 2025 and 2024

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

	Nine Months Ended September 30, 2025	2024	Change
Operating expenses:			
Research and development	$ 112,668	$ 133,887	$ (21,219)
General and administrative	71,817	76,624	(4,807)
Restructuring	3,299	-	3,299
Total operating expenses	187,784	210,511	(22,727)
Loss from operations	(187,784)	(210,511)	22,727
Interest expense	(1,418)	(1,413)	(5)
Interest and other income, net	2,528	6,650	(4,122)
Accretion of discount and amortization of premium on investments, net	6,089	6,855	(766)
Total other income, net	7,199	12,092	(4,893)
Net loss	$ (180,585)	$ (198,419)	$ 17,834

Research and Development Expenses

R&D expenses decreased $21.2 million to $112.7 million for the nine months ended September 30, 2025, compared to the nine months ended September 30, 2024. The decrease in R&D expenses was primarily driven by decreases in manufacturing and development and direct costs of $8.4 million, professional fees of $5.7 million, lab supplies and office expense of $3.2 million, and compensation and benefits expense of $0.9 million due to decreased R&D headcount. Reflected in the decrease in R&D expenses was the receipt of $2.7 million of CIRM grant recorded as a reduction of R&D expenses in the first quarter of 2025.

General and Administrative Expenses

G&A expenses decreased $4.8 million to $71.8 million for the nine months ended September 30, 2025, compared to the nine months ended September 30, 2024. The decrease in G&A expenses was primarily driven by decreases in commercial preparation related expenses of $6.5 million and stock based compensation expenses of $1.5 million. The decrease in G&A expenses was partially offset by increases in legal expenses of $3.7 million.

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 $3.3 million in restructuring expenses, consisting of employee severance payments and other termination benefits.

Other Income, Net

Other income decreased $4.9 million to $7.2 million for the nine months ended September 30, 2025, compared to the nine months ended September 30, 2024. The decrease in other income was primarily driven by a decrease in interest and other income, net, of $4.1 million due to a decrease in interest earned on investments due to lower interest rates year over year and a decrease in fair value of warrant liabilities in 2024 and a decrease in accretion of discount on investments, net, of $0.8 million.

Liquidity and Capital Resources

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

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

Our consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities in the ordinary course of business. We have incurred net losses and negative cash flows from its operations each year since inception. We incurred net losses of $180.6 million for the nine months ended September 30, 2025, and $258.7 million for the year ended December 31, 2024. We have experienced negative cash flows from operations and as of September 30, 2025, and December 31, 2024, we had an accumulated deficit of $1.40 billion and $1.22 billion, respectively. As of September 30, 2025, we had $222.8 million of cash, cash equivalents and investments. We believe that in accordance with the current operating plan, which reflects a strategic corporate reorganization and reprioritization announced in July 2025, such resources will be sufficient to fund our operating expenses and capital expenditure requirements into the second quarter of 2027. We have financed our operations primarily through proceeds from the sale of equity securities and continue to manage our capital resources with discipline and a focus on 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. We may also raise capital through the sale of priority review vouchers to third parties, 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:

	Nine Months Ended September 30,	
	2025	**2024**
Net cash used in operating activities	$ (155,174)	$ (162,782)
Net cash provided by investing activities	67,342	169,577
Net cash provided by financing activities	148	2,890
Net (decrease) increase in cash, cash equivalents and restricted cash	$ (87,684)	$ 9,685

Operating Activities

During the nine months ended September 30, 2025, operating activities used $155.2 million of cash and cash equivalents, primarily resulting from our net loss of $180.6 million offset by net non-cash charges of $33.9 million, including non-cash stock-based compensation expense of $30.9 million, depreciation and amortization expense of $8.8 million, partially offset by accretion of discount on investments of $5.9 million. Changes in our operating assets and liabilities for the nine months ended September 30, 2025, included a decrease in accounts payable and accrued expenses of $9.4 million and an increase in our prepaid expenses of $0.9 million.

During the nine months ended September 30, 2024, operating activities used $162.8 million of cash and cash equivalents, primarily resulting from our net loss of $198.4 million offset by net non-cash charges of $31.1 million, including non-cash stock-based compensation expense of $32.8 million, depreciation and amortization expense of $7.0 million, partially offset by accretion of discount on investments of $6.8 million and reduction in fair value of warrant liabilities of $1.9 million. Changes in our operating assets and liabilities for the nine months ended September 30, 2024, included an increase in accounts payable and accrued expenses of $5.3 million and an increase in our prepaid expenses of $1.0 million.

Investing Activities

During the nine months ended September 30, 2025, net cash provided by investing activities was $67.3 million, primarily resulting from proceeds of $289.8 million from the maturities of investments, offset by purchases of investments of $222.1 million, and purchases of property and equipment of $0.4 million.

During the nine months ended September 30, 2024, net cash provided by investing activities was $169.6 million, primarily resulting from proceeds of $297.0 million from the maturities of investments, offset by purchases of investments of $121.8 million, and purchases of property and equipment of $5.6 million.

Financing Activities

During the nine months ended September 30, 2025, financing activities provided $0.1 million of cash, consisting of return of short-swing profits of $0.2 million partially offset by repurchase of RSUs of $0.1 million.

During the nine months ended September 30, 2024, financing activities provided $2.9 million of cash, consisting of proceeds from the exercise of stock options.

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 September 30, 2025, and December 31, 2024, we had cash, cash equivalents and investments of $222.8 million and $372.3 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 September 30, 2025, the net effect on the net fair value of our investments would have resulted in a hypothetical decline of $0.3 million. While we believe our cash, cash equivalents, and marketable securities do not contain excessive risk, we cannot provide absolute assurance that, in the future, our investments will not be subject to adverse changes in market value.

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 September 30, 2025, 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

In June 2025, the Company entered into a settlement agreement with Lexeo Therapeutics, Inc. ("Lexeo") to resolve all claims in ongoing litigation between the parties in the United States District Court for the Southern District of New York. The litigation involved allegations by the Company of trade secret misappropriation and tortious interference, and counterclaims by Lexeo including correction of inventorship, breach of contract, and trade secret misappropriation. As part of a comprehensive resolution, Rocket also entered into separate settlement agreements on the same day with certain individuals formerly affiliated with the Company, who were also named in the litigation.

Under the terms of the settlement agreement between the Company and Lexeo, the litigation has been resolved amicably and fully, and without admission of liability by any party. For the three and nine months ended September 30, 2025, the gain or loss in connection with this settlement agreement was not material.

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 and between September 17, 2024 and May 26, 2025, 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 will have until November 18, 2025 to file a consolidated amended complaint. We intend to vigorously defend against such allegations. 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.

A putative derivative action was filed on October 22, 2025 in the District of New Jersey, naming as Defendants certain of our 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. We intend to vigorously defend the litigation. 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 2024 Form 10-K. Other than the below, there have been no material changes from the risk factors previously disclosed in such filing.

We are and may continue to be targets of securities-related class action and derivative lawsuits and defending against these claims could result in substantial costs and divert management time and resources and have a material adverse effect on our results of operations. These lawsuits, and any other lawsuits to which we are subject, may be costly to defend or pursue and are uncertain in their outcome

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 and between September 17, 2024 and May 26, 2025, 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.

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The outcome of litigation is necessarily uncertain, and we cannot predict the outcome of these pending legal proceedings. An unfavorable outcome in any such proceeding could have an adverse impact on our business, financial condition, results of operations and cash resources. In addition, we may be exposed to additional litigation even if no wrongdoing occurred and we ultimately prevail. Continuing or additional litigation, or responding to any related investigation or enforcement action, as well as a material final judgment or decree against us, would be expensive, divert management's attention and resources, and could adversely affect our business, financial condition, results of operations and cash resources. Moreover, if our stock price is volatile, we could face additional securities class action lawsuits in the future.

Our strategic restructuring may not result in the savings we anticipate, could result in total costs and expenses that are greater than expected and could disrupt our operations

On July 23, 2025, the Company began implementation of a strategic corporate reorganization and pipeline prioritization aimed at maximizing near-term value, extending operational runway into the second quarter of 2027, and positioning the company for sustained long-term growth. As part of the restructuring, the Company implemented a reduction in workforce of approximately 30% (the "RIF"), which, along with other planned cost-saving initiatives, is expected to reduce the Company's 12-month operating expenses by nearly 25%.

We may not realize, in full or in part, the anticipated benefits, savings and improvements from our restructuring and RIF due to unforeseen difficulties, delays or unexpected costs. If we are unable to realize the expected operational efficiencies and cost savings from our restructuring, our operating results and financial condition would be adversely affected. We also cannot guarantee that we will not have to undertake additional workforce reductions or restructuring activities in the future. In addition, the RIF could yield unanticipated consequences, such as attrition beyond planned staff reductions, or disruptions in our day-to-day operations. The RIF could also harm our ability to attract and retain qualified management, scientific, clinical, manufacturing and sales and marketing personnel who are critical to our business. Any failure to attract or retain qualified personnel could prevent us from successfully developing and commercializing, if approved, our product candidates,

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 September 30, 2025, 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)
10.1	Separation and Release Agreement, dated as of September 11, 2025, by and between Rocket Pharmaceuticals, Inc. and Kinnari Patel (incorporated by reference to Exhibit 10.1 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on September 17, 2025)
10.2	Consulting Agreement, dated as of September 11, 2025, by and between Rocket Pharmaceuticals, Inc. and Kinnari Patel (incorporated by reference to Exhibit 10.2 to the Company's Current Report on Form 8-K (001-36829), filed with the SEC on September 17, 2025)
10.3*	Executive Employment Agreement, dated August 29, 2025, by and between the registrant and Syed Rizvi
10.4*	First Amendment to Executive Employment Agreement, dated October 1, 2025, by and between the registrant and Syed Rizvi
10.5*	First Amendment to Executive Employment Agreement, dated June 18, 2025, by and between the registrant and Aaron Ondrey
31.1*	Certification of Principal Executive Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
31.2*	Certification of Principal Financial Officer pursuant to Rule 13a-14(a) or Rule 15d-14(a) of the Securities Exchange Act of 1934, as adopted pursuant to Section 302 of the Sarbanes-Oxley Act of 2002
32.1**	Certification of Principal Executive Officer and Principal Financial Officer pursuant to 18 U.S.C. Section 1350, as adopted pursuant to Section 906 of the Sarbanes-Oxley Act of 2002
101.INS	Inline XBRL Instance Document
101.SCH	Inline XBRL Taxonomy Extension Schema With Embedded Linkbase Documents.
104	Cover Page Interactive Data File (the cover page XBRL tags are embedded within the Inline XBRL document)

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

SIGNATURES

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

ROCKET PHARMACEUTICALS, INC.

November 6, 2025

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

November 6, 2025

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

Exhibit 10.3

EXECUTIVE EMPLOYMENT AGREEMENT

THIS EXECUTIVE EMPLOYMENT AGREEMENT (this "**Agreement**") is between Rocket Pharmaceuticals, Inc. a Delaware corporation (the "**Company**") and Syed Rizvi ("**Executive**") and is dated as of August 29, 2025 (the "**Effective Date**"). Each of the Company and Executive are sometimes referred to in this Agreement individually as a "**Party**" and together as the "**Parties**."

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

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

1. Employment.

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

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

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

(a)*Base Salary*. During the Term, the Company will pay Executive, as compensation for Executive's services, a base salary at a gross annual rate of $550,000.00, less all required tax withholdings and other applicable deductions, in accordance with the Company's standard payroll procedures. The annual compensation specified in this Section 2(a), together with any modifications in such compensation that the Company may make from time to time in accordance with the following sentence, is referred to in this Agreement as the "**Base Salary**." Executive's Base Salary will be subject to review in accordance with the Company's normal performance review practices. Effective as of the date

of any change to Executive's Base Salary, the Base Salary as so changed will be considered the new Base Salary for all purposes of this Agreement.

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

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

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

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

(c)*Annual Incentive Bonus*. Subject to the subsections set forth herein, Executive will be eligible to receive an annual cash incentive bonus (the "**Incentive Bonus**"). The Compensation Committee of the Board (the "**Compensation Committee**") will establish the applicable performance goals required to be met by the Company for a fiscal year in order for Executive to be eligible for an Incentive Bonus, payable no later than March 15 of the following fiscal year. If the Company achieves the applicable performance goals for any such fiscal year, the Compensation Committee will determine Executive's actual Incentive Bonus amount in the Compensation Committee's sole and absolute discretion based on its evaluation of Executive's performance. Executive's target Incentive Bonus, assuming Executive fully and satisfactorily meets all expectations and obligations as Chief Medical Officer, as determined by the Compensation Committee in its sole and absolute discretion, shall be 45% of Executive's Base Salary. Notwithstanding the foregoing, Executive's Incentive Bonus payout each year shall not exceed 54% of Executive's Base Salary for that particular year.

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

(ii) If Executive's employment with the Company is terminated by the Company for Cause prior to the date on which any Incentive Bonus for the prior fiscal year to which Executive may be entitled is to be paid, Executive will not be entitled to receive, and

shall forfeit all right, interest and entitlement to, any such Incentive Bonus payment for such fiscal year.

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

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

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

(d) *Long-Term Incentive Awards*.

(i) Effective the first Monday of the month following Executive's start date, Executive will be granted two equity awards covering the Company's common stock (the "**Grants**") under the Amended and Restated Rocket Pharmaceuticals, Inc. 2014 Stock Option and Incentive Plan (the "**Stock Plan**"), as may be amended from time to time or any successor plan. The Grants will have an aggregate grant date fair value for accounting purposes of $1,800,000 (the "**Total Value**") and will be apportioned among the two Grants as set in this Section 2(d) using the closing price on the day of the grant. One of the two Grants, representing 50% of the Total Value, will be in the form of an option to purchase shares of the Company's common stock, and the other of the two Grants, representing 50% of the Total Value, will be in the form of restricted stock units settled in shares of the Company's common stock. The Grants will be subject to the terms and conditions of the Plan and the grant agreements issued by the Company thereunder.

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

(e)*Sign-On Bonus*. As an incentive to join the Company, the Company shall pay Executive a one-time sign-on bonus of $100,000 (the "**Sign-On Bonus**"), subject to the terms outlined below:

(i) The Sign-On Bonus will be paid to Executive in two equal payments

of $50,000, less all required tax withholdings and other applicable deductions, the first of which shall be paid within 30 days of Executive's start date, provided that Executive remains actively employed as of such date. The second payment shall be paid within 30 days the first anniversary of the Executive's start date, provided that Executive remains actively employed as of such date.

(ii) In the event Executive voluntarily resigns Executive's employment with the Company without Good Reason, or Executive is terminated for Cause within 12 months of Executive's start date, Executive agrees to repay the net amount of the Sign-On Bonus Executive received in full within 30 days of Executive's last day of employment. In the event Executive voluntarily resigns Executive's employment with the Company without Good Reason, or Executive is terminated for Cause after 12 months but within 24 months of Executive's start date, Executive agrees to repay half of the net amount of the Sign-On Bonus Executive received within 30 days of Executive's last day of employment.

(iii) Payment of the Sign-On Bonus does not alter the at-will nature of Executive's employment or create any guarantee of continued employment with the Company.

(f)*Additional Equity Award.* Effective the first Monday of the month following Executive's start date, Executive will be granted RSUs having an aggregate grant- date fair market value of $200,000 (the "**RSU Grant**") under the Company's Stock Plan. Subject to the Executive's continuous employment from the start date through each applicable vesting date, 50% of the RSU Grant shall vest on the first anniversary of the grant date, and the remaining 50% of the RSU Grant shall vest on the second anniversary of the grant date. The RSU Grant and the Executive's rights with respect to the RSUs are subject to all of the terms and conditions of the Plan and the applicable RSU award agreement, as may be amended from time to time.

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

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

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

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

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

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

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

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

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

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

form provided by the Company (the "**Release**"), then Executive will receive, subject to such Release becoming irrevocable, in addition to the Accrued Amounts:

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

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

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

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

(i) The Accrued Amounts; and

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

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

(i) The Accrued Amounts;

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

(iii) A lump sum amount equal to any Incentive Bonus to which Executive would have been entitled during the fiscal year in which the termination occurred,

such Incentive Bonus to be determined in the sole and absolute discretion of the Compensation Committee and consistent with any bonus awarded to the Company's Chief Executive Officer in that same year; and

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

(v) A "**Change in Control**" will mean (i) the sale of all or substantially all of the assets of the Company on a consolidated basis to an unrelated person or entity, (ii) a merger, reorganization or consolidation pursuant to which the holders of the Company's outstanding voting power and outstanding stock immediately prior to such transaction do not own a majority of the outstanding voting power and outstanding stock or other equity interests of the resulting or successor entity (or its ultimate parent, if applicable) immediately upon completion of such transaction, (iii) the sale of all of the stock of the Company to an unrelated person, entity or group thereof acting in concert or (iv) any other transaction in which the owners of the Company's outstanding voting power immediately prior to such transaction do not own at least a majority of the outstanding voting power of the Company or any successor entity immediately upon completion of the transaction other than as a result of the acquisition of securities directly from the Company.

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

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

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

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

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

Date, or the Company's employment relationship with Executive may be terminated.

9. Section 409A of the Code.

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

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

(c)*Specified Employee.* Notwithstanding any other provision of this Agreement, if any payment or benefit provided to Executive in connection with Executive's termination of employment is determined to constitute "nonqualified deferred compensation" within the meaning of Section 409A of the Code and Executive is determined to be a "specified employee" as defined in Section 409A(a)(2)(b)(i) of the Code, then such payment or benefit will not be paid until the first payroll date to occur following the six-month anniversary of the Termination Date or, if earlier, on Executive's death (the "Specified Employee Payment Date"). The aggregate of any payments that would otherwise have been paid before the

Specified Employee Payment Date will be paid to Executive in a lump sum on the Specified Employee Payment Date and thereafter, any remaining payments will be paid without delay in accordance with their original schedule.

10. Section 280G of the Code. If any of the payments or benefits received or to be received by Executive (including, without limitation, any payment or benefits received in connection with a Change in Control or Executive's termination of employment, whether pursuant to the terms of this Agreement or any other plan, arrangement or agreement, or otherwise) (all such payments collectively referred to as the "**280G Payments**") constitute "parachute payments" within the meaning of Section 280G of the Code and would, but for this Section 12, be subject to the excise tax imposed under Section 4999 of the Code (the "**Excise Tax**"), then prior to making the 280G Payments, a calculation will be made comparing (i) the Net Benefit (as defined below) to Executive of the 280G Payments after payment of the Excise Tax to (ii) the Net Benefit to Executive if the 280G Payments are limited to the extent necessary to avoid being subject to the Excise Tax. Only if the amount calculated under (i) above is less than the amount under (ii) above will the 280G Payments be reduced to the minimum extent necessary to ensure that no portion of the 280G Payments is subject to the Excise Tax. For purposes of this Agreement, "**Net Benefit**" means the present value of the 280G Payments net of all federal, state, local or foreign income, employment and excise taxes. Any reduction made pursuant to this Section 10 will be made in a manner determined by the Company that is consistent with the requirements of Section 409A of the Code. All calculations and determinations under this Section 10 will be made by an independent accounting firm or independent tax counsel appointed by the Company ("**Tax Counsel**") whose determinations will be conclusive and binding on the Company and Executive for all purposes. For purposes of making the calculations and determinations required by this Section 10, Tax Counsel may rely on reasonable, good faith assumptions and approximations concerning the application of Sections 280G and 4999 of the Code. The Company and Executive will furnish the Tax Counsel with such information and documents as Tax Counsel may reasonably request to make its determinations under this Section 10, and the costs of such determination will be borne by the Company.

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

with Executive's obligations under this Section 10, *provided* that such expenses are approved in advance by the Company.

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

To the Company:

Rocket Pharmaceuticals, Inc.
9 Cedarbrook Drive
Cranbury, NJ 08512
Attn: General Counsel
Telephone Number: (609) 451-2045
E-Mail: mwilson@rocketpharma.com
To Executive:
Syed Rizvi at the contact information on file with the Company

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

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

14. Entire Agreement. This Agreement, the Confidentiality and Restrictive Covenant Agreement, the Indemnification Agreement and any exhibits attached thereto constitute the entire agreement and understanding between the Parties with respect to the subject matter of such agreements, and supersede all other understandings and negotiations with respect thereto.

15. Severability and Headings. It is the intention of the Parties that the provisions in this Agreement will be enforceable to the fullest extent permitted under applicable law, and that the unenforceability of any provisions of this Agreement, or any portion thereof, will not render unenforceable or otherwise impair any other provisions or portions thereof. Each term, condition, covenant or provision of this Agreement will be viewed as separate and distinct, and in the event that any such term, covenant or provision is held by a court of competent jurisdiction to be invalid, the remaining provisions will continue in full force and effect. The

section headings in this Agreement are for reference purposes only and are not intended in any way to describe, interpret, define or limit the extent or intent of this Agreement or of any part of this Agreement.

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

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

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

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

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

21. Certain Acknowledgements. EXECUTIVE ACKNOWLEDGES THAT, BEFORE SIGNING THIS AGREEMENT, EXECUTIVE WAS GIVEN AN OPPORTUNITY TO READ IT, CAREFULLY EVALUATE IT AND ASK ANY QUESTIONS EXECUTIVE

MAY HAVE HAD REGARDING IT OR ITS PROVISIONS. EXECUTIVE ALSO ACKNOWLEDGES THAT EXECUTIVE HAD THE RIGHT TO HAVE THIS AGREEMENT REVIEWED BY AN ATTORNEY OF EXECUTIVE'S CHOOSING AND THAT THE COMPANY GAVE EXECUTIVE A REASONABLE PERIOD OF TIME TO DO SO IF EXECUTIVE SO WISHED. EXECUTIVE FURTHER ACKNOWLEDGES THAT EXECUTIVE IS NOT BOUND BY ANY AGREEMENT THAT WOULD PREVENT EXECUTIVE FROM PERFORMING EXECUTIVE'S DUTIES AS SET FORTH IN THIS AGREEMENT, NOR DOES EXECUTIVE KNOW OF ANY OTHER REASON WHY EXECUTIVE WOULD NOT BE ABLE TO PERFORM EXECUTIVE'S DUTIES AS SET FORTH IN THIS AGREEMENT.

[Signature Page Follows]

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

Company:
ROCKET PHARMACEUTICALS, INC.

Isabel Carmona Chief Resources Office 8/31/2025

Executive:

Syed Rizvi 8/30/2025

Exhibit 10.4

Amendment No. 1 to Executive Employment Agreement
("Amendment No. 1")

Amendment No. 1 Date: October 1, 2025 ("Amendment No.1 Date")

Name of Original Agreement: Executive Employment Agreement (the "Agreement")

Effective Date of Original Agreement: August 29, 2025 ("Effective Date")

Parties: Rocket Pharmaceuticals, Inc. (the "Company") and Syed Rizvi ("Executive")

WHEREAS, the Parties desire to amend certain terms of the Agreement by means of this Amendment No. 1.

NOW, THEREFORE, in order to accommodate the desired amendment(s), the Parties hereby agree as follows:

1. Defined Terms. Capitalized terms used but not defined herein shall have the respective meanings ascribed to such terms in the Agreement.

2. Amendment to the Agreement.

2.1. Section 2(e)(i) (*Sign-On Bonus*) shall be amended as follows:

"(e) Sign-On Bonus. As an incentive to join the Company, the Company shall pay Executive a one-time sign-on bonus of $100,000 (the "Sign-On Bonus"), subject to the terms outlined below:

(i) The Sign-On Bonus will be paid to Executive in two equal payments of $50,000, less all required tax withholdings and other applicable deductions, the first of which shall be paid upon the first regularly scheduled payroll date in January 2026, provided that Executive remains actively employed as of such date. The second payment shall be paid within 30 days the first anniversary of the Executive's start date, provided that Executive remains actively employed as of such date."

3. Ratification of the Agreement. Except as expressly set forth in Article 2 above, the Agreement shall remain unmodified and in full force and effect. The execution, delivery and effectiveness of this Amendment No. 1 shall not, except as expressly provided herein, operate as a waiver of any right, power or remedy of the parties to the Agreement, nor constitute a waiver of any provision of the Agreement.

4. Counterparts. This Amendment No. 1 may be executed in any number of counterparts, each of which shall be an original instrument and all of which, when taken together, shall constitute one and the same agreement.

IN WITNESS WHEREOF, the duly authorized representatives of the Company and Executive have executed this Amendment No. 1 as of the date first above written.

SYED RIZVI ROCKET PHARMACEUTICALS, INC.

Syed Rizvi 10/1/2025 Martin Wilson General Counsel 10/1/2025

Exhibit 10.5

FIRST AMENDMENT TO EXECUTIVE EMPLOYMENT AGREEMENT

This FIRST AMENDMENT TO EXECUTIVE EMPLOYMENT AGREEMENT ("Amendment") is entered into by and between Rocket Pharmaceuticals, Inc., a Delaware corporation (the "Company"), and Aaron Ondrey ("Executive") effective as of June 18, 2025 (the "Effective Date").

WHEREAS, the Company and Executive entered into an Executive Employment Agreement dated March 25, 2024 (the "Employment Agreement"); and

WHEREAS, the Company and Executive desire to amend certain provisions of the Employment Agreement;

NOW, THEREFORE, in consideration of the premises and for valuable consideration the receipt and sufficiency of which is acknowledged, the Company and Executive agree as follows:

1. **Section 3(d) 4 of the Employment Agreement is amended by adding the following new paragraph at the end thereof:**

> A "**Change in Control**" will mean (i) the sale of all or substantially all of the assets of the Company on a consolidated basis to an unrelated person or entity, (ii) a merger, reorganization or consolidation pursuant to which the holders of the Company's outstanding voting power and outstanding stock immediately prior to such transaction do not own a majority of the outstanding voting power and outstanding stock or other equity interests of the resulting or successor entity (or its ultimate parent, if applicable) immediately upon completion of such transaction, (iii) the sale of all of the stock of the Company to an unrelated person, entity or group thereof acting in concert or (iv) any other transaction in which the owners of the Company's outstanding voting power immediately prior to such transaction do not own at least a majority of the outstanding voting power of the Company or any successor entity immediately upon completion of the transaction other than as a result of the acquisition of securities directly from the Company.

Except as set forth in this Amendment, the Employment Agreement shall remain in full force and effect as written.

IN WITNESS WHEREOF, the parties have executed this First Amendment as of the Effective Date.

Company: Rocket Pharmaceuticals, Inc. **Executive:**

By: Isabel Carmona
Name: Aaron Ondrey
Title: Chief Human Resources Officer

Exhibit 31.1

CERTIFICATIONS

I, Gaurav Shah, MD, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended September 30, 2025 of Rocket Pharmaceuticals, Inc.;

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

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

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

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

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

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

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

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

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

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

Date: November 6, 2025

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

Exhibit 31.2

CERTIFICATIONS

I, Martin Wilson, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended September 30, 2025 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: November 6, 2025

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

Exhibit 32.1

CERTIFICATION PURSUANT TO

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

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the quarterly report on Form 10-Q of Rocket Pharmaceuticals, Inc. (the "Company") for the period ended September 30, 2025, as filed with the United States Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers hereby certifies, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, 18 U.S.C. Section 1350, that to his knowledge:

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

Date: November 6, 2025

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

Date: November 6, 2025

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