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

Form 10-Q

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

For the quarterly period ended June 30, 2026 or

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

For the transition period from_____to

Commission File Number: 001-36829

Rocket Pharmaceuticals, Inc.

(Exact name of registrant as specified in its charter)

Delaware	**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 July 31, 2026, there were 109,774,597 shares of common stock, $0.01 par value per share, outstanding.

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Summary of Abbreviated Terms

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

AAV	Adeno-associated virus	IV	Intravenous
AAV9	Adeno-associated virus serotype 9	IPR&D	In process research and development
ACM	Arrhythmogenic cardiomyopathy	KCCQ-12	Kansas City Cardiomyopathy Questionnaire-12
ASC	Accounting Standard Codification	LAD-I	Leukocyte Adhesion Deficiency-I
ASGCT	American Society of Gene & Cell Therapy	LAMP2	Lysosome-associated membrane protein 2
ATMP	Advanced Therapy Medicinal Product	LV	Lentiviral vector
BAG3	BCL2-associated athanogene 3	LVMI	Left ventricular mass index
BAG3-DCM	BCL2-associated athanogene 3 mutations associated with dilated cardiomyopathy	NYHA	New York Heart Association
BLA	Biologics License Application	PDUFA	Prescription Drug User Fee Act
BNP	Brain natriuretic peptide	PKD	Pyruvate Kinase Deficiency
Cantor	Cantor Fitzgerald & Co.	PKP2	Plakophilin-2
cGMP	Current Good Manufacturing Practice	PKP2-ACM	Plakophilin-2 Arrhythmogenic Cardiomyopathy
cKO	conditional knockout	PRV	Rare Pediatric Disease Priority Review Voucher
CIRM	California Institute for Regenerative Medicine	PSU	Performance-Based Restricted Stock Unit
CMC	Chemistry Manufacturing Controls	PRIME	Priority Medicines
CODM	Chief Operating Decision Maker	R&D	Research and development
CRL	Complete Response Letter	Renovacor	Renovacor, Inc. acquired on December 1, 2022
CTIS	Clinical Trials Information System	RIF	Reduction in workforce
DCM	Dilated cardiomyopathy	RMAT	Regenerative Medicine Advanced Therapy
DD	Danon Disease	RSU	Restricted Stock Unit
DNA	Deoxyribonucleic acid	RTW	RTW Investments, L.P
EMA	European Medicines Agency	SAE	Serious Adverse Event
ESB Lease Agreement	Office Lease agreement for office space in the Empire State Building in New York City	SCD	Sudden cardiac death
EU	European Union	SEC	Securities and Exchange Commission
Exchange Offer	Offer to eligible employees to exchange certain stock options between April 27, 2026 and May 26, 2026	Stanford	Center for Definitive and Curative Medicine at Stanford University School of Medicine
FA	Fanconi Anemia	UCLA	University of California, Los Angeles
FDA	U.S. Food and Drug Administration	UCLB	UCL Business PLC
GMP	Good Manufacturing Practice	UCSD	The Regents of the University of California, San Diego
HF	Heart failure	UPC	Unitary Patent Court
HLA	Human Leukocyte Antigen	U.K.	United Kingdom
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 Cardioverter-Defibrillator	USPTO	U.S. Patent and Trademark Office
IND	Investigational New Drug application		

Cautionary Statement Regarding Forward-Looking Statements

This Quarterly Report on Form 10-Q for the quarter ended June 30, 2026 contains forward-looking statements that involve risks and uncertainties, as well as assumptions that, if they do not materialize or prove incorrect, could cause our results to differ materially from those expressed or implied by such forward-looking statements. We make such forward-looking statements pursuant to the safe harbor provisions of the Private Securities Litigation Reform Act of 1995 and other federal securities laws. All statements other than statements of historical facts contained in this Quarterly Report on Form 10-Q are forward-looking statements. In some cases, you can identify forward-looking statements by words such as "aim," "anticipate," "believe," "can," "contemplate," "continue," "could," "design," "develop," "estimate," "expect," "expand," "future," "hope," "intend," "likely," "may," "plan," "potential," "predict," "project," "pursue," "seek," "should," "strategy," "target," "will," "would," or the negative of these words or other comparable terminology. These forward-looking statements include, but are not limited to, statements about:

- our ability to obtain additional funding to conduct our planned R&D efforts;
- our ability to meet our anticipated milestones for our various drug candidates with respect to the initiation and timing of clinical studies;
- federal, state, and non-U.S. regulatory requirements, including regulation of our current or any other future product candidates by the FDA;
- the timing of and our ability to submit regulatory filings with the FDA and to obtain and maintain FDA or other regulatory authority approval of, or other action with respect to, our product candidates;
- our competitors' activities, including decisions as to the timing of competing product launches, pricing, and discounting;
- whether safety and efficacy results of our clinical trials and other required tests for approval of our product candidates provide data to warrant progression of clinical trials, potential regulatory approval, or further development of any of our product candidates;
- our ability to develop, acquire and advance product candidates into, enroll a sufficient number of patients into, and successfully complete, clinical studies, and our ability to apply for and obtain regulatory approval for such product candidates, within currently anticipated timeframes, or at all;
- our ability to establish key collaborations and vendor relationships for our product candidates and any other future product candidates;
- our ability to develop our sales and marketing capabilities or enter into agreements with third parties to sell and market any of our product candidates;
- our ability to acquire additional businesses, form strategic alliances or create joint ventures and our ability to realize the benefit of such acquisitions, alliances, or joint ventures;
- our ability to successfully develop and commercialize any technology that we may in-license or products we may acquire;
- the development of our direct manufacturing capabilities for our AAV programs;
- our ability to expand our pipeline to target additional indications that are compatible with our genetic medicines technologies;
- our ability to achieve the expected benefits of our portfolio prioritization and strategic restructuring, including extending our cash runway, and our estimates related to the costs and timing of implementing such initiative;
- our ability to successfully operate in non-U.S. jurisdictions in which we currently or in the future do business, including compliance with applicable regulatory requirements and laws;
- our ability to obtain and enforce patents to protect our product candidates, and our ability to successfully defend ourselves against unforeseen third-party infringement claims;
- anticipated trends and challenges in our business and the markets in which we operate;
- our estimates regarding our capital requirements; and
- our ability to obtain additional financing and raise capital as necessary to fund operations or pursue business opportunities.

We caution you that the foregoing list may not contain all of the forward-looking statements made in this Quarterly Report on Form 10-Q.

Any forward-looking statements in this Quarterly Report on Form 10-Q reflect our current views with respect to future events or to our future financial performance and involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance, or achievements to be materially different from any future results, performance or achievements expressed or implied by these forward-looking statements. We have included important factors in the cautionary statements included in this Quarterly Report on Form 10-Q, particularly in the "Risk Factors" section incorporated by reference from our Annual Report for the year ended December 31, 2025, on Form 10-K, that could cause actual results or events to differ materially from the forward-looking statements that we make. Given these uncertainties, you should not place undue reliance on these forward-looking statements. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures, or investments we may make or enter into.

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

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

PART I — FINANCIAL INFORMATION

Item 1. Financial Statements

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

	June 30, 2026	December 31, 2025
	(unaudited)	
Assets		
Current assets:		
Cash and cash equivalents	$ 224,507	$ 77,558
Investments	59,240	111,371
Prepaid expenses and other current assets	5,803	3,805
Total current assets	289,550	192,734
Property and equipment, net	25,099	28,170
Goodwill	39,154	39,154
Intangible assets	25,150	25,150
Restricted cash	1,341	1,340
Deposits	482	482
Operating lease right-of-use assets, net	3,190	3,210
Finance lease right-of-use asset, net	39,132	40,209
Total assets	$ 423,098	$ 330,449
Liabilities and stockholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 28,341	$ 27,291
Operating lease liabilities, current	1,039	1,003
Finance lease liability, current	1,940	1,912
Total current liabilities	31,320	30,206
Operating lease liabilities, non-current	2,488	2,600
Finance lease liability, non-current	19,331	19,362
Other liabilities	1,129	1,060
Total liabilities	54,268	53,228
Commitments and contingencies (Note 14)		
Stockholders' equity:		
Preferred stock, $0.01 par value, authorized 5,000,000 shares:		
Series A convertible preferred stock; 300,000 shares designated; 0 shares issued and outstanding	-	-
Series B convertible preferred stock; 300,000 shares designated; 0 shares issued and outstanding	-	-
Common stock, $0.01 par value, 180,000,000 shares authorized; 109,539,424 and 108,319,783 shares issued and outstanding as of June 30, 2026 and December 31, 2025, respectively	1,095	1,083
Additional paid-in capital	1,733,460	1,717,408
Accumulated other comprehensive loss	(106)	(31)
Accumulated deficit	(1,365,619)	(1,441,239)
Total stockholders’ equity	368,830	277,221
Total liabilities and stockholders’ equity	$ 423,098	$ 330,449

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

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	29,497	42,658	60,951	78,600
General and administrative	17,425	25,020	34,482	53,466
Restructuring	-	3,471	-	3,471
Total operating expenses	46,922	71,149	95,433	135,537
Loss from operations	(46,922)	(71,149)	(95,433)	(135,537)
Gain from sale of PRV	178,190	-	178,190	-
Interest expense	(473)	(473)	(946)	(945)
Interest and other income, net	347	483	508	1,819
Accretion of discount on investments, net	693	2,220	1,922	4,410
Earnings (losses) before Income Taxes	131,835	(68,919)	84,241	(130,253)
Provision for Income Taxes	(8,621)	-	(8,621)	-
Net income (loss)	$ 123,214	$ (68,919)	$ 75,620	$ (130,253)
Net income (loss) per share - basic	$ 1.09	$ (0.62)	$ 0.67	$ (1.18)
Net income (loss) per share - diluted	$ 1.08	$ (0.62)	$ 0.66	$ (1.18)
Weighted-average common shares outstanding - basic	112,844,880	111,019,647	112,491,433	110,559,113
Weighted-average common shares outstanding - diluted	114,513,964	111,019,647	114,329,763	110,559,113

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

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Net income (loss)	$ 123,214	$ (68,919)	$ 75,620	$ (130,253)
Other comprehensive loss:				
Net unrealized gain (loss) on investments	6	(80)	(75)	(173)
Total comprehensive income (loss)	$ 123,220	$ (68,999)	$ 75,545	$ (130,426)

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

Rocket Pharmaceuticals, Inc.
Consolidated Statements of Stockholders' Equity
For the three and six months ended June 30, 2026 and 2025
($ in thousands except share amounts)
(unaudited)

	Common Stock		Additional Paid-In Capital	Accumulated Other Comprehensive Income/(Loss)	Accumulated Deficit	Total Stockholders' Equity
	Shares	Amount				
Balance at December 31, 2025	108,319,783	$ 1,083	$ 1,717,408	$ (31)	$ (1,441,239)	$ 277,221
Issuance of common stock pursuant to vesting of restricted stock units	803,888	8	(8)	-	-	-
Unrealized comprehensive loss on investments	-	-	-	(81)	-	(81)
Stock-based compensation	-	-	8,493	-	-	8,493
Net loss	-	-	-	-	(47,594)	(47,594)
Balance at March 31, 2026	109,123,671	1,091	1,725,893	(112)	(1,488,833)	238,039
Issuance of common stock pursuant to exercise of stock options	95,537	1	151			152
Issuance of common stock pursuant to vesting of restricted stock units	320,216	3	(3)	-	-	-
Unrealized comprehensive gain on investments	-	-	-	6	-	6
Stock-based compensation	-	-	7,419	-	-	7,419
Net income	-	-	-	-	123,214	123,214
Balance at June 30, 2026	109,539,424	$ 1,095	$ 1,733,460	$ (106)	$ (1,365,619)	$ 368,830

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

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

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

	Six Months Ended June 30,	
	2026	**2025**
Operating activities:		
Net income (loss)	$ 75,620	$ (130,253)
Adjustments to reconcile net income (loss) to net cash used in operating activities:		
Depreciation and amortization of property and equipment	3,172	4,491
Amortization of finance lease right of use asset	1,077	1,077
Stock-based compensation	15,912	21,188
Accretion of discount on investments, net	(1,663)	(4,287)
Gain from sale of PRV, net	(178,190)	-
Changes in operating assets and liabilities:		
Prepaid expenses and other assets	(1,998)	200
Accounts payable and accrued expenses	1,050	2,863
Operating lease liabilities and right of use assets, net	(56)	22
Finance lease liability	-	25
Other liabilities	69	(80)
Net cash used in operating activities	(85,007)	(104,754)
Investing activities:		
Proceeds from sale of PRV, net	178,190	-
Purchases of investments	(63,531)	(192,620)
Proceeds from maturities of investments	117,250	166,760
Purchases of property and equipment	(101)	(414)
Net cash provided by (used in) investing activities	231,808	(26,274)
Financing activities:		
Issuance of common stock pursuant to exercise of stock options	152	
Return of related party short-swing profits	-	215
Finance lease liability	(3)	-
Net cash provided by financing activities	149	215
Net change in cash, cash equivalents and restricted cash	146,950	(130,813)
Cash, cash equivalents and restricted cash at beginning of period	78,898	164,997
Cash, cash equivalents and restricted cash at end of period	$ 225,848	$ 34,184
Supplemental disclosure of non-cash financing and investing activities:		
Net unrealized loss on investments	$ (75)	$ (173)

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

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

1. Nature of Business

Rocket Pharmaceuticals, Inc. (the "Company" or "Rocket") is a fully integrated, commercial-stage biotechnology company advancing genetic medicines for rare and devastating diseases, with a strategic focus on inherited cardiovascular conditions. The Company's prioritized development portfolio consists of AAV-based gene therapies for genetically defined cardiomyopathies, complemented by KRESLADI™, its first FDA-approved product, for the treatment of pediatric patients with severe LAD-I who have biallelic mutations in the *ITGB2* gene and do not have a suitable HLA-matched sibling donor.

Rocket's *in vivo* 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* LV vector-based hematology portfolio includes KRESLADI™, which received accelerated approval from the FDA in March 2026 for the treatment of eligible pediatric patients with severe LAD-I, as well as additional programs for Fanconi Anemia (FA) and Pyruvate Kinase Deficiency (PKD).

2. Risks and Liquidity

The Company has not generated revenue from product sales to date. KRESLADI™ received FDA accelerated approval in March 2026. The Company is currently advancing commercial launch activities, including Qualified Treatment Center onboarding, manufacturing readiness, and patient access initiatives. Commercial product revenue will depend on the timing of treatment center activation and patient treatment. KRESLADI™ was approved under the FDA's accelerated approval pathway based on an increase in neutrophil CD18 and CD11a surface expression. Continued approval may be contingent upon verification and description of clinical benefit through ongoing clinical follow-up and additional post-marketing data collection.

Operations of the Company are subject to certain risks and uncertainties, including, among others, uncertainty of drug candidate development, technological uncertainty, uncertainty regarding patents and proprietary rights, having limited commercial manufacturing, marketing and 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 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 commercialization of KRESLADI™ is subject to significant operational, logistical and reimbursement-related risks. The delivery of autologous gene therapies requires complex coordination, including treatment center onboarding, manufacturing, and vein-to-vein logistics, which may impact the timing and pace of commercial adoption.

The Company's product candidates are in the development and clinical stage. There can be no assurance that the Company's research and development efforts will be successfully completed, that adequate protection for the Company's intellectual property will be obtained, that any additional products developed will obtain necessary government approval, or that any approved products will be commercially successful. 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 technological change and substantial competition from pharmaceutical and biotechnology companies.

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

The Company's consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities in the ordinary course of business. The Company has incurred recurring losses and negative cash flows from operations and had an accumulated deficit of $1.37 billion as of June 30, 2026. As of June 30, 2026, the Company had $283.7 million of cash, cash equivalents, and investments. In April 2026, the Company entered into an agreement to sell its PRV for $180 million, and the transaction closed in June 2026. The proceeds from the sale provided substantial non-dilutive capital, strengthened the Company's balance sheet, enhanced liquidity, and increased the Company's financial flexibility to execute on its focused cardiovascular genetic medicines strategy and other strategic priorities. The additional capital provides the Company with greater flexibility to advance its clinical, regulatory, manufacturing, and commercial priorities in support of its long-term strategic objectives. Based on the Company's current operating plan and its cash, cash equivalents, investments, including proceeds received from the PRV sale, the Company believes it has sufficient capital to fund operations into the second quarter of 2028.

In the longer term, the future viability of the Company is dependent on its ability to generate cash from operating activities or to raise additional capital to finance its operations. The Company expects to continue to generate operating losses for the foreseeable future and to finance its future cash needs through, but not limited to, one or a combination of equity offerings, debt financings, collaborations, strategic partnerships and alliances or licensing arrangements. If the Company is unable to obtain funding, the Company would be forced to delay, reduce or eliminate some or all of its R&D programs, preclinical and clinical testing or commercialization efforts, which could adversely affect its business prospects. The Company's failure to raise capital as and when needed could have a negative impact on its financial condition and ability to pursue its business strategies.

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

3. Basis of Presentation, Principles of Consolidation and Summary of Significant Accounting Policies

Basis of Presentation

The accompanying unaudited interim consolidated financial statements should be read in conjunction with the Company's consolidated financial statements for the year ended December 31, 2025 included in the Annual Report on Form 10-K filed with the Securities and Exchange Commission on February 26, 2026. The unaudited interim consolidated financial statements have been prepared on the same basis as the audited annual financial statements and, in the opinion of management, reflect all adjustments, which include only normal recurring adjustments, necessary for the fair statement of the Company's consolidated financial position as of June 30, 2026 and the results of its operations and its cash flows for the six months ended June 30, 2026. The financial data and other information disclosed in these consolidated notes related to the three and six months ended June 30, 2026 and 2025 are unaudited. The results for the three and six months ended June 30, 2026 are not necessarily indicative of results to be expected for the year ending December 31, 2026 and any other interim periods or any future year or period.

Significant Accounting Policies

The significant accounting policies used in the preparation of these consolidated financial statements for the three and six months ended June 30, 2026 are consistent with those disclosed in Note 3 to the consolidated financial statements in the 2025 Form 10-K with most significant policies also being listed here.

Principles of Consolidation

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

Use of Estimates

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

Cash, Cash Equivalents and Restricted Cash

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

Restricted cash consists of deposits collateralizing letters of credit issued by a bank in connection with the Company's operating leases (see Note 13 "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:

	June 30, 2026	December 31, 2025
Cash and cash equivalents	$ 224,507	$ 77,558
Restricted cash	1,341	1,340
Total cash, cash equivalents and restricted cash	$ 225,848	$ 78,898

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 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 six months ended June 30, 2026 and 2025, there were no unrealized losses that were credit related. For the three and six months ended June 30, 2026, there were unrealized gain on investments of less than $0.1 million and unrealized loss of $0.1 million, respectively. For the three and six months ended June 30, 2025, there were net unrealized losses on investments of $0.1 million and $0.2 million, respectively.

Intangible Assets

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

Goodwill

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

Property and Equipment, Net

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

Fair Value Measurements

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

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

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

Warrants

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

Stock-Based Compensation

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

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

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

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

Net Income (Loss) Per Shares

Basic net income or loss per share is computed by dividing the net income or loss for the period by the weighted-average number of shares of common stock outstanding during the period. Diluted net income per share is computed by dividing the net income for the period by the weighted-average number of shares of common stock plus dilutive potential common stock considered outstanding during the period. Such dilutive shares consist of incremental shares that would be issued upon exercise of the Company's common stock options and the vesting of outstanding unvested RSUs, calculated using the treasury stock method. Dilutive earnings per share is not presented when it would be antidilutive to do so. In periods where losses are reported, the weighted-average number of common stock outstanding excludes common stock equivalents, because their inclusion would be anti-dilutive.

Segment Reporting

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

Recent Accounting Pronouncements

Accounting Pronouncements Not Adopted as of June 30, 2026

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

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

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

4. Fair Value of Financial Instruments

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

		Fair Value Measurements as of June 30, 2026, Using:			
	Amortized Cost	Level 1	Level 2	Level 3	Total
Assets:					
Cash equivalents:					
Money market mutual funds	$ 40,953	$ 40,953	$ -	$ -	$ 40,953
U.S. Treasury securities	-	-	-	-	-
	40,953	40,953	-	-	40,953
Investments:					
U.S. Treasury securities	59,246	-	59,240	-	59,240
	59,246	-	59,240	-	59,240
Total assets	$ 100,199	$ 40,953	$ 59,240	$ -	$ 100,193

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

The amortized cost for marketable debt securities approximated its fair value and these securities matures within one year as of both June 30, 2026 and December 31, 2025.

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

5. Property and Equipment, Net

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

	June 30, 2026	December 31, 2025
Laboratory equipment	$ 32,239	$ 32,138
Machinery and equipment	12,133	12,133
Computer equipment	1,015	1,015
Furniture and fixtures	2,777	2,777
Leasehold improvements	7,327	7,327
Internal use software	1,903	1,903
	57,394	57,293
Less: accumulated depreciation and amortization	(32,295)	(29,123)
Total property and equipment, net	$ 25,099	$ 28,170

During the three and six months ended June 30, 2026, the Company recognized $1.6 million and $3.2 million of depreciation and amortization expense, respectively. During the three and six months ended June 30, 2025, the Company recognized $2.0 million and $4.5 million of depreciation and amortization expense, respectively.

6. Intangible Assets and Goodwill

The Company's intangible assets consisted of an acquired IPR&D asset received in the acquisition of Renovacor with a carrying value of $25.2 million as of June 30, 2026 and December 31, 2025.

The carrying value of goodwill as of June 30, 2026 and December 31, 2025 was $39.2 million.

7. Accounts Payable and Accrued Expenses

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

	June 30, 2026	December 31, 2025
Research and development	$ 9,400	$ 10,302
Employee compensation	7,069	12,294
Professional fees	1,903	2,318
Restructuring	-	46
Income taxes	8,621	-
Other	1,348	2,331
Total accounts payable and accrued expenses	$ 28,341	$ 27,291

8. Stockholders' Equity

At-the-Market Offering Program

On March 10, 2026, the Company entered into a sales agreement (the "Sales Agreement") with Cantor with respect to an at-the-market offering program pursuant to which the Company may offer and sell, from time to time at its sole discretion, shares having an aggregate price of up to $100 million through Cantor as its sales agent. The shares to be offered and sold under the Sales Agreement, if any, will be offered and sold pursuant to the Company's shelf registration statement on Form S-3. The Company filed a prospectus supplement with the SEC on March 10, 2026, in connection with the offer and sale of the shares pursuant to the Sales Agreement. The Company will pay Cantor a cash commission of up to 3.0% of gross proceeds from the sale of the shares pursuant to the Sales Agreement. Through June 30, 2026, the Company has not sold any shares under the at-the-market offering program.

9. Stock-Based Compensation

Stock Option Exchange Program

On April 27, 2026, the Company commenced an offer to exchange certain eligible stock options held by eligible employees of the Company for new stock options (the "Exchange Offer"). Members of the Company's board of directors, the Company's executive officers at the EVP-level and above and past or present advisers, consultants, contractors or former employees of the Company were not eligible to participate. The Exchange Offer expired on May 26, 2026.

Under the Exchange Offer, 157 eligible employees elected to exchange, and the Company accepted for cancellation eligible stock options to purchase an aggregate of 1,376,937 shares of our common stock, representing approximately 94.6% of the total shares of common stock underlying eligible options. Immediately following the expiration of the Exchange Offer, the Company granted replacement stock options to purchase an aggregate of 686,137 shares of our common stock. The exercise price of the replacement stock options was $3.00 per share, which was the closing price of the Company's common stock on May 26, 2026. The replacement stock options are subject to new vesting schedules based on continued service.

The exchange of stock options was subject to modification accounting under ASC 718. Modifications to share-based awards were treated as an exchange of the original award for a new award with total compensation equal to the grant-date fair value of the original award plus any incremental value of the modification. The incremental value was based on the excess of the fair value of the modified award over the fair value of the original award immediately before the modification, calculated using the lattice option pricing model.The incremental share-based compensation resulting from the modification was $0.2 million, which will be recognized together with any unrecognized compensation cost remaining on the exchanged options over the remaining requisite service period of the modified awards.

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:

	Six Months Ended June 30,	
	2026	2025
Risk-free interest rate	2.99%	4.31%
Expected term (in years)	4.24	5.42
Expected volatility	71.87%	71.68%
Expected dividend yield	0.00%	0.00%
Exercise price	$ 4.25	$ 10.51
Fair value of common stock	$ 4.25	$ 10.51

The following table summarizes stock option activity for the six months ended June 30, 2026:

	Number of Shares	Weighted Average Exercise Price	Weighted Average Contractual Term (Years)	Aggregate Intrinsic Value
Outstanding as of December 31, 2025	12,163,019	$ 19.98	4.53	706
Granted	3,433,942	4.25	7.96	
Exercised	(95,537)	1.59		
Cancelled or forfeited	(2,036,132)	23.45		
Outstanding as of June 30, 2026	13,465,292	$ 15.58	4.99	1,106
Options vested and exercisable as of June 30, 2026	8,635,055	$ 21.66	3.04	$ 136
Options unvested as of June 30, 2026	4,830,237	$ 4.71	8.48	$ 970

The weighted average grant-date fair value per share of stock options granted outside the stock option exchange program during the six months ended June 30, 2026, and 2025 was $3.14 and $5.86, respectively.

The total grant date fair value of options vested during the six months ended June 30, 2026 and 2025 was $5.1 million and $18.8 million, respectively.

Total intrinsic value of options exercised during the six months ended June 30, 2026 and 2025 was $0.1 million and $6.9 million, respectively.

Restricted Stock Units

The following table summarizes the Company's RSU activity for the six months ended June 30, 2026:

	Number of Shares	Weighted Average Grant Date Fair Value
Unvested as of December 31, 2025	5,000,566	$ 6.51
Granted	4,338,039	4.19
Vested[(1)]	(1,351,771)	8.22
Forfeited	(691,375)	6.18
Unvested as of June 30, 2026	7,295,459	$ 4.84

(1) Includes 227,667 vested shares for the six months ended June 30, 2026, that were not distributed until July 2026.

The total grant date fair value of RSUs vested during the six months ended June 30, 2026 and 2025 was $11.1 million and $11.1 million, respectively.

The total distribution date fair value of RSUs distributed during the six months ended June 30, 2026 and 2025 was $4.3 million and $3.9 million, respectively.

Performance-Based Restricted Stock Units

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

Stock-Based Compensation Expense

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Stock options	$ 2,536	$ 5,454	$ 6,084	$ 11,361
RSUs	4,883	5,403	9,828	9,827
Total stock-based compensation expense	$ 7,419	$ 10,857	$ 15,912	$ 21,188

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Research and development	$ 3,492	$ 4,821	$ 7,799	$ 9,209
General and administrative	3,927	6,036	8,113	11,979
Total stock-based compensation expense	$ 7,419	$ 10,857	$ 15,912	$ 21,188

As of June 30, 2026, the Company had an aggregate of $44.1 million of unrecognized stock-based compensation expense related to stock options and RSU grants, which is expected to be recognized over a weighted average period of 1.95 years.

10. Warrants

A summary of the warrants outstanding as of June 30, 2026 is as follows:

Exercise Price	Outstanding	Grant/Assumption Date	Expiration Date
$57.11	603,386	December 21, 2020	December 21, 2030
$33.63	301,291	August 9, 2021	August 9, 2031
$22.51	153,155	December 17, 2021	December 17, 2031
$22.51	153,155	December 17, 2021	December 17, 2031
$65.23	760,086	December 1, 2022	September 2, 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

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

11. Net Income (Loss) Per Share

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Numerator:				
Net income (loss) attributable to common stockholders	$ 123,214	$ (68,919)	$ 75,620	$ (130,253)
Denominator:				
Weighted-average common shares outstanding - basic	112,844,880	111,019,647	112,491,433	110,559,113
Effect of dilutive securities	1,669,084	-	1,838,330	-
Weighted-average common shares outstanding - diluted	114,513,964	111,019,647	114,329,763	110,559,113
Net income (loss) per share				
Net income (loss) per share - basic	$ 1.09	$ (0.62)	$ 0.67	$ (1.18)
Net income (loss) per share - diluted	$ 1.08	$ (0.62)	$ 0.66	$ (1.18)

For the three and six months ended June 30, 2026 and 2025, the Company included the 3,126,955 potential shares from pre-funded warrants acquired by RTW in 2023 and the 400,000 potential shares from pre-funded warrants acquired by RTW in 2024 in the basic weighted-average common shares outstanding as the warrants only require the holder to pay $0.01 per share upon exercise. The Company used the treasury stock method for the effect of dilutive securities from RSUs and stock options.

For the three and six months ended June 30, 2026, the Company recorded net income and, as such, used diluted weighted-average common shares outstanding when calculating diluted income per share for the three and six months ended June 30, 2026. Stock options and RSUs that could potentially dilute basic earnings per share ("EPS") in the future are included in the computation of diluted income per share. For the three and six months ended June 30, 2025, the Company recorded a net loss and, as such, diluted loss per share is the same as basic loss per share, as the inclusion of any potentially dilutive securities would be antidilutive. Stock options, RSUs, and PSUs that could potentially dilute basic EPS in the future were excluded from the computation of diluted loss per share because their effect would be antidilutive.

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 income or loss per share:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Warrants exercisable for common shares	1,971,073	1,971,073	1,971,073	1,971,073
Options to purchase common shares	13,148,949	16,362,169	13,027,851	16,362,169
RSUs and PSUs	3,363,715	4,419,582	3,229,942	4,419,582
Excluded from diluted net income (loss) per share	18,483,737	22,752,824	18,228,866	22,752,824

12. Income Taxes

For the three and six months ended June 30, 2026, the Company recorded income tax expense of $8.6 million and $8.6 million, respectively, compared to $0 for the same periods in 2025. The tax provision for the three and six months ended June 30, 2026 was primarily attributable to tax on the gain from the sale of a Priority Review Voucher, partially offset by the release of a valuation allowance associated with the expected utilization of current-year and certain historical tax attributes against that gain.

The Company's tax provision and the resulting effective tax rate for interim periods is determined based upon its estimated annual effective tax rate ("AETR"), adjusted for the effect of discrete items arising in that quarter. The impact of such inclusions could result in a higher or lower effective tax rate during a particular quarter, based upon the mix and timing of actual earnings or losses versus annual projections. In each quarter, the Company updates its estimate of the annual effective tax rate, and if the estimated annual tax rate changes, a cumulative adjustment is made in that quarter.

Deferred tax assets and deferred tax liabilities are recognized based on temporary differences between the financial reporting and tax bases of assets and liabilities using statutory rates. Management of the Company has evaluated the positive and negative evidence bearing upon the realizability of its deferred tax assets, which are comprised principally of net operating loss carryforwards and research and development credits. Under the applicable accounting standards, management has considered the Company's history of losses and concluded that it is more likely than not that the Company will not recognize the benefits of federal and state deferred tax assets. Accordingly, a full valuation allowance has been established against the Company's otherwise recognizable net deferred tax assets.

13. Leases

Finance Lease

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

Estimated rent payments for the NJ Lease Agreement are $1.2 million per annum, payable in monthly installments, and subject to annual base rent increases of 3%. The total commitment under the lease is estimated to be approximately $29.3 million over the 15-year term of the lease. The Company paid a cash security deposit of $0.3 million to the landlord in connection with the NJ Lease Agreement which has been reflected as part of deposits in the Consolidated Balance Sheets as of June 30, 2026 and December 31, 2025.

Operating Leases

During the year ended December 31, 2025, the Company was negotiating a sublease of the ESB lease and determined that the changes in the intended use of the lease represented an impairment indicator as negotiations indicated that the carrying value of the right-of-use asset may not be recoverable. This resulted in a right-of-use asset impairment charge of approximately $0.3 million in 2025. The ESB sublease was completed in April 2026. In conjunction with the completion of the sublease, the Company received a security deposit of approximately $0.1 million. Rental income received under the sublease agreement was less than $0.1 million for the three and six months ended June 30, 2026.

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 June 30, 2026 and December 31, 2025.

In connection with the acquisition of Renovacor, the Company added operating leases for space at facilities in Hopewell, New Jersey. The Company intends to sublease the facilities in Hopewell, New Jersey and signed the first agreement to sublease one of these facilities in January 2024. Rental income received under sublease agreements was less than $0.1 million for the three and six months ended June 30, 2026 and 2025, respectively.

Rent expense excluding rental income was $0.3 million and $0.5 million for the three and six months ended June 30, 2026, respectively. Rent expense excluding rental income was $0.3 million and $0.6 million for the three and six months ended June 30, 2025, respectively.

The total restricted cash balance for the Company's operating and finance leases as of June 30, 2026 and December 31, 2025 was $0.8 million.

The following table summarizes lease cost for the six months ended June 30, 2026 and 2025:

Lease cost	Six Months Ended June 30, 2026	2025
Operating lease cost	$ 454	$ 523
Finance lease cost:		
Amortization of right of use asset	1,077	1,077
Interest on lease liabilities	946	945
Total lease cost	$ 2,477	$ 2,545

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,	Amounts
2026 (six months)	$ 513
2027	872
2028	638
2029	627
2030	557
Thereafter	1,323
Total lease payments	$ 4,530
Less: interest	(1,003)
Total operating lease liabilities	$ 3,527

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

Fiscal Year Ending December 31,	Amounts
2026 (six months)	$ 963
2027	1,969
2028	2,028
2029	2,089
2030	2,152
Thereafter	36,763
Total lease payments	$ 45,964
Less: interest	(24,693)
Total finance lease liability	$ 21,271

The following table summarizes the operating and financing lease liabilities and right-of-use assets as of June 30, 2026 and December 31, 2025:

Leases	June 30, 2026	December 31, 2025
Operating right-of-use assets	$ 3,190	$ 3,210
Operating current lease liabilities	$ 1,039	$ 1,003
Operating noncurrent lease liabilities	2,488	2,600
Total operating lease liabilities	$ 3,527	$ 3,603
Finance right-of-use assets	$ 39,132	$ 40,209
Finance current lease liability	$ 1,940	$ 1,912
Finance noncurrent lease liability	19,331	19,362
Total finance lease liability	$ 21,271	$ 21,274

Other Information	Six Months Ended June 30, 2026	2025
Cash paid for amounts included in the measurement of lease liabilities:		
Operating cash flows from operating leases	$ 510	$ 500
Cash flows from finance lease	$ 948	$ 921

	As of June 30, 2026	2025
Weighted-average remaining lease term - operating leases	5.7 years	6.4 years
Weighted-average remaining lease term - finance lease	18.2 years	19.2 years
Weighted-average discount rate - operating leases	8.94%	8.83%
Weighted-average discount rate - finance lease	8.96%	8.96%

14. Commitments and Contingencies

Litigation

On June 11, 2025 and July 18, 2025, two stockholders filed putative securities class action lawsuits against the Company and certain of its executive officers in the United States District Court for the District of New Jersey, purportedly on behalf of classes of the Company's investors who purchased or otherwise acquired the Company's common stock between February 27, 2025 and May 26, 2025 (Ho v. Rocket Pharmaceuticals, Inc., and Gaurav Shah, Case No. 3:25-cv-10049) and between September 17, 2024 and May 26, 2025 (Yankov v. Rocket Pharmaceuticals, Inc., Gaurav Shah and Aaron Ondrey, 3:25-cv-13532), respectively. The complaints allege violations of Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 and Rule 10b-5 promulgated thereunder in connection with various public statements made by the Company regarding its Phase 2 clinical trial for RP-A501 for Danon disease. The actions seek unspecified damages, costs and expenses, including attorneys' fees. On September 9, 2025, the Court consolidated the two pending putative securities class action lawsuits, appointed two stockholders as co-lead plaintiffs, and approved their selection of co-lead counsel. Pursuant to a stipulation approved by the Court on September 22, 2025, the co-lead plaintiffs filed a consolidated amended complaint on November 18, 2025. The consolidated amended complaint, captioned in re Rocket Pharmaceuticals, Inc. Securities Litigation, 3:25-cv-10049, is brought on behalf of persons who purchased or otherwise acquired the Company's securities during the period of February 28, 2024 through August 25, 2025 (inclusive). Plaintiffs claim that the lawsuit arises from Defendants' public statements and purported omissions concerning Rocket's Phase 2 clinical trial for RP-A501 for DD. Among other things, Plaintiffs allege that Defendants failed to disclose certain SAEs that impacted patients during the Phase 2 clinical trial and the introduction of a C3 inhibitor to the trial's protocol and that Defendants purportedly lacked a viable trial design that could safely and effectively dose patients while managing the risk of serious adverse events. According to Plaintiffs, Rocket's stock price was inflated as a result of these purported misstatements and omissions. We intend to vigorously defend against the consolidated amended complaint's allegations. On January 30, 2026, the Company filed a motion to dismiss the consolidated amended complaint. The Plaintiffs response to the Company's motion was filed on April 1, 2026 and the Defendants reply was filed on May 15, 2026. Given the nature of the cases, including that the proceedings are in their early stages, the Company is unable to predict the ultimate outcome of the cases or estimate the range of potential loss, if any.

On October 22, 2025, a putative derivative action was filed in the United States District Court for the District of New Jersey, naming as defendants certain of the Company's officers and current or former directors. 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 those made in the previously filed putative securities class action complaints, with additional allegations regarding a purported lack of internal controls and alleged insider trading. The complaint seeks declaratory relief, an award of damages to the Company, an order directing the Company and the individual defendants to institute certain requested corporate governance reforms, restitution from the individual defendants, and costs and disbursements related to the lawsuit. The parties agreed to stay all proceedings in the putative derivative action until any motions to dismiss the putative securities class action are resolved, and on December 22, 2025, the court approved the parties' stipulation to that effect. The Company intends to vigorously defend the litigation. The Company will pay the legal fees related to the putative derivative action against the Company's officers and directors. Given the nature of the litigation, including the fact that it is in its early stages, the Company is unable to predict its ultimate outcome or estimate the range of potential loss, if any.

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

Indemnification Arrangements

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

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

15. Agreements Related to Intellectual Property

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

16. CIRM Grants

DD CIRM Grant

On August 18, 2024, CIRM awarded the Company up to $5.8 million under a CLIN2 grant award to support the clinical development of its AAV-based genetic medicines, RP-A501 for the treatment of DD. Proceeds from the grant would help fund clinical trial costs as well as manufactured drug product for Phase 1/2 patients. During the three and six months ended June 30, 2026, the Company did not receive any grants. During the three and six months ended June 30, 2025, the Company received grants of $0 and $2.7 million, respectively, which were recorded as a reduction of R&D expenses. Through June 30, 2026, the Company has received total RP-A501 grants of $5.0 million from CIRM. No additional milestones were met during the six months ended June 30, 2026.

17. Related Party Transactions

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 research and development activities. As compensation for services rendered during 2025, the consultant received $125,000 in cash and $125,000 of RSUs valued as of the closing price on March 3, 2025 which cliff vested on December 31, 2025. The agreement ended on December 31, 2025. The board member was paid approximately $50,000 for the period ended June 30, 2025 for services provided under the consulting agreement.

In February 2026, the Company entered into an agreement to receive services from a firm whose CEO and founder is the spouse of the same board member. As compensation for services being rendered during 2026, the firm received a total of $65,000.

In July 2026, the Company entered into a consulting agreement with one of the Company's board members for services related to the Company's strategic and financial activities. As compensation for services rendered, the consultant was granted 150,000 RSUs with a grant date of August 3, 2026. One-third of the RSUs shall vest on the first anniversary of the grant date and the remaining RSUs will vest in equal quarterly installments over the following two years. The agreement will terminate on August 3, 2029, unless terminated earlier by the Company for cause or voluntarily by consultant.

18. 401(k) Savings Plan

The Company has a defined contribution savings plan (the "Plan") under Section 401(k) of the Internal Revenue Code of 1986. This Plan covers substantially all employees who meet minimum age and service requirements and allows participants to defer a portion of their annual compensation on a pre-tax basis. Company contributions to the Plan may be made at the discretion of the Company's Board of Directors. The Company has elected the safe harbor match of 4% of employee contributions to the Plan, subject to certain limitations. The Company's matching contribution for the three and six months ended June 30, 2026, was $0.3 million and $0.7 million, respectively. The Company's matching contributions for the three and six months ended June 30, 2025, were $0.7 million and $1.2 million, respectively.

19. 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.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Revenue	$ -	$ -	$ -	$ -
Operating expenses:				
Research and development	29,497	42,658	60,951	78,600
Non-commercial general and administrative	13,665	17,042	27,766	37,657
Commercial general and administrative	3,760	7,978	6,716	15,809
Restructuring	-	3,471	-	3,471
Total operating expenses	46,922	71,149	95,433	135,537
Loss from operations	(46,922)	(71,149)	(95,433)	(135,537)
Gain on sale of PRV	178,190	-	178,190	-
Interest expense	(473)	(473)	(946)	(945)
Interest and other income, net	347	483	508	1,819
Accretion of discount on investments, net	693	2,220	1,922	4,410
Earnings (losses) before Income Taxes	131,835	(68,919)	84,241	(130,253)
Provision for Income Taxes	(8,621)	-	(8,621)	-
Net Segment income (loss) and Net income (loss)	$ 123,214	$ (68,919)	$ 75,620	$ (130,253)

The Company's CODM uses net loss to evaluate past spending and to guide decisions regarding future spending. Net income (loss) is used to monitor budget versus actual results. The CODM also uses net income (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.

20. 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 $3.5 million in June 2025, based on initial estimated restructuring costs related to severance and employee termination costs. This estimate was later reduced in the second half of 2025 to approximately $3.2 million of restructuring costs that were paid out over multiple months.

The following table summarizes the accrued liabilities activity in connection with the restructuring plan for the six months ended June 30, 2026 and 2025:

	Six Months Ended June 30, 2026	Six Months Ended June 30, 2025
Beginning balance	$ 46	$ -
Restructuring charges incurred during the period	-	3,471
Restructuring charges eliminated during the period	(46)	-
Ending balance	$ -	$ 3,471

21. Gain on Sale of Priority Review Voucher

On April 26, 2026, the Company entered into a definitive agreement to sell its PRV that was originally issued in connection with the FDA's approval of the BLA for KRESLADI™ for $180 million. The Company announced the closing of the transaction on June 12, 2026, and the Company recognized a net gain of $178.2 million related to the sale.

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

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

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

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

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

Business Highlights

During the second quarter of 2026, Rocket continued executing on its strategy to advance a focused portfolio of genetic medicines for inherited cardiovascular diseases, while progressing commercialization of its first approved product. The Company strengthened its balance sheet through the completed monetization of its Rare Pediatric Disease Priority Review Voucher (PRV), continued advancing its late-stage Danon disease program following resumption of dosing, progressed development across its cardiovascular pipeline, and continued commercial launch preparations for KRESLADI™.

Major developments during the quarter included:

- Completion of the $180 million sale of the Company's Rare Pediatric Disease Priority Review Voucher (PRV), providing substantial non-dilutive capital to support the Company's strategic priorities.
- Continued advancement of the RP-A501 Phase 2 pivotal Danon disease study following resolution of the FDA clinical hold and resumption of patient dosing.
- Continued execution across the Company's cardiovascular genetic medicines portfolio, including advancement of the RP-A501 (Danon disease), RP-A601 (PKP2-ACM) and RP-A701 (BAG3-DCM) programs.
- Continued commercial launch activities for KRESLADI™, including Qualified Treatment Center onboarding, manufacturing readiness, patient identification, reimbursement planning, and commercial infrastructure development.

The second quarter of 2026 reflected continued execution against the Company's strategic priorities. Management remained focused on advancing its lead cardiovascular genetic medicines programs, preparing for the commercial launch of KRESLADI™, strengthening the Company's financial position through completion of the PRV monetization transaction, and maintaining disciplined execution across clinical, regulatory, manufacturing, and commercial activities.

Overview

Rocket Pharmaceuticals is a fully integrated, commercial-stage biotechnology company advancing genetic medicines for rare and devastating diseases, with a strategic focus on inherited cardiovascular conditions. Our prioritized development portfolio includes AAV-based gene therapies targeting genetically defined cardiomyopathies, complemented by KRESLADI™, our first FDA-approved product, for the treatment of pediatric patients with severe LAD-I who have biallelic mutations in the *ITGB2* gene and do not have a suitable HLA-matched sibling donor. Our capabilities span clinical development, regulatory execution, manufacturing and commercialization, supported by in-house research and development expertise and AAV cGMP manufacturing infrastructure.

The Company's activities during the quarter reflect continued execution across our prioritized cardiovascular genetic medicines programs, alongside commercial readiness activities for KRESLADI™. Given the ultra-rare patient population and anticipated phased commercial rollout, the Company does not expect KRESLADI™ to generate material revenue in the near term.

We aim to develop and commercialize genetic medicines that address the underlying causes of rare and devastating diseases with significant unmet need. Our current development strategy is centered on inherited cardiovascular diseases, where our scientific, clinical, manufacturing and regulatory capabilities may support a portfolio of differentiated and potentially first- or best-in-class therapies.

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

In March 2026, KRESLADI™ (marnetegragene autotemcel) received accelerated approval from the FDA for the treatment of pediatric patients with severe LAD-I who have biallelic mutations in the *ITGB2* gene and do not have a suitable HLA-matched sibling donor. In connection with the approval, the Company was awarded a PRV and, in April 2026, entered into a definitive agreement to sell the PRV for $180 million. The transaction closed in June 2026. The Company intends to pursue a focused commercial strategy for KRESLADI™ that is appropriately scaled to the exceptionally small patient population affected by this ultra-rare disease.

Our strategy is built on several foundational pillars:

- **First-and-Best-in-Class Approach:** With our program selection, we apply a rigorous, disease-based selection approach to identify and prioritize programs: targeting complex genetic disorders with differentiated therapies that offer the potential to be first-, best-, or only-in-class, focusing on monogenic disease with on-target mechanisms of action to directly address the root cause of the disease to offer superior clinical profiles, and choosing indications with sizable market opportunities to enable broad patient impact and sustainable value creation.
- **Strategic Focus on Rare Cardiovascular Indications:** Our near-term research and development investments are focused on applying our AAV capabilities to genetically defined cardiovascular diseases. Collectively, our clinical cardiovascular genetic medicines programs address genetically defined forms of hypertrophic, arrhythmogenic and dilated cardiomyopathy, representing three major categories of inherited heart disease with significant unmet need.
- **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 portfolio of product candidates targeting monogenic cardiovascular diseases with substantial unmet need across stages of clinical development.
- Continuing to build and scale proprietary in-house analytics, process development, and manufacturing capabilities to support clinical and commercial supply.
- Evaluating potential strategic partnerships or other transactions for certain non-core programs to enable continued development, regulatory approval, and commercialization.

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

- Submit BLAs for certain of our clinical programs.
- Evaluate opportunities to expand our cardiovascular genetic medicines portfolio into additional genetically defined indications that are compatible with our AAV capabilities and core strategy.
- Pursue potential eligibility for FDA priority review voucher programs.

Genetic Medicines Overview

Genetic medicines are 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. Genetic medicines aim 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.

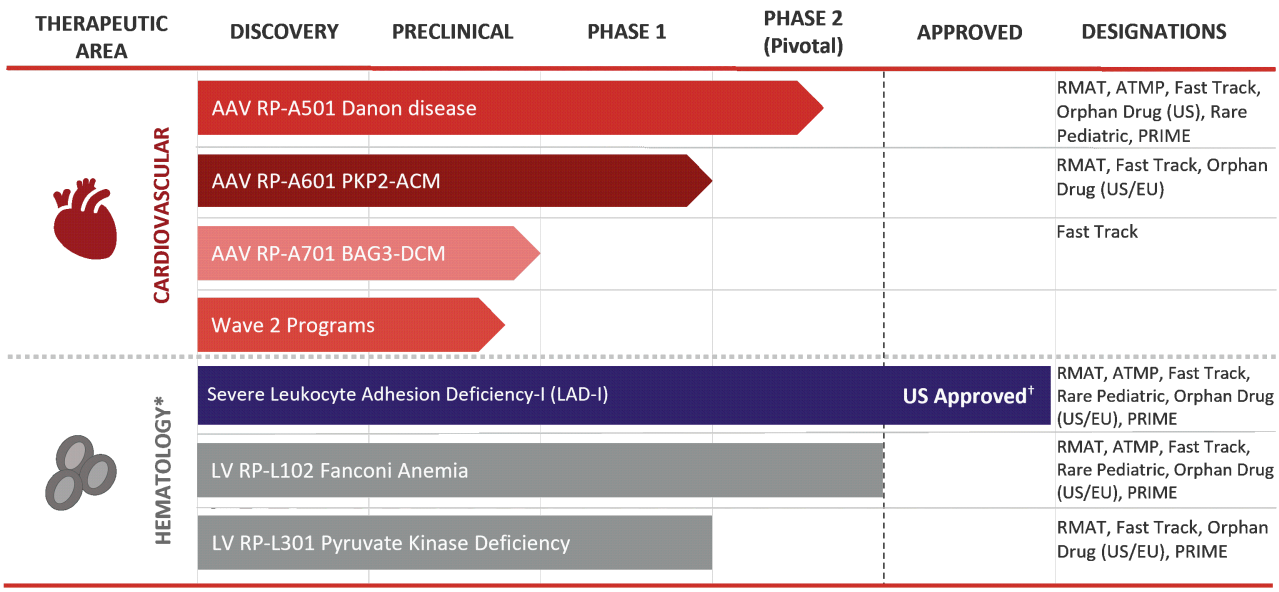
We are developing genetic medicine product candidates utilizing modified, non-pathogenic viruses as delivery vehicles. Viruses are inherently effective for gene delivery due to their natural ability to enter cells and deliver genetic material. In engineering our viral vectors, the native viral genes 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 genetic medicine programs using two primary vector approaches: adeno-associated virus (AAV) vectors and lentiviral (LV) vectors. We believe our AAV- and LV-based programs have the potential to provide meaningful and durable therapeutic benefit by addressing the underlying genetic cause of disease. Our genetic medicine product candidates are administered either (1) in vivo, in which an AAV vector is delivered directly to the patient, either systemically or through targeted tissue delivery, to enable in situ transduction of the desired cell populations, or (2) ex vivo, in which a patient's hematopoietic stem cells (HSCs) are collected, genetically modified with an LV vector in a controlled laboratory environment, and then reinfused into the patient.

We believe that scientific advances, clinical progress, and the greater regulatory acceptance of genetic medicines have created a promising environment to advance genetic medicine 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 genetic medicines in recent years indicates that there is a regulatory pathway forward for genetic medicine products.

Pipeline Overview

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



*Rocket prioritized approval of the severe LAD-I program within its LV portfolio; external partnership opportunities for RP-L102 and RP-L301 are under evaluation.
†Accelerated Approval (AA) granted by FDA on March 26, 2026, for severe LAD-I.

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

Cardiovascular Programs

Danon disease

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

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

As previously announced, a patient receiving therapy in 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 ventricular ejection fraction (32%) on echocardiogram and markedly elevated left ventricle filling pressure prior to treatment. The patient's clinical course was characteristic of DD progression. The patient is doing well post-transplant.

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

In November 2024, we announced positive results and presented long-term safety and efficacy results of the Phase 1 study at the American Heart Association's 2024 Late-Breaking Science sessions and simultaneously published these data in the *New England Journal of Medicine*. The long-term safety and efficacy results from the Phase 1 RP-A501 study showed that RP-A501 was generally well tolerated and all evaluable DD patients demonstrated LAMP2 protein expression at 12 months (sustained up to 60 months) and reduction of left ventricular mass index by ≥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 genetic medicine 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

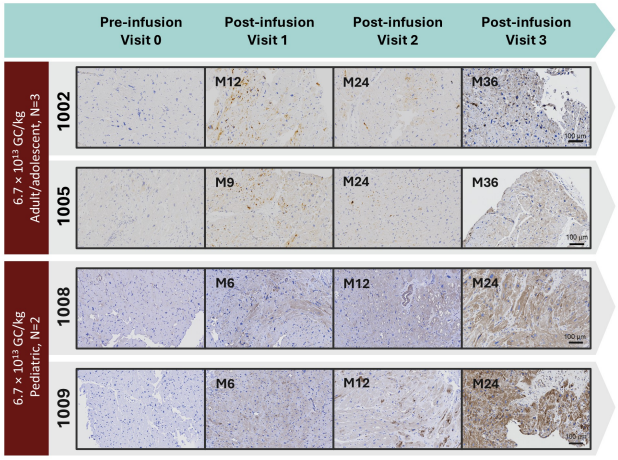
Cohort	Patient	BL	M6	M12	M18	M24	M30	M36	M60‡
6.7 × 10^{13} GC/kg Adult/adolescent	1001	0	++	+	NP	NP	0*	0*	++ + ‡
	1002	0	NP	++ +	++	++	++	++	
	1005	0	NP	++ †	NP	+	+	+	
1.1 × 10^{14} GC/kg Adult/adolescent[a]	1006	0	+	+	+	+	NP	++ +	
6.7 × 10^{13} GC/kg Pediatric	1008	0	++	+	NP	++	Visits Pending		
	1009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

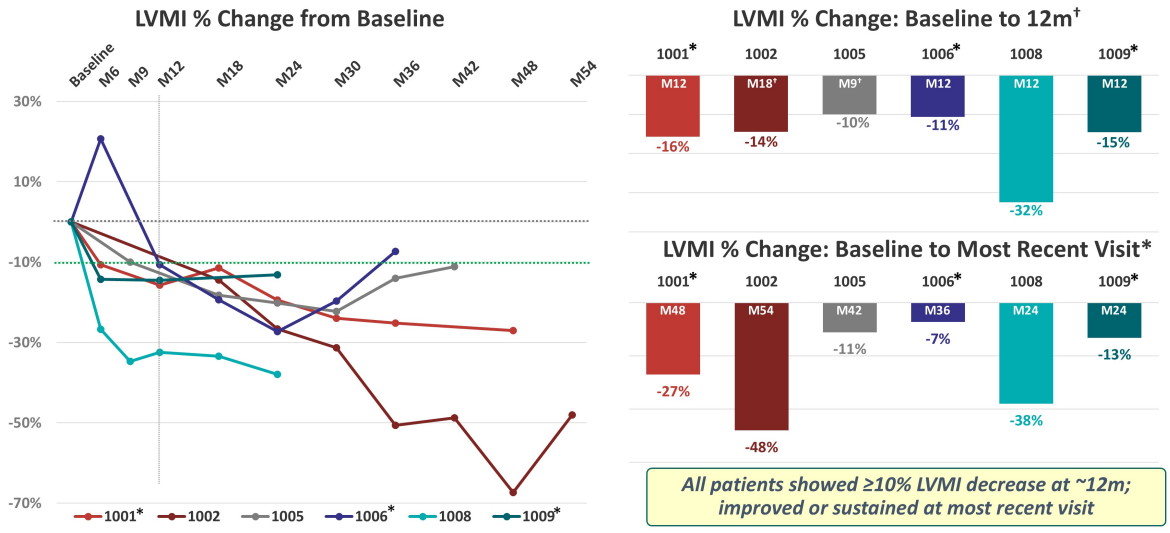
* Centrally evaluated (blinded) MRI data were utilized for LVMI when available. All other measurements of cardiac structure and function reflect centrally evaluated (blinded) echocardiogram data.
† Central laboratory assessment of cTnI were performed on cryopreserved and non-cryopreserved samples. Values 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.

Collectively, these findings continue to support evidence of biologic activity, favorable long-term durability, and clinically meaningful improvements across multiple measures of disease. 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 ventricular ejection fraction (LVEF);
- Reduction or stabilization of cardiac biomarkers (median cardiac troponin I [cTnI] and BNP reductions of 84% and 57%, respectively);
- Improvement in NYHA class from Class II at baseline to Class I at most recent follow-up visit;
- Improvements in KCCQ-12 scores (median improvement of 27 points) that persisted up to 54 months of follow-up; and
- Preliminary long-term follow-up assessments for Patient 1001 were positive for immunohistochemical staining and appear to show Grade 3 expression in the heart at the five-year timepoint.

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



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. A global natural history study is also running concurrently with the Phase 2 pivotal trial.

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

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

Drug product for the Phase 2 study is being produced in-house at our GMP manufacturing facility in Cranbury, New Jersey. We have successfully produced multiple Danon AAV cGMP batches at this facility since 2022.

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

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

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

Prior to the clinical hold, six patients with Danon disease were treated with RP-A501 in the Phase 2 study. Following resumption of dosing under the modified protocol, the initial three patients treated under the modified protocol received RP-A501 sequentially at the recalibrated dose of 3.8×10^{13} GC/kg together with a refined immunomodulatory regimen. As of August 3, 2026, no thrombotic microangiopathy, capillary leak syndrome or other significant safety concerns had been observed in these patients. The Company is actively engaging with the FDA to align on the path to dosing additional patients and completing the pivotal Phase 2 trial and expects to provide an update on the regulatory pathway in the second half of 2026. The Company also remains on track to provide a comprehensive Danon disease program update in the second half of 2026.

Plakophilin-2 Arrhythmogenic Cardiomyopathy

Plakophilin-2 related arrhythmogenic cardiomyopathy, otherwise known as PKP2-ACM, is an inherited cardiac disorder caused by pathogenic variants in the *PKP2* gene and characterized by life-threatening ventricular arrhythmias, cardiac structural abnormalities, and sudden cardiac death. Most commonly, the cardiomyopathy initially manifests in the right ventricular free wall, so the disease was originally termed arrhythmogenic right ventricular dysplasia/cardiomyopathy or ARVD/C. However, since left dominant and biventricular forms have also been observed, this has led more recently to the use of the term ACM. Mutations in the *PKP2* gene comprise the most frequent genetically identified etiology of familial ACM. Patients with mutations in *PKP2* are typically heterozygous and demonstrate reduced expression of the PKP2 protein in the myocardium. *PKP2* encodes for the protein Plakophilin-2, which is a component of the desmosome, an intercellular complex involved in cell-cell adhesion. The PKP2 protein is also involved in transcriptional regulation of calcium signaling between cardiomyocytes. PKP2-ACM is most commonly diagnosed in young adults, with a mean age at presentation of 35 years. Patients have a very high lifetime risk of life-threatening ventricular arrhythmias, with annual event rates of approximately 5% to 10% and rates as high as 10% to 20% among higher-risk patients (e.g., those with ICDs).

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

RP-A601 is our investigational genetic medicine 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 designations in the US and Orphan Drug designations in both the U.S. and EU.

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

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

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

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 intercalated disc proteins between 3 and 12 months after treatment. In addition, preliminary observations suggest potential improvement or stabilization in arrhythmia burden, cardiac function, and quality of life, although these findings are based on a limited number of patients and require further evaluation. Based on available data to date, we have selected 8 x 10^{13} GC/kg as the dose to be further evaluated in subsequent clinical development, and we do not currently plan to evaluate higher dose levels in this study. Collectively, these findings continue to support the Company's disease-modifying approach for genetically defined arrhythmogenic cardiomyopathy and further inform ongoing development of the program.

BAG3 Dilated Cardiomyopathy

Dilated cardiomyopathy is the most common form of cardiomyopathy and is characterized by enlargement of the heart chambers and progressive impairment of cardiac function. Pathogenic variants in the *BAG3* gene are among the more common genetic causes of familial DCM and are associated with early-onset, progressive heart failure, significant morbidity and mortality. The prevalence of BAG3-associated DCM in the United States is estimated to be as many as 30,000 individuals.

There are currently no approved therapies specifically indicated for the treatment of BAG3-associated DCM. Current medical management follows guideline-directed therapy for heart failure with reduced ejection fraction and may include pharmacologic therapy, implantable cardiac devices, catheter ablation and, in advanced cases, heart transplantation. Although heart transplantation may be lifesaving, it is not curative and is associated with substantial morbidity and mortality.

RP-A701 is our investigational AAVrh.74-based genetic medicine for the treatment of BAG3-associated DCM. RP-A701 is designed to deliver a functional BAG3 gene to cardiomyocytes with the goal of restoring BAG3 protein expression and addressing the underlying genetic cause of disease.

Previously completed nonclinical efficacy studies in a BAG3 knockout mouse model, in which treatment was initiated following disease onset, demonstrated dose-dependent improvements in cardiac systolic function and reduction in left ventricular dimensions relative to control-treated animals. The studies also demonstrated dose-dependent expression of human BAG3 protein in cardiac tissue, increased expression of HSPB8, a key BAG3 co-chaperone protein, and reductions in the profibrotic marker Col1a1, consistent with restoration of BAG3 pathway biology. In separate rodent and non-human primate studies, AAVrh.74-BAG3 demonstrated a favorable nonclinical safety profile. In non-human primates, findings were consistent with known AAV class effects, including transient elevations in AST and ALT, hepatocyte necrosis in high-dose male animals and non-adverse dorsal root ganglion histopathologic findings, with no treatment-related adverse effects observed on cardiac function or other standard safety assessments. Collectively, these nonclinical studies supported advancement of RP-A701 into clinical development.

RP-A701 Restores Cardiac Function Dose-Dependently in a BAG3-KO Mouse Model[1]

Restoration of Cardiac Protein Expression

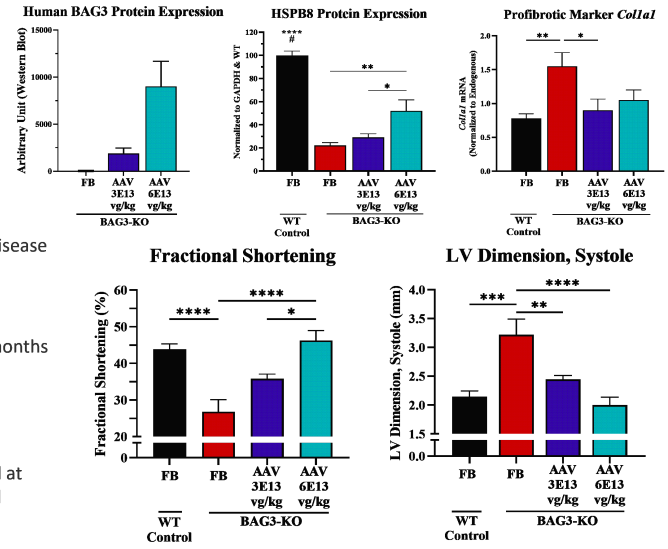
- AAVrh.74-BAG3 produced dose-dependent changes in cardiac protein expression, beyond the BAG3 protein including:
  - Dose-related increase in myocardial HSPB8, a key BAG3 co-chaperone
  - Robust reduction in myocardial expression of the profibrotic marker *Col1a1*

Dose-Dependent Improvement of Cardiac Function

- AAVrh.74-BAG3 Efficacy Study in BAG3 Knockout Mouse Model Post-disease Onset:
  - Dose-related 33–72% improvement in cardiac systolic function (fractional shortening) at 4 months post-infusion
  - Dose-related 23–37% reduction in LV end-systolic dimension at 4 months post-infusion

Favorable Safety Profile

- AAVrh.74-BAG3 Nonclinical Safety Profile:
  - Well tolerated, with no treatment-related adverse effects observed at efficacious doses in WT and BAG3-KO mouse models or at a ≥3-fold higher dose (1.8E14 vg/kg) in NHP studies



RP-A701 (AAVrh.74-BAG3) is an AAVrh.74-based gene therapy encoding human BAG3 under the control of a cardiac-selective promoter. All nonclinical studies shown used a toxicology lot manufactured using a process representative of the clinical manufacturing process.
[1]Rocket Pharmaceuticals internal nonclinical studies; data on file.
FB, Formulation Buffer; LV, left ventricle; WT, Wild Type

In June 2025, we received FDA clearance of our IND application for RP-A701. In July 2025, the FDA granted Fast Track designation to RP-A701 for the treatment of BAG3-associated DCM. Our ongoing Phase 1 clinical trial is a multicenter, dose-escalation study designed to evaluate the safety, biological activity and preliminary efficacy of RP-A701 in adults with BAG3-associated DCM. We continue to advance RP-A701 as part of our strategy to build a differentiated pipeline of genetic medicines targeting inherited cardiovascular diseases.

Hematology Programs

Leukocyte Adhesion Deficiency-I

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

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

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

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

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

Following approval, the Company has focused commercial efforts on establishing Qualified Treatment Centers, manufacturing readiness, including commercial manufacturing and supply chain preparedness, patient identification, reimbursement, and other launch activities in preparation for commercial patient treatment.

On April 26, 2026, the Company entered into a definitive agreement to sell its PRV that was originally issued in connection with the FDA's approval of the BLA for KRESLADI™ for $180 million. The transaction was subject to customary closing conditions, including the expiration or termination of the applicable waiting period under the Hart-Scott-Rodino Antitrust Improvements Act of 1976 and the Company announced the closing of the transaction on June 12, 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. The prevalence of FA in the U.S. and the EU is estimated to be approximately 5,500 to 7,000 patients.

Although improvements in allogeneic (donor-mediated) HSCT, currently the most frequently utilized therapy for FA, have resulted in frequent hematologic correction of the disorder, HSCT is associated with both acute and long-term risks, including transplant-related mortality, graft failure, and graft versus host disease, a sometimes fatal side effect of allogeneic transplant characterized by painful ulcers in the GI tract, liver toxicity and skin rashes, as well as increased risk of subsequent cancers. Our genetic medicine 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 genetic medicine 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 genetic medicine for the treatment of FA. RP-L102's LV carries the *FANCA* gene as part of the PGK-FANCA-WPRE expression cassette which includes a phosphoglycerate kinase (PGK) promoter and an optimized woodchuck hepatitis virus post transcriptional regulatory element (WPRE). The Phase 2 study of RP-L102 for the treatment of FA type A without the use of myeloablative conditioning treated a total of 14 patients from the U.S. and EU. Patients received a single intravenous infusion of RP-L102 that utilizes fresh cells and an improved process which incorporates a modified stem cell enrichment process, transduction enhancers, as well as commercial-grade vector and final drug product. The Company holds FDA RMAT, Rare Pediatric, and Fast Track designations in the U.S., PRIME and ATMP designations in the EU, and Orphan Drug designations in both regions for the program.

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

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

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

Pyruvate Kinase Deficiency

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

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

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

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

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

Future Opportunities

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

cGMP Manufacturing

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

Financial Overview

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

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

Operating expenses during the quarter continued to reflect investment in the Company's lead cardiovascular genetic medicine programs and commercial launch activities for KRESLADI™, partially offset by the benefits of the Company's previously announced portfolio prioritization and organizational restructuring.

From inception through June 30, 2026, we have raised net cash proceeds of approximately $1.2 billion from investors through equity and convertible debt financings to fund our operations.

In April 2026, the Company entered into an agreement to sell its PRV for $180 million, which closed in June 2026, providing non-dilutive capital to support advancement of its cardiovascular genetic medicines pipeline.

Revenue

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

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

Research and Development Expenses

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

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

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

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

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

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

The following table presents R&D expenses tracked on a program-by-program basis as well as by type and nature of expense for the three and six months ended June 30, 2026 and 2025:

	Three Months Ended June 30,		Six Months Ended June 30,	
	2026	2025	2026	2025
Direct Expenses:				
Danon Disease (AAV) RP-A501	$ 5,362	$ 6,455	$ 10,434	$ 7,341
Plakophilin-2 Arrhythmogenic Cardiomyopathy (AAV) RP-A601	1,977	2,324	3,687	4,135
Leukocyte Adhesion Deficiency (LV) RP-L201	1,107	3,327	2,791	7,330
Fanconi Anemia (LV) RP-L102	951	5,276	2,323	11,296
Pyruvate Kinase Deficiency (LV) RP-L301	173	794	590	1,884
BAG3-DCM (AAV) RP-A701	2,162	707	3,906	1,212
Other product candidates	208	447	815	288
Total direct expenses	11,940	19,330	24,546	33,486
Unallocated Expenses:				
Employee compensation	10,208	12,493	20,532	24,732
Stock-based compensation expense	3,492	4,821	7,799	9,209
Depreciation and amortization expense	1,274	1,614	2,557	3,478
Laboratory and related expenses	480	1,905	1,212	2,819
Professional fees	1,138	1,529	2,281	2,589
Other expenses	965	966	2,024	2,287
Total other research and development expenses	17,557	23,328	36,405	45,114
Total research and development expense	$ 29,497	$ 42,658	$ 60,951	$ 78,600

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

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

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

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

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

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

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

General and Administrative Expenses

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

Restructuring Expense

In June 2025, the Company's Board of Directors approved a restructuring plan to reduce the Company's workforce and incurred aggregate charges of approximately $3.5 million in restructuring expenses, consisting of employee severance payments and other termination benefits.

Interest Expense

Interest expense for the three and six months ended June 30, 2026 and 2025 was related to our financing lease obligation for our Cranbury, NJ facility.

Interest and Other Income

Interest and other income for the three and six months ended June 30, 2026 and 2025 was related to interest earned from investments and cash equivalents.

Income Taxes

Income tax expense for the three and six months ended June 30, 2026 was $8.6 million in each period, compared with no income tax expense in the corresponding 2025 periods. The net income tax expense of $8.6 million recognized for the six months ended June 30, 2026 primarily reflects the expected tax associated with the gain on the sale of the Company's PRV, including the expected utilization of current-year and certain historical tax attributes.

Critical Accounting Policies and Significant Judgments and Estimates

Income Taxes

Income taxes are accounted for under the asset and liability method. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases and operating loss and tax credit carryforwards. A valuation allowance is required when, based on the weight of the available evidence, it is more likely than not that some portion or all of the deferred tax assets will not be realized. Judgment is required to determine whether certain income tax positions are more likely than not to be sustained. These judgments may change from period to period as facts and circumstances change. We make estimates and judgments about our future taxable income that are based on assumptions that are consistent with our plans and estimates. Should the actual amounts differ from the estimates, the amount of our valuation allowance could be materially impacted. Changes in these estimates may result in significant increases or decreases to our tax provision in a period in which such estimates are changed, which would affect net income or loss.

We consider future taxable income and our historical performance in assessing the need for a valuation allowance. We periodically reassess the need for a valuation allowance and if we expect to realize deferred tax assets for which we have previously recorded a valuation allowance, we will reduce the valuation allowance in the period in which such determination is first made.

Due to the Company's lack of earnings history, we determined that a full valuation allowance was required to offset the net deferred tax assets, (exclusive of the net deferred tax liabilities related to indefinite lived intangibles), at December 31, 2025. In assessing the need for a valuation allowance as of June 30, 2026, management considered the gain on the sale of the PRV as a significant source of positive evidence supporting future taxable income. Because the sale agreement was executed on April 26, 2026, the anticipated gain was incorporated into the valuation allowance analysis. As a result, the Company recognized an income tax benefit related to the expected utilization of current-year losses and the release of valuation allowance associated with certain historical losses. This benefit was substantially offset by the corresponding income tax expense associated with the anticipated gain on the PRV sale.

There have been no other material changes in our critical accounting policies and estimates in the preparation of our consolidated financial statements during the six months ended June 30, 2026 compared to those disclosed in our 2025 Form 10-K.

Results of Operations

Comparison of the Three Months Ended June 30, 2026 and 2025

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

	Three Months Ended June 30,		
	2026	2025	Change
Operating expenses:			
Research and development	$ 29,497	$ 42,658	$ (13,161)
General and administrative	17,425	25,020	(7,595)
Restructuring	-	3,471	(3,471)
Total operating expenses	46,922	71,149	(24,227)
Loss from operations	(46,922)	(71,149)	24,227
Gain from sale of PRV	178,190	-	178,190
Interest expense	(473)	(473)	-
Interest and other income, net	347	483	(136)
Accretion of discount on investments, net	693	2,220	(1,527)
Total other income, net	178,757	2,230	176,527
Earnings (losses) before Income Taxes	131,835	(68,919)	200,754
Provision for Income Taxes	(8,621)	-	(8,621)
Net income (loss)	$ 123,214	$ (68,919)	$ 192,133

Research and Development Expenses

R&D expenses decreased $13.2 million to $29.5 million for the three months ended June 30, 2026, compared to the three months ended June 30, 2025. The decrease in R&D expenses was primarily driven by decreases in manufacturing and development and direct material costs of $7.5 million, stock-based and other compensation and benefits expense of $3.6 million due to decreased R&D headcount, depreciation expenses of $1.0 million due to decreased asset base, and clinical trial expenses of $1.0 million.

General and Administrative Expenses

G&A expenses decreased $7.6 million to $17.4 million for the three months ended June 30, 2026, compared to the three months ended June 30, 2025. The decrease in G&A expenses was primarily driven by decreases in commercial preparation related expenses of $4.8 million due to lower headcount and lower spending on commercial launch, stock-based and other compensation and benefit expenses of $1.9 million due to decreased G&A headcount, and legal expenses of $1.4 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 $3.5 million in restructuring expenses, consisting of employee severance payments and other termination benefits.

Other Income, Net

Other income increased $176.5 million to $178.8 million for the three months ended June 30, 2026, compared to the three months ended June 30, 2025. The increase in other income was primarily driven by the sale of the PRV for net $178.2 million. The increase was partially offset by a decline in accretion of discount on investments, net, of $1.5 million due to lower investment balance and interest rates year over year.

Comparison of the Six Months Ended June 30, 2026 and 2025

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

	Six Months Ended June 30,		
	2026	**2025**	**Change**
Operating expenses:			
Research and development	$ 60,951	$ 78,600	$ (17,649)
General and administrative	34,482	53,466	(18,984)
Restructuring	-	3,471	(3,471)
Total operating expenses	95,433	135,537	(40,104)
Loss from operations	(95,433)	(135,537)	40,104
Gain from sale of PRV	178,190	-	178,190
Interest expense	(946)	(945)	(1)
Interest and other income, net	508	1,819	(1,311)
Accretion of discount on investments, net	1,922	4,410	(2,488)
Total other income, net	179,674	5,284	174,390
Earnings (losses) before Income Taxes	84,241	(130,253)	214,494
Provision for Income Taxes	(8,621)	-	(8,621)
Net income (loss)	$ 75,620	$ (130,253)	$ 205,873

Research and Development Expenses

R&D expenses decreased $17.6 million to $61.0 million for the six months ended June 30, 2026, compared to the six months ended June 30, 2025. The decrease in R&D expenses was primarily driven by decreases in manufacturing and development and direct material costs of $13.3 million, stock-based and other compensation and benefits expense of $5.6 million due to decreased R&D headcount, and depreciation expenses of $1.4 million due to decreased asset base. The decrease was partially offset by increases in clinical trial expenses of $1.8 million and consulting expenses of $1.2 million.

General and Administrative Expenses

G&A expenses decreased $19.0 million to $34.5 million for the six months ended June 30, 2026, compared to the six months ended June 30, 2025. The decrease in G&A expenses was primarily driven by decreases in commercial preparation related expenses of $8.5 million due to lower headcount and lower spending on commercial launch, legal expenses of $7.0 million as a result of litigation settlement in 2025, and stock-based and other compensation and benefit expenses of $5.6 million due to decreased G&A headcount. The decrease was partially offset by milestone expenses upon approval of KRESLADI™ of $2.4 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 $3.5 million in restructuring expenses, consisting of employee severance payments and other termination benefits.

Other Income, Net

Other income increased $174.4 million to $179.7 million for the six months ended June 30, 2026, compared to the six months ended June 30, 2025. The increase in other income was primarily driven by the sale of the PRV for net $178.2 million. The increase was partially offset by decreases in interest and other income, net, of $1.6 million and accretion of discount on investments, net, of $2.5 million due to lower investment balance and interest rates year over year.

Liquidity and Capital Resources

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

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

Our consolidated financial statements have been prepared on the basis of continuity of operations, realization of assets and the satisfaction of liabilities in the ordinary course of business. Rocket has historically incurred recurring net losses and negative cash flows from operations. Although the Company recognized net income for the six months ended June 30, 2026, primarily due to the gain recognized on the sale of the PRV, the Company expects to incur operating losses for the foreseeable future as it advances its development and commercialization activities. Rocket has incurred net losses and negative cash flows from its operations each year since inception. We had a net income of $75.6 million for the six months ended June 30, 2026, and a net loss of $223.1 million for the year ended December 31, 2025. We have experienced negative cash flows from operations and as of June 30, 2026 and December 31, 2025, we had an accumulated deficit of $1.37 billion and $1.44 billion, respectively. As of June 30, 2026, we had $283.7 million of cash, cash equivalents and investments. In April 2026, we entered into an agreement to sell our PRV for $180 million, which closed in June 2026, providing non-dilutive capital to support advancement of the Company's cardiovascular genetic medicines pipeline and extend its operational runway. We believe that, based on our current operating plan, our existing cash, cash equivalents, and investments, will be sufficient to fund our operating expenses and capital expenditure requirements into the second quarter of 2028. Since inception, we have financed our operations primarily through the sale of equity securities and continue to manage our capital resources in a disciplined manner with a focus on operational execution, strategic prioritization, and long-term sustainability.

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

Cash Flows

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

	Six Months Ended June 30,	
	2026	2025
Net cash used in operating activities	$ (85,007)	$ (104,754)
Net cash provided by (used in) investing activities	231,808	(26,274)
Net cash provided by financing activities	149	215
Net increase (decrease) in cash, cash equivalents and restricted cash	$ 146,950	$ (130,813)

Operating Activities

During the six months ended June 30, 2026, operating activities used $85.0 million of cash and cash equivalents, primarily resulting from reduction of our net income of $75.6 million by the gain on sale of PRV of $178.2 million and changes in operating assets and liabilities of, net, $0.9 million. These were reduced by net non-cash charges of $18.5 million, including non-cash stock-based compensation expense of $15.9 million, depreciation and amortization expense of $4.2 million, partially offset by accretion of discount on investments of $1.7 million. Changes in our operating assets and liabilities for the six months ended June 30, 2026 included a increase in accounts payable and accrued expenses of $1.1 million and an increase in our prepaid expenses of $2.0 million.

During the six months ended June 30, 2025, operating activities used $104.8 million of cash and cash equivalents, primarily resulting from our net loss of $130.3 million offset by net non-cash charges of $22.5 million, including non-cash stock-based compensation expense of $21.2 million, depreciation and amortization expense of $5.6 million, partially offset by accretion of discount on investments of $4.3 million. Changes in our operating assets and liabilities for the six months ended June 30, 2025, included an increase in accounts payable and accrued expenses of $2.9 million and a decrease in our prepaid expenses of $0.2 million.

Investing Activities

During the six months ended June 30, 2026, net cash provided by investing activities was $231.8 million, primarily resulting from net proceeds of $178.2 million from the sale of the PRV, $117.3 million from the maturities of investments, offset by purchases of investments of $63.5 million, and purchases of property and equipment of $0.1 million.

During the six months ended June 30, 2025, net cash used by investing activities was $26.3 million, primarily resulting from proceeds of $166.8 million from the maturities of investments, offset by purchases of investments of $192.6 million, and purchases of property and equipment of $0.4 million.

Financing Activities

During the six months ended June 30, 2026, financing activities provided $0.1 million of cash consisting of proceeds from issuance of common stock from the exercise of stock options, partially offset by pay down of finance lease obligations.

During the six months ended June 30, 2025, financing activities provided $0.2 million of cash, consisting of return of short-swing profits.

Contractual Obligations and Commitments

Information regarding contractual obligations and commitments may be found in Note 14 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 June 30, 2026 and December 31, 2025, we had cash, cash equivalents and investments of $283.7 million and $188.9 million, respectively. The Company's investments are primarily in U.S. Treasury Securities. Our primary exposure to market risk is interest rate sensitivity, which is affected by changes in U.S. interest rates and our investments that can decline in value if market interest rates increase. We do not utilize interest rate hedging agreements or other interest rate derivative instruments.

If market interest rates were to increase immediately and uniformly by 100 basis points, or one percentage point, from levels at June 30, 2026, the net effect on the net fair value of our investments would have resulted in a hypothetical decline of $0.1 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 officer 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 June 30, 2026, our principal executive officer and principal financial officer concluded that our disclosure controls and procedures as of such date were effective at the reasonable assurance level. The term "disclosure controls and procedures," as defined in Rules 13a-15(e) and 15d-15(e) under the Exchange Act, means controls and other procedures of a company that are designed to ensure that information required to be disclosed by a company in the reports that it files or submits under the Exchange Act is recorded, processed, summarized, and reported within the time periods specified in the SEC's rules and forms.

Disclosure controls and procedures include, without limitation, controls and procedures designed to ensure that information required to be disclosed by us in the reports we file or submit under the Exchange Act is accumulated and communicated to our management, including our principal executive officer and principal financial officer, as appropriate to allow timely decisions regarding required disclosure. Management recognizes that any controls and procedures, no matter how well designed and operated, can provide only reasonable assurance of achieving their objectives and our management necessarily applies its judgment in evaluating the cost-benefit relationship of possible controls and procedures.

Inherent Limitations of Internal Controls

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

Changes in Internal Control over Financial Reporting

There were no changes in our internal control over financial reporting during the period covered by this report that have materially affected, or are reasonably likely to materially affect, our internal control over financial reporting.

PART II – OTHER INFORMATION

Item 1. Legal Proceedings

On June 11, 2025 and July 18, 2025, two stockholders filed putative securities class action lawsuits against us and certain of our executive officers in the United States District Court for the District of New Jersey, purportedly on behalf of classes of the Company's investors who purchased or otherwise acquired the Company's common stock between February 27, 2025 and May 26, 2025 (Ho v. Rocket Pharmaceuticals, Inc., and Gaurav Shah, Case No. 3:25-cv-10049) and between September 17, 2024 and May 26, 2025 (Yankov v. Rocket Pharmaceuticals, Inc., Gaurav Shah and Aaron Ondrey, 3:25-cv-13532), respectively. The complaints allege violations of Sections 10(b) and 20(a) of the Securities Exchange Act of 1934 and Rule 10b-5 promulgated thereunder in connection with various public statements made by the Company regarding its Phase 2 clinical trial for RP-A501 for Danon disease. The actions seek unspecified damages, costs and expenses, including attorneys' fees. On September 9, 2025, the Court consolidated the two pending putative securities class action lawsuits, appointed two stockholders as co-lead plaintiffs, and approved their selection of co-lead counsel. Pursuant to a stipulation approved by the Court on September 22, 2025, the co-lead plaintiffs filed a consolidated amended complaint on November 18, 2025. The consolidated amended complaint, captioned In re Rocket Pharmaceuticals, Inc. Securities Litigation, 3:25-cv-10049, is brought on behalf of persons who purchased or otherwise acquired the Company's securities during the period of February 28, 2024 through August 25, 2025 (inclusive). Plaintiffs claim that the lawsuit arises from Defendants' public statements and purported omissions concerning Rocket's Phase 2 clinical trial for RP-A501 for DD. Among other things, Plaintiffs allege that Defendants failed to disclose certain SAEs that impacted patients during the Phase 2 clinical trial and the introduction of a C3 inhibitor to the trial's protocol and that Defendants purportedly lacked a viable trial design that could safely and effectively dose patients while managing the risk of serious adverse events. According to Plaintiffs, Rocket's stock price was inflated as a result of these purported misstatements and omissions. We intend to vigorously defend against the consolidated amended complaint's allegations. On January 30, 2026, the Company filed a motion to dismiss the consolidated amended complaint. The Plaintiffs response to the Company's motion was filed on April 1, 2026 and the Defendants' reply was filed on May 15, 2026. Given the nature of the cases, including that the proceedings are in their early stages, the Company is unable to predict the ultimate outcome of the cases or estimate the range of potential loss, if any.

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

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

Item 1A. Risk Factors

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

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

None.

Item 3. Defaults Upon Senior Securities

None.

Item 4. Mine Safety Disclosures

Not applicable.

Item 5. Other Information

During the three months ended June 30, 2026, none of our directors or officers adopted or terminated a "Rule 10b5-1 trading arrangement" or "non-Rule 10b5-1 trading arrangement," as each term is defined in Item 408(a) of Regulation S-K.

Item 6. Exhibits

Exhibit Number	Description of Exhibit
2.1	Agreement and Plan of Merger and Reorganization, dated as of September 12, 2017, by and among Inotek Pharmaceuticals Corporation, Rocket Pharmaceuticals, Ltd., and Rome Merger Sub (incorporated by reference to Exhibit 2.1 to the Company's Current Report on Form 8- K (001-36829), filed with the SEC on September 13, 2017)
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, 2018
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†*	Priority Review Voucher Asset Purchase Agreement, dated as of April 26, 2026, by and between the Company and the Buyer.
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
† Portions of this exhibit have been omitted pursuant to Item 601(b)(10)(iv) of Regulation S-K.

SIGNATURES

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

ROCKET PHARMACEUTICALS, INC.

August 10, 2026

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

August 10, 2026

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

Exhibit 10.1

ASSET PURCHASE AGREEMENT

by and between

[***]

-and-

ROCKET PHARMACEUTICALS, INC.

Dated as of April 26, 2026

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Schedule 4.11 – Document Disclosure

ASSET PURCHASE AGREEMENT

This ASSET PURCHASE AGREEMENT (this "***Agreement***") is made and entered into as of April 26, 2026 (the "***Effective Date***"), by and between [***], a limited company organized under the laws of [***] ("***Buyer***"), and Rocket Pharmaceuticals, Inc., a corporation organized under the laws of Delaware ("***Seller***"). Buyer and Seller may hereinafter be referred to individually as a "***Party***" and collectively as the "***Parties***".

RECITALS

WHEREAS, Seller is the holder of all right, title and interest in and to the Priority Review Voucher (as defined below);

WHEREAS, Seller and Buyer each (i) desire that Buyer purchase from Seller, and Seller sell, transfer and assign to Buyer, the Purchased Assets (as defined below), all on the terms set forth herein (such transaction, the "***Asset Purchase***") and (ii) in furtherance thereof, have duly authorized, approved and executed this Agreement and the other transactions contemplated by this Agreement in accordance with all applicable Legal Requirements (as defined below); and

WHEREAS, Seller and Buyer desire to make certain representations, warranties, covenants and other agreements in connection with the Asset Purchase as set forth herein.

NOW, THEREFORE, in consideration of the foregoing and their mutual undertakings hereinafter set forth, and intending to be legally bound, the Parties agree as follows:

ARTICLE I.
DEFINITIONS

Section 1.01 Certain Definitions. As used in this Agreement, the following terms shall have the meanings indicated below:

(a) "***Affiliate***" means with respect to any Person, any other Person which, directly or indirectly through one or more intermediaries, controls, is controlled by or is under common control with such first Person, for so long as such control exists, whether such Person is or becomes an Affiliate on or after the Effective Date. A Person shall be deemed to "control" another Person if it: (i) with respect to such other Person that is a corporation, owns, directly or indirectly, beneficially or legally, at least fifty percent (50%) or more of the outstanding voting securities or capital stock (or such lesser percentage which is the maximum allowed to be owned by such Person in a particular jurisdiction) of such other Person, or, with respect to such other Person that is not a corporation, has other comparable ownership interest; or (ii) has the power, whether pursuant to contract, ownership of securities or otherwise, to direct the management and policies of such other Person.

(b) "***Agreement***" has the meaning set forth in the Recitals.

(c) " ***Alternative Transaction***" means, other than the transactions contemplated by this Agreement, any proposal or offer from any Person or group of Persons (other than Buyer or its Affiliates or their respective Representatives) for any acquisition by, or transfer, assignment, encumbrance, license or other grant of rights or disposition to, such Person or group of Persons of any right, title or interest in or to the Purchased Assets; *provided*, that "***Alternative Transaction***" shall not include any debt or equity financing transaction of the Seller or any acquisition of substantially all of Seller's assets or a majority of

the direct or indirect equity interests in Seller (whether through a stock purchase, merger, sale of all or substantially all assets or otherwise) so long as such acquisition provides that this Agreement continues to be binding, enforceable and in full force and effect on the same terms in effect as of the Effective Date.

(d) "***Approval Letter***" means the letter from the FDA dated March 26, 2026, approving the Subject BLA, attached hereto as Exhibit A.

(e) "***Asset Purchase***" has the meaning set forth in the Recitals.

(f) " ***Business Day***" means a day (i) other than Saturday or Sunday and (ii) on which commercial banks are open for business in New York, New York.

(g) "***Buyer***" has the meaning set forth in the Preamble.

(h) "***Buyer Indemnitee***" has the meaning set forth in Section 8.01(a).

(i) "***Closing***" has the meaning set forth in Section 3.01.

(j) "***Closing Date***" has the meaning set forth in Section 3.01.

(k) "***Confidential Information***" means (i) any and all confidential and proprietary information, including but not limited to, data, results, conclusions, know-how, experience, financial information, plans and forecasts, that may be delivered, made available, disclosed or communicated by a Party or its Affiliates or their respective Representatives to the other Party or its Affiliates or their respective Representatives, related to the subject matter hereof or otherwise in connection with this Agreement and (ii) the terms and conditions of this Agreement that are not made publicly available pursuant to the press release contemplated by the first sentence of Section 10.05 and those that are redacted in the version of this Agreement filed by Seller with the United States Securities and Exchange Commission pursuant to Section 10.05. "***Confidential Information***" will not include information that (A) at the time of disclosure, is generally available to the public, (B) after disclosure hereunder, becomes generally available to the public, except as a result of a breach of this Agreement by the recipient of such information, (C) becomes available to the recipient of such information from a Third Party that is not legally or contractually prohibited by the disclosing Party from disclosing such Confidential Information; or (D) was developed by or for the recipient of such information without the use of or reference to any of the Confidential Information of the disclosing Party or its Affiliates, as evidenced by the recipient's contemporaneous written records. Notwithstanding anything herein to the contrary, all Confidential Information included within the Purchased Assets (which, for the avoidance of doubt, shall not include any confidential or proprietary information relating to the product to which the Subject BLA relates) shall constitute Confidential Information of the Buyer from and after the Closing Date.

(l) "***Confidentiality Agreement***" means that certain Mutual Confidential Disclosure Agreement, dated as of April 11, 2026, by and between Buyer and Seller.

(m) " ***Contract***" means any written or oral legally binding contract, agreement, instrument, commitment or undertaking (including leases, licenses, mortgages, notes, guarantees, sublicenses, subcontracts and purchase orders).

(n) "***Damages***" has the meaning set forth in Section 8.01(a).

(o) "***Effective Date***" has the meaning set forth in the Preamble.

(p) " ***Encumbrance***" means any lien, pledge, charge, mortgage, easement, encroachment, imperfection of title, title exception, title defect, right of possession, lease, security interest, encumbrance, right of negotiation or refusal, adverse claim, interference or other restriction on ownership, use or transfer, excluding the requirement to pay the Priority Review Fee.

(q) "***Excluded Liabilities***" has the meaning set forth in Section 2.01(b).

(r) "***FDA***" means the United States Food and Drug Administration.

(s) "***FDCA***" means the United States Federal Food, Drug, and Cosmetic Act, as amended, and including any rules and regulations promulgated thereunder.

(t) "***Fraud***" means a party's actual and intentional fraud under Delaware common law in the making of any representations and warranties made by such party as expressly set forth in Article IV or Article V hereof, as applicable.

(u) " ***Fundamental Representations***" means the representations and warranties contained in Section 4.01, Section 4.02, Section 4.03(b)(i), Section 4.05, Section 4.07(b), Section 4.10, and Section 4.13.

(v) "***Governmental Entity***" means any supranational, national, state, municipal, local or foreign government, any court, tribunal, arbitrator, administrative agency, commission or other governmental official, authority or instrumentality, in each case whether domestic or foreign, any stock exchange or similar self-regulatory organization or any quasi-governmental or private body exercising any regulatory, taxing or other governmental or quasi-governmental authority.

(w) "***HSR Act***" means the Hart-Scott-Rodino Antitrust Improvements Act of 1976, as amended, and the rules and regulations promulgated thereunder.

(x) "***Indemnitee***" has the meaning set forth in Section 8.02(a).

(y) "***Indemnitor***" has the meaning set forth in Section 8.02(a).

(z) " ***Knowledge***" means, with respect to Seller, the actual knowledge of the facts and information of (i) Gaurav Shah, MD (Chief Executive Officer), (ii) Sanchali Kasbekar, PharmD (Associate Vice President, Global Regulatory Affairs, Cell and Gene Therapy), and (iii) Martin Wilson (General Counsel and Chief Corporate Officer), after performing a reasonable inquiry with respect to such facts and information.

(aa) " ***Legal Requirements***" means any federal, state, foreign, local, municipal or other law, statute, constitution, principle of common law, code, rule, regulation, or ruling issued, enacted, adopted, promulgated, implemented or otherwise put into effect by or under the authority of any Governmental Entity and any Orders applicable to a Party or to any of its assets, properties or businesses. Legal Requirements shall include, with respect to Seller, any responsibilities, requirements, parameters and conditions relating to the Priority Review Voucher set forth in (i) the Approval Letter, (ii) any other correspondence received by Seller or its Affiliates from the FDA regarding the Priority Review Voucher, or (iii) Section 529 of the FDCA (21 U.S.C. § 360ff), including as interpreted by the FDA in FDA's Draft Guidance, "Rare Pediatric Disease Priority Review Vouchers – Guidance for Industry" (July 2019).

(bb) " ***Liabilities***" means all debts, Taxes, liabilities and obligations, whether presently in existence or arising hereafter, accrued or fixed, absolute or contingent, matured or unmatured, determined

or determinable, asserted or unasserted, known or unknown, including those arising under any Legal Requirement or any Contract.

(cc) "***Notice of Intent to Use***" means notification to the FDA not later than ninety (90) days prior to the submission of a human drug application of the intent to use the Priority Review Voucher to obtain Priority Review of a human drug application, as described in 21 U.S.C. § 360ff(b)(4)(B)(i).

(dd) " ***Order***" means any order, decree, edict, injunction, writ, award or judgment of any Governmental Entity.

(ee) "***Outside Date***" has the meaning set forth in Section 9.01(b).

(ff) "***Party***" has the meaning set forth in the Preamble.

(gg) " ***Person***" means any natural person, company, corporation, limited liability company, general partnership, limited partnership, trust, proprietorship, joint venture, business organization or Governmental Entity.

(hh) "***Pre-Closing Period***" has the meaning set forth in Section 7.03.

(ii) "***Priority Review***" means review and action on a human drug application (as defined in 21 U.S.C. § 379g(1)) by the FDA in accordance with the timelines set forth by the FDA for "priority review" applications in the then-current Prescription Drug User Fee Act goals letter, as described in FDA Draft Guidance, "Rare Pediatric Disease Priority Review Vouchers – Guidance for Industry" (July 2019).

(jj) "***Priority Review Fee***" has the meaning set forth in Section 11.02.

(kk) "***Priority Review Voucher***" means the priority review voucher issued by the United States Secretary of Health and Human Services, Food and Drug Administration, to Seller, as evidenced in the Approval Letter, identified by priority review voucher number PRV BLA 125806.

(ll) " ***Proceeding***" means any action, arbitration, audit, hearing, investigation, proceeding, litigation or suit (whether civil, criminal, administrative, judicial or investigative, whether formal or informal, whether public or private) commenced, brought, conducted or heard by or before, or otherwise involving, any Governmental Entity or arbitrator.

(mm) "***Purchase Price***" has the meaning set forth in Section 2.02.

(nn) "***Purchased Assets***" means (i) the Priority Review Voucher, and (ii) any and all rights, benefits and entitlements afforded to the holder of the Priority Review Voucher.

(oo) "***Regulatory Change"*** means any term or condition that is not set forth in the Approval Letter imposed by the FDA on the Priority Review Voucher that (x) is not generally imposed on priority review vouchers under the FDCA, and (y) adversely impacts or limits, in any material respect, the manner in which Buyer may use, receive, hold or otherwise exploit the Priority Review Voucher.

(pp) "***Representative***" means, with respect to a particular Person, any director, officer, manager, employee, agent, consultant, advisor, accountant, financial advisor, legal counsel or other representative of that Person.

(qq) "***Seller***" has the meaning set forth in the Preamble.

(rr) "***Seller Indemnitee***" has the meaning set forth in Section 8.01(b).

(ss) "***Seller Notice of Transfer Submission***" has the meaning set forth in Section 3.02(e).

(tt) "***Subject BLA***" means BLA Number 125806, approved by the FDA on March 26, 2026, for KRESLADI™ (marnetegragene autotemcel) for the treatment of pediatric patients with severe leukocyte adhesion deficiency-I (LAD-I) due to biallelic variants in *ITGB2* without an available human leukocyte antigen-matched sibling donor for allogeneic hematopoietic stem cell transplant.

(uu) "***Tax***" or "***Taxes***" means any and all domestic and non-U.S., federal, state, provincial, local, municipal and other taxes, fees, levies, duties, tariffs, imposts and like assessments or charges of whatever kind, including taxes or charges on, or measured by or with respect to, gross or net income, gain, gross receipts, capital, franchise, windfall and other profits, sales, use, real or personal property, payroll, as well as any value added, ad valorem, transfer, license, withholding, employment, unemployment, excise, severance, stamp, occupation, municipal, municipal surcharge, environmental, social security, escheat, unclaimed property and other tax, together with any interest or any penalty thereon and addition thereto, whether disputed or not.

(vv) " ***Tax Authority***" means, with respect to any Tax, the Governmental Entity having jurisdiction over the assessment, determination, collection or imposition of such Tax.

(ww) "***Tax Deduction***" means any deduction or withholding for or on account of Tax.

(xx) "***Tax Treaty***" has the meaning set forth in Section 2.04.

(yy) "***Third Party***" means any Person other than a Party and such Party's Affiliates.

(zz) "***Third Party Claims***" has the meaning set forth in Section 8.01(a).

(aaa) "***Transfer Taxes***" has the meaning set forth in Section 11.02.

(bbb) "***Valid Account Details***" has the meaning set forth in Section 2.03.

(ccc) "***VAT***" means the Tax as currently constituted by the [***] and any other Tax imposed in addition or in substitution for it at the rate from time to time imposed.

Other capitalized terms defined elsewhere in this Agreement and not defined in this Section 1.01 shall have the meanings assigned to such terms in this Agreement.

ARTICLE II.
PURCHASE AND SALE

Section 2.01 Purchase and Sale; No Assumed Liabilities.

(a) Upon the terms and subject to the conditions of this Agreement, Buyer agrees to purchase from Seller, and Seller agrees to sell, transfer, convey, assign and deliver to Buyer, at the Closing all of Seller's right, title and interest in, to and under the Purchased Assets, in each case free and clear of all Encumbrances.

(b) For the avoidance of doubt, (i) the sale, assignment, transfer and conveyance of the Purchased Assets from Seller to Buyer shall not include the transfer, conveyance or assumption of any

Liabilities from Seller to Buyer, and (ii) Buyer shall not assume or be liable for any Liabilities of Seller or its Affiliates (fixed, contingent or otherwise, and whether or not accrued), including Liabilities relating to the Purchased Assets (other than such obligations as are imposed generally by applicable Legal Requirements solely on the holder of the Priority Review Voucher in respect of its use or transfer following the sale thereof pursuant to this Agreement, including, without limitation, the Priority Review Fee and any other user fees required to be paid to redeem the Priority Review Voucher) (such Liabilities, "***Excluded Liabilities***").

Section 2.02 Purchase Price. The total consideration (the "***Purchase Price***") to be paid by Buyer to Seller for all of the Purchased Assets shall be One Hundred and Eighty Million Dollars (U.S. $180,000,000) due and payable upon the Closing Date.

Section 2.03 Method of Payment. Payment of the Purchase Price to Seller shall be made in cash by wire transfer of immediately available funds to a bank account specified by Seller in writing to Buyer in the form of Valid Account Details no later than five (5) Business Days prior to the Closing Date. "***Valid Account Details***" means, with respect to any bank account, the valid (a) name of bank, (b) bank's address, (c) account number, (d) account name and (e) ABA/Routing number.

Section 2.04 Taxes. For the avoidance of doubt, Seller alone shall be responsible for paying to the appropriate Tax Authority all Taxes (other than Tax Deductions or Transfer Taxes for which the Buyer is responsible pursuant to Section 11.02) levied on Seller on account of, or measured in whole or in part by reference to, the Purchase Price. Where Buyer is required by applicable Legal Requirements to make a Tax Deduction from the Purchase Price, Buyer shall be entitled to make such Tax Deduction and remit such Tax Deduction to the appropriate Tax Authority in accordance with the applicable Legal Requirements; *provided, however*, that prior to making any such Tax Deduction, Buyer shall provide written notice to Seller of Buyer's determination of, and a reasoned legal basis for, its requirement to make such Tax Deduction no later than five (5) Business Days prior to the Closing Date (the "***Notice Requirement***"). As of the Effective Date, it is the Parties' mutual understanding that no Tax Deductions are required in connection with the payment of the Purchase Price both under applicable Legal Requirements [***] (the "***Tax Treaty***"). Seller hereby represents that it (a) is tax resident in the United States of America by application of Article 4 of the Tax Treaty; (b) is the beneficial owner of all payments due to Seller under this Agreement, as would be required in order to apply Article 12 of the Tax Treaty with regard to such payments; (c) does not carry on business through a permanent establishment situated in [***] and (d) is not subject to any restriction or limitation in applying Article 12 of the Tax Treaty imposed by Article 23, Limitations on Benefits. Buyer shall reasonably cooperate with Seller to obtain any available reduction of or relief from such deduction or withholding to the extent permitted by applicable Legal Requirements. To the extent Buyer is required by applicable Legal Requirements to make a Tax Deduction and complied with the Notice Requirement with respect to such Tax Deduction, such Tax Deduction shall be treated for all purposes of this Agreement as having been paid to the Person in respect of which such deduction and withholding was made.

Section 2.05 Tax Cooperation. The Parties shall procure that their group tax functions shall fully cooperate with each other in relation to any reasonable request in connection with any matter relating to Tax arising out of the transactions contemplated by this Agreement, including information required for the preparation and filing of any Tax return or the conduct of any audit, investigation, dispute or appeal or any other communication with any Tax Authority, in each case if and to the extent: (a) legally permissible; and (b) that such disclosure would not breach any duty of confidentiality or waive any privilege. The requesting Party shall be responsible for any third-party costs properly incurred by the other Party in complying with this Section 2.05.

Section 2.06 Intended Tax Treatment. The purchase and sale of the Purchased Assets in exchange for the Purchase Price is intended to be treated for all U.S. federal, state, local and non-U.S. Tax purposes as the sale of property that is not contingent on the productivity, use, or disposition of the property (the "***Intended Tax Treatment***"). Neither Party will take any position (whether in financial statements, audits, Tax returns or otherwise) that is inconsistent with the Intended Tax Treatment unless (a) otherwise required by applicable Legal Requirements and (b) such Party has obtained a written opinion from an internationally recognized independent tax advisory firm, in form and substance reasonably acceptable to the other Party, supporting the basis for such inconsistent position.

ARTICLE III.
CLOSING

Section 3.01 Closing. The consummation of the Asset Purchase (the "***Closing***") shall be conducted telephonically or via email or other similar means of correspondence on such date to be mutually agreed upon by Buyer and Seller, which date shall be no later than three (3) Business Days after all of the conditions set forth in Article VI have been satisfied or waived (other than those conditions which, by their terms, are intended to be satisfied at the Closing, but subject to satisfaction or waiver of such conditions). The date on which the Closing actually takes place is referred to in this Agreement as the "***Closing Date***."

Section 3.02 Transactions to be Effected at Closing. At the Closing,

(a) Seller shall deliver, or cause to be delivered, to Buyer an executed Bill of Sale substantially in the form attached hereto as Exhibit B;

(b) Seller shall deliver, or cause to be delivered, to Buyer an executed certificate from a duly authorized officer of the Seller certifying as to the matters set forth in Section 6.02(c);

(c) Buyer shall deliver, or cause to be delivered, to Seller an executed certificate from a duly authorized officer of the Buyer certifying as to the matters set forth in Section 6.03(c);

(d) Seller shall deliver, or cause to be delivered, to Buyer an executed certificate of the secretary or an assistant secretary (or equivalent duly authorized officer or other representative) of Seller certifying (i) that attached thereto are true and complete copies of all resolutions adopted by the board of directors of Seller authorizing the execution, delivery and performance of this Agreement and the consummation of the transactions contemplated hereby, and that all such resolutions are in full force and effect and are all the resolutions adopted in connection with the transactions contemplated hereby, and (ii) as to the incumbency of each person executing this Agreement and any other document delivered in connection herewith on behalf of Seller and that the signature of each such person on this Agreement and such other document is such person's genuine signature;

(e) Within one (1) Business Day following the Closing Date, Seller shall (on behalf of Buyer) submit, or cause to be submitted, to the FDA the separate notifications referred to in Section 3.02(g) and Section 3.02(h), respectively, as a submission to the Subject BLA through the FDA's Electronic Submissions Gateway under the cover letter in the form attached as Exhibit C. Seller shall provide to Buyer, within two (2) Business Days following their submission to the FDA, confirmation from the FDA of successful submission and a complete copy of such submission (the "***Seller Notice of Transfer Submission***"). Buyer may also submit the duly executed letters provided to be delivered in Section 3.02(g) and Section 3.02(h) hereof to the FDA following Seller's notification to Buyer of its submission, and Buyer's receipt from Seller, of a copy of the Seller Notice of Transfer Submission.

(f) Buyer shall pay the Purchase Price to Seller by wire transfer of immediately available funds to an account or accounts designated in writing by Seller to Buyer in the form of Valid Account Details, such designation to occur at least five (5) Business Days prior to the Closing Date;

(g) Seller shall deliver to Buyer a letter addressed to Buyer, substantially in the form set forth on Exhibit D hereto and duly executed by Seller, acknowledging the transfer of the Priority Review Voucher from Seller to Buyer, in accordance with this Agreement; and

(h) Buyer shall deliver to Seller a letter addressed to Seller, substantially in the form set forth on Exhibit E hereto and duly executed by Buyer, acknowledging the transfer of the Priority Review Voucher from Seller to Buyer, in accordance with this Agreement.

Section 3.03 Title Passage. Upon the Closing, all of the right, title and interest of Seller in and to the Purchased Assets shall pass to Buyer.

ARTICLE IV.
REPRESENTATIONS AND WARRANTIES OF SELLER

Seller represents and warrants to Buyer, as of the Effective Date and the Closing Date, as follows:

Section 4.01 Organization, Standing and Power. Seller is a corporation duly organized and validly existing under the laws of Delaware. Seller has the corporate power and authority to own, operate and lease its properties and to carry on its business as presently conducted and is duly qualified or licensed to do business and is in good standing in each jurisdiction where the character of its properties owned or leased or the nature of its activities make such qualification or licensing necessary, except where the failure to be so qualified or licensed would not, individually or in the aggregate, reasonably be expected to adversely affect any of the Purchased Assets, Seller's ability to consummate the transactions contemplated by this Agreement, or Buyer's ownership and rights with respect to any of the Purchased Assets after the Closing. Seller is not in violation of its certificate of incorporation or bylaws.

Section 4.02 Due Authority. Seller has the requisite corporate power and authority to enter into, deliver and perform its obligations under, and consummate the transactions contemplated by, this Agreement. The execution, delivery and performance of this Agreement, and the consummation of the Asset Purchase, have been duly and validly approved and authorized by all necessary corporate action on the part of Seller, and this Agreement has been duly executed and delivered by Seller. This Agreement, upon execution by the Parties, will constitute a valid and binding obligation of Seller enforceable against Seller in accordance with its terms, subject only to the effect, if any, of (a) applicable bankruptcy and other similar laws affecting the rights of creditors generally and (b) rules of law governing specific performance, injunctive relief and other equitable remedies. The approval of Seller's stockholders is not required for the execution, delivery and performance of this Agreement, and the consummation of the Asset Purchase.

Section 4.03 Noncontravention. The execution and delivery by Seller of this Agreement does not, and the consummation of the transactions contemplated hereby, including the transfer of title to, ownership in, and possession of the Purchased Assets, will not, (a) result in the creation of any Encumbrance on any of the Purchased Assets or (b) conflict with, or result in any violation of or default under (with or without notice or lapse of time, or both), or give rise to a right of termination, revocation, suspension, cancellation or acceleration of any obligation or loss of any benefit under, or require any consent, approval or waiver from any Person pursuant to, (i) any provision of the certificate of incorporation or bylaws of Seller, (ii) the Approval Letter or any Contract to which Seller is a party or by which it is bound which involves or affects in any way any of the Purchased Assets or (iii) except as may be required to comply with the HSR Act, any Legal Requirements applicable to Seller or any of the Purchased Assets, except, in

the case of clauses (ii) and (iii) above, as would not, individually or in the aggregate, have an adverse effect on Seller's ability to consummate the sale of the Purchased Assets at the Closing and perform its other obligations under this Agreement or Buyer's ownership and rights with respect to any of the Purchased Assets after the Closing.

Section 4.04 No Consents. Except for the letters referenced in Section 3.02(g) and Section 3.02(h) and the filing of any Premerger Notification and Report Form required under the HSR Act, no filing, authorization, consent, approval, permit, order, registration or declaration, governmental or otherwise, is necessary to enable or authorize Seller to enter into, and to perform its obligations under, this Agreement.

Section 4.05 Title to Purchased Assets. Seller is the sole and exclusive owner of the Purchased Assets and owns and at the Closing will transfer to Buyer good and transferable title to the Purchased Assets free and clear of any Encumbrances. Neither Seller nor any of its Affiliates has sold, transferred, conveyed, assigned, or delivered any Purchased Assets to any Person, and Seller has the full and sole right to sell, transfer, convey, assign, and deliver the Purchased Assets to Buyer free and clear of all Encumbrances and, at the Closing, will sell, transfer, convey, assign and deliver to Buyer good and transferable title to the Purchased Assets free and clear of any Encumbrances. Except as set forth in Section 4.13 hereof, no Third Party is entitled to any portion of the proceeds of the transactions contemplated by this Agreement.

Section 4.06 Contracts. Except for this Agreement, there is no Contract to which Seller or any Affiliate of Seller is a party that involves or affects, or would reasonably be expected to involve or affect, the ownership of, transfer or licensing of, title to, or use of any of the Purchased Assets.

Section 4.07 Compliance With Legal Requirements.

(a) Seller and its Affiliates are, and at all times have been, in compliance in all material respects with each Legal Requirement that is or was applicable to (a) Seller's and its Affiliates' conduct, acts, or omissions with respect to any of the Purchased Assets or (b) any of the Purchased Assets. Seller and its Affiliates have not received any written or, to the Seller's Knowledge, oral notice or other communication from any Person, including the FDA, regarding any actual, alleged, possible or potential violation of, or failure to comply with, any such Legal Requirement.

(b) During the three (3) year period prior to the Closing Date and as it relates to the FDA approval of the Subject BLA, the Approval Letter, the Priority Review Voucher or the activities giving rise to such FDA approval of the Subject BLA, the Approval Letter or the Priority Review Voucher, neither Seller, any Affiliate of Seller, nor to the Knowledge of Seller, any Representative of Seller or any Affiliate of Seller, has (i) made an untrue statement of material fact or a fraudulent statement to the FDA or any other Governmental Entity, or (ii) committed an act, made a statement or failed to make a statement that, in each case ((i) or (ii)), at the time such disclosure was made or should have been made, as applicable, would reasonably be expected to provide a basis for the FDA to invoke its policy respecting "Fraud, Untrue Statements of Material Facts, Bribery, and Illegal Gratuities," set forth in 56 Fed. Reg. 46191 (September 10, 1991) or for any other Governmental Entity to invoke any similar policy and which could reasonably be expected to result in a revocation of the Priority Review Voucher.

Section 4.08 Legal Proceedings. There is no pending, or to Seller's Knowledge, threatened Proceeding involving Seller or any of its Affiliates, nor has there been any Proceeding involving Seller or any of its Affiliates, and neither Seller nor any of its Affiliates are a party or subject to the provisions of any Order, in each case, (a) that involves or affects (or may involve or affect) the issuance of, continued validity of, ownership of, transfer or license of, title to, or use of any of the Purchased Assets (including any such Order that seeks to prohibit or limit in any respect, or place any conditions on, the ownership or

use by Buyer or its Affiliates of any of the Purchased Assets, in each case, as a result of the transactions contemplated by this Agreement), or (b) that otherwise challenges or seeks to restrain, prohibit, prevent, enjoin, alter, or delay the consummation of the transactions contemplated by this Agreement.

Section 4.09 Governmental Authorizations. Neither Seller nor any of its Affiliates is required to hold any license, registration, or permit issued by any Governmental Entity to own, use or transfer the Purchased Assets, other than such licenses, registrations or permits that have already been obtained.

Section 4.10 Revocation; Regulatory Change. The Priority Review Voucher has been duly granted and issued and has not been terminated, cancelled, revoked, or used. Neither Seller nor any of its Affiliates has done or omitted to do any act, and, to the Seller's Knowledge, there are no facts or circumstances that would reasonably be expected to (with or without notice or lapse of time or both) give rise to a right of the FDA to revoke (or that would otherwise result in the termination, cancellation, revocation, or use of) the Priority Review Voucher, or result in the redemption or transfer of the Priority Review Voucher (other than pursuant to the transactions contemplated by this Agreement), or that would preclude or interfere with the sale and transfer of the Purchased Assets to Buyer or Buyer's use of the Purchased Assets following the Closing to obtain Priority Review (other than as set forth in any Legal Requirements in existence on the date hereof). To the Knowledge of Seller, there is no term or condition imposed by the FDA on the Priority Review Voucher that is not set forth in the Approval Letter or provided for under applicable Legal Requirements. From the date that the Priority Review Voucher was issued until the Effective Date, there has not occurred any Regulatory Change.

Section 4.11 Document Disclosure. Attached as Schedule 4.11 is a true, correct and complete list of all formal written communications between Seller or its Affiliates, on the one hand, and the FDA, on the other hand, with respect to the Purchased Assets. True, correct and complete copies of each of such communications have been made available to Buyer as of the close of business on the last Business Day immediately preceding the Closing Date.

Section 4.12 Intent to Use. Neither Seller nor any of its Affiliates has filed or submitted, or permitted any Third Party to file or submit, to the FDA a Notice of Intent to Use the Priority Review Voucher.

Section 4.13 No Broker. Except for Leerink Partners LLC, the fees and expenses of which shall be paid by Seller, there is no investment banker, broker, finder or other intermediary which has been authorized to act on behalf of Seller who might be entitled to any fee or commission in connection with the transactions contemplated by this Agreement.

Section 4.14 Taxes. Seller and its Affiliates have timely paid all amounts of Tax required to be paid on or prior to the date hereof, if a failure to pay such Tax could reasonably be expected to result in a lien on any of the Purchased Assets. There are no liens on account of Taxes on the Purchased Assets and no material audits, controversies or claims by a Governmental Entity pending or threatened against Seller with respect to Taxes relating to the Purchased Assets.

Section 4.15 No Other Representations. Neither Seller nor any of its Representatives is making any representation or warranty of any kind or nature whatsoever, oral or written, express or implied, except as otherwise expressly set forth in this Article IV, and Seller hereby disclaims any such other representations and warranties.

ARTICLE V.
REPRESENTATIONS AND WARRANTIES OF BUYER

Buyer represents and warrants to Seller, as of the Effective Date and the Closing Date, as follows:

Section 5.01 Organization, Standing and Power. Buyer is a limited company organized under the laws of [***]. Buyer has the corporate power and authority to own, operate and lease its properties and to carry on its business as presently conducted and is duly qualified or licensed to do business and is in good standing in each jurisdiction where the character of its properties owned or leased or the nature of its activities make such qualification or licensing necessary, except where the failure to be so qualified or licensed would not, individually or in the aggregate, reasonably be expected to adversely affect Buyer's ability to consummate the transactions contemplated by this Agreement. Buyer is not in violation of its memorandum and articles of association.

Section 5.02 Authority. Buyer has the requisite corporate power and authority to enter into and perform its obligations under this Agreement. The execution, delivery and performance of this Agreement, and the consummation of the Asset Purchase, have been duly and validly approved and authorized by all necessary corporate action on the part of Buyer, and this Agreement has been duly executed and delivered by Buyer. This Agreement, upon execution by the Parties, will constitute a valid and binding obligation of Buyer enforceable against Buyer in accordance with its terms, subject only to the effect, if any, of (a) applicable bankruptcy and other similar laws affecting the rights of creditors generally and (b) rules of law governing specific performance, injunctive relief and other equitable remedies.

Section 5.03 Noncontravention. The execution and delivery by Buyer of this Agreement does not, and the consummation of the transactions contemplated hereby will not, conflict with, or result in any violation of or default under (with or without notice or lapse of time, or both), or give rise to a right of termination, revocation, suspension, cancellation or acceleration of any obligation or loss of any benefit under, or require any consent, approval or waiver from any Person pursuant to, (a) any provision of the memorandum and articles of association of Buyer, (b) any Contract to which Buyer is a party or by which it is bound which involves or affects in any way the Asset Purchase or (c) except as may be required to comply with the HSR Act, any Legal Requirements applicable to Buyer.

Section 5.04 No Consents. Except for the letters referenced in Section 3.02 and the filing of any Premerger Notification and Report Form required under the HSR Act, no filing, authorization, consent, approval, permit, order, registration or declaration, governmental or otherwise, is necessary to enable or authorize Buyer to enter into, and to perform its obligations under, this Agreement.

Section 5.05 Funding. Buyer has, and will at Closing have, sufficient funds to consummate the transactions contemplated by this Agreement.

Section 5.06 No Broker. Buyer has not engaged, retained or entered into an agreement with any investment banker, broker, finder or other intermediary who has been authorized to act on behalf of Buyer who would be entitled to any fee or commission payable by Seller in connection with the transactions contemplated by this Agreement.

Section 5.07 Non-Reliance. Neither Seller nor any of its Affiliates nor any of their Representatives makes, or has made any representation or warranty, oral or written, express or implied, as to the accuracy or completeness of any information concerning the Purchased Assets contained herein or made available in connection with Buyer's investigation of the foregoing, except as expressly set forth in this Agreement. Buyer acknowledges that Seller, its Affiliates and their Representatives expressly disclaim any and all liability that may be based on such information or errors therein or omissions therefrom. Buyer

has not relied and is not relying on any statement, representation or warranty, oral or written, express or implied (including any representation or warranty as to merchantability or fitness for a particular purpose), made by Seller, any of its Affiliates or any of their Representatives, except as expressly set forth in this Agreement.

ARTICLE VI.
CONDITIONS TO CLOSING

Section 6.01 Conditions Precedent of Buyer and Seller. Each Party's obligations to consummate the transactions contemplated by this Agreement are subject to the satisfaction or waiver, at or prior to the Closing Date, of each of the following conditions precedent:

(a) HSR Act. The applicable waiting period under the HSR Act relating to the transactions contemplated by this Agreement shall have expired or been terminated.

(b) No Injunctions or Restraints. No temporary restraining order, preliminary or permanent injunction or other material Order issued or promulgated by a Governmental Entity preventing the consummation of the transactions contemplated by this Agreement shall be in effect, and there shall not be any applicable Legal Requirement that makes consummation of the transactions contemplated by this Agreement illegal.

(c) No Governmental Litigation. There shall not be any Proceeding commenced or pending by a Governmental Entity seeking to prohibit, limit, delay, or otherwise restrain the consummation of this Agreement and/or the transactions contemplated hereby.

Section 6.02 Buyer's Conditions Precedent. The obligations of Buyer to consummate the transactions contemplated by this Agreement are subject to the satisfaction or waiver, at or prior to the Closing Date, of each of the following conditions precedent:

(a) Accuracy of Representations. Each of the representations and warranties made by Seller in this Agreement (other than the Fundamental Representations) shall be true and correct (without giving effect to any limitation or qualification as to "materiality" (including the word "material") or "material adverse effect" set forth therein) in all material respects at and as of the Effective Date and as of the Closing Date (or, if made as of a specified period or date, as of such period or date). Each of the Fundamental Representations shall be true and correct in all respects at and as of the Effective Date and as of the Closing Date (or, in each case, if made as of a specified period or date, as of such period or date).

(b) Performance of Covenants. All of the covenants and obligations that Seller is required to comply with or to perform hereunder at or prior to the Closing Date shall have been complied with and performed in all material respects.

(c) Closing Certificate. Seller shall have delivered to Buyer a certificate, dated the Closing Date and duly executed by Seller, certifying that the conditions set forth in Section 6.02(a) and Section 6.02(b) have been satisfied.

(d) No Regulatory Change. Since the Effective Date there shall not have occurred and remain in effect any Regulatory Change.

Section 6.03 Seller's Conditions Precedent. The obligations of Seller to consummate the transactions contemplated by this Agreement are subject to the satisfaction or waiver, at or prior to the Closing Date, of each of the following conditions precedent:

(a) Accuracy of Representations. Each of the representations and warranties made by Buyer in this Agreement shall be true and correct (without giving effect to any limitation or qualification as to "materiality" (including the word "material") or "material adverse effect" set forth therein) in all material respects at and as of the Closing Date (or, if made as of a specified period or date, as of such period or date), except to the extent that such representations and warranties are qualified by the term "material", or words of similar import, in which case such representations and warranties (as so written, including the terms "material", or words of similar import) shall be true and correct in all respects at and as of the Closing Date (or, if made as of a specified period or date, as of such period or date).

(b) Performance of Covenants. All of the covenants and obligations that Buyer is required to comply with or to perform hereunder at or prior to the Closing Date shall have been complied with and performed in all material respects.

(c) Closing Certificate. Buyer shall have delivered to Seller a certificate, dated the Closing Date and duly executed by Buyer, certifying that the conditions set forth in Section 6.03(a) and Section 6.03(b) have been satisfied.

ARTICLE VII.
PRE-CLOSING COVENANTS AND AGREEMENTS

Section 7.01 Antitrust Notification.

(a) The Parties shall use their reasonable best efforts to take, or cause to be taken, all actions and to do, or cause to be done, all things necessary or advisable to consummate the transactions contemplated by this Agreement. Without limiting the foregoing, Seller and Buyer shall file, or shall cause their ultimate parent entities as defined in the HSR Act to file, as soon as practicable (but not later than fifteen (15) Business Days) after the Effective Date, any notifications required under the HSR Act, and shall respond as promptly as practicable to all inquiries or requests received from the Federal Trade Commission, the Antitrust Division of the U.S. Department of Justice or any other Governmental Entity for additional information or documentation. In connection therewith, the Parties shall, or shall cause their respective Affiliates to, (i) furnish to the other Party such necessary information and reasonable assistance as the other Party may reasonably request in connection with its preparation of any filing or submission that is necessary under the HSR Act, and (ii) keep the other Party reasonably apprised of the status of any communications with, and any inquiries or requests for additional information from the applicable Governmental Entity. Neither Party shall request, or permit its "ultimate parent entity" to request, early termination of the HSR Act waiting period unless otherwise determined by Buyer in which case both Parties shall make such request for early termination.

(b) Subject to applicable confidentiality restrictions or restrictions required by applicable Legal Requirements, each Party will notify the other promptly upon the receipt of (i) any comments or questions from any Governmental Entity in connection with any filings made pursuant to Section 7.01 or the transactions contemplated by this Agreement and (ii) any request by any Governmental Entity for information or documents relating to an investigation of the transactions contemplated by this Agreement. Without limiting the generality of the foregoing, each Party shall provide to the other (or the other's respective advisors) upon request copies of all substantive correspondence between such Party and any Governmental Entity relating to the transactions contemplated by this Agreement. The Parties may, as they deem advisable and necessary, designate any competitively sensitive materials provided to the other under this Section 7.01 as "outside counsel only." Such materials and the information contained therein shall be given only to outside counsel of the recipient and will not be disclosed by such outside counsel to employees, officers, or directors of the recipient without the advance written consent of the Party providing such materials. In addition, to the extent reasonably practicable, representatives of both Parties shall have

a reasonable opportunity to participate in all substantive discussions, telephone calls, and meetings with a Governmental Entity regarding the transactions contemplated by this Agreement to the extent permitted by such Governmental Entity. Subject to applicable Legal Requirements, the Parties will consult and cooperate with each other in good faith in connection with any analyses, appearances, presentations, memoranda, briefs, arguments, and proposals made or submitted to any Governmental Entity regarding the transactions contemplated by this Agreement by or on behalf of any Party. Notwithstanding anything herein to the contrary, Buyer shall have, except where prohibited by applicable Legal Requirement, sole and complete responsibility for determining the strategy for obtaining consents and approvals of any Governmental Entity, having in good faith considered comments made by Seller.

(c) Notwithstanding the foregoing, nothing in this Agreement shall require, or be construed to require, the Parties or any of their respective Affiliates to offer or agree to (i) (A) sell, hold, hold separate, divest, license, discontinue or limit, before or after the Closing Date, any assets, businesses, equity holdings, intellectual property, or other interests or (B) any conditions relating to, or changes or restrictions in, the operations of any such assets, businesses, equity holdings, intellectual property or interests (including any requirements to enter into new Contracts or modify or terminate existing Contracts), including with respect to the Purchased Assets and use of the Priority Review Voucher to obtain Priority Review of a product candidate of Buyer or its Affiliates or any other benefit associated with the Purchased Assets or (ii) any material modification or waiver of the terms and conditions of this Agreement.

(d) Buyer shall bear all filing fees related to any notifications under the HSR Act.

Section 7.02 Regulatory Change Notification. Until the earlier of the Closing or the termination of this Agreement, Seller shall provide Buyer with prompt written notification of the occurrence of any Regulatory Change of which Seller becomes aware.

Section 7.03 Efforts. During the period from the Effective Date and continuing until the earlier of the termination of this Agreement or the Closing Date (the "***Pre-Closing Period***"), except as otherwise expressly contemplated by this Agreement or with such other Party's prior written consent, which consent shall not be unreasonably withheld, conditioned or delayed, each Party shall not, and shall cause its Affiliates not to, knowingly take or permit any action that, or omit to take any action the absence of which, would reasonably be expected to prevent or materially delay the satisfaction of the conditions set forth in Article VI.

Section 7.04 No Solicitation. During the Pre-Closing Period, Seller shall not and shall cause its controlled Affiliates not to, nor shall it authorize or instruct any of its other Affiliates or its or their Representatives to, (i) solicit, initiate, facilitate or encourage any inquiries, proposals or offers with respect to, or the submission of, any Alternative Transaction by any Person (other than Buyer or its Affiliates or their respective Representatives) or any inquiry, proposal or offer that is reasonably likely to lead to an Alternative Transaction, (ii) engage, continue or participate in any discussions or negotiations regarding, or take any other action intended or reasonably expected to facilitate the making of any inquiry, proposal or offer to Seller that constitutes, or may reasonably be expected to lead to, any Alternative Transaction by any Person (other than Buyer or its Affiliates or their respective Representatives) other than to state that they are not permitted to have discussions, (iii) accept any inquiry, proposal or offer from any Person (other than Buyer) in respect of an Alternative Transaction, or (iv) resolve to propose or agree to do any of the foregoing.

Section 7.05 Exclusivity. Until the earlier of the Closing or the termination of this Agreement, Seller shall not (a) transfer or assign the Priority Review Voucher to any Person other than Buyer or enter into any Contract with respect thereto, (b) encumber or otherwise grant or allow to exist any Encumbrance

on the Priority Review Voucher (other than pursuant to this Agreement), or (c) take any action or inaction that would reasonably be expected to prevent the satisfaction of the conditions set forth in Article VI.

ARTICLE VIII.
INDEMNIFICATION

Section 8.01 Indemnification.

(a) Indemnification by Seller. From and after the Closing, Seller will indemnify, defend and hold Buyer and its Affiliates, and their respective Representatives, successors and assigns (each, a "***Buyer Indemnitee***") harmless for, from and against any and all Liabilities, losses, damages, claims, costs and expenses (including reasonable attorneys' fees) (collectively, "***Damages***"), whether or not arising from, relating to, or otherwise in connection with a claim of a Third Party (each, a "***Third Party Claim***"), which any Buyer Indemnitee may suffer, incur, sustain, or become subject to, to the extent arising from, relating to or otherwise in connection with (i) any breach of, or inaccuracy in, any of Seller's representations and warranties made under this Agreement or any certificate delivered by Seller hereunder; (ii) any breach of, or failure to perform, any of Seller's covenants or obligations made under this Agreement or any certificate delivered by Seller hereunder; or (iii) arising out of any Excluded Liabilities.

(b) Indemnification by Buyer. From and after the Closing, Buyer will indemnify, defend and hold Seller and its Affiliates, and their respective Representatives, successors and assigns (each, a "***Seller Indemnitee***") harmless for, from and against any and all Damages, whether or not arising from, relating to or otherwise in connection with a Third Party Claim, which any Seller Indemnitee may suffer, incur, sustain, or become subject to, to the extent arising from, relating to or otherwise in connection with (i) any breach of, or inaccuracy in any of Buyer's representations and warranties made under this Agreement or any certificate delivered by Buyer hereunder; or (ii) any breach of, or failure to perform, any of Buyer's covenants or obligations made under this Agreement or any certificate delivered by Buyer hereunder.

Section 8.02 Indemnification Procedures.

(a) A Person entitled to indemnification pursuant to Section 8.01 will hereinafter be referred to as an *"**Indemnitee**."* A Party obligated to indemnify an Indemnitee hereunder will hereinafter be referred to as an *"**Indemnitor**."*

(b) A claim for indemnification for any matter not involving a Third Party Claim may be asserted by written notice to the Indemnitor. Such notice shall include the facts constituting the basis for such claim for indemnification, the Sections of this Agreement upon which such claim for indemnification is then based and an estimate, to the extent known, of the amount of Damages suffered or reasonably expected to be suffered by the Indemnitee; *provided*, that the failure to give such notification or any deficiency in such notification will not relieve such Indemnitor from any obligation under this Article VIII, except (i) to the extent such failure to give such notification or such deficiency in such notification actually and materially prejudices such Indemnitor or (ii) as provided in Section 11.01.

(c) In the event of any instituted or asserted Third Party Claim against an Indemnitee, Indemnitee shall inform Indemnitor of such Third Party Claim as soon as reasonably practicable after such Third Party Claim arises; *provided*, that the failure to give such notification or any deficiency in such notification will not relieve such Indemnitor from any obligation under this Article VIII, except (i) to the extent such failure to give such notification or such deficiency in such notification actually and materially prejudices such Indemnitor or (ii) as provided in Section 11.01.

(d) The Indemnitor shall have the right to defend, at its sole cost and expense (with counsel reasonably selected by the Indemnitor), a Third Party Claim by all appropriate proceedings, which proceedings shall be prosecuted diligently by the Indemnitor to a final conclusion or settled at the discretion of the Indemnitor; *provided*, *however*, that the Indemnitor may not assume control of defense to a Third Party Claim (i) unless it covenants to the Indemnitee in writing within ten (10) Business Days after the Indemnitee has given written notice of the Third Party Claim to the Indemnitor to indemnify, defend and hold harmless the Indemnitee from and against the entirety of any and all Damages that the Indemnitee may suffer resulting from or arising out of the Third Party Claim (subject, however, to the limitations set forth in Section 8.03), (ii) in which equitable relief other than monetary damages is sought, (iii) if such Third Party Claim is a criminal enforcement matter brought against Buyer, or (iv) if the Indemnitee has been advised in writing by outside counsel that a legal conflict or potential legal conflict exists between the Indemnitee and the Indemnitor in connection with conducting the defense of the Third Party Claim; *provided*, *further*, *however*, that the Indemnitor may not enter into any compromise or settlement without the prior written consent of the Indemnitee (which consent shall not be unreasonably withheld, conditioned or delayed) unless (A) such compromise or settlement includes the giving by each claimant or plaintiff to the Indemnitee of an unconditional release from all liability in respect of such Third Party Claim, (B) such settlement does not involve any admission of legal wrongdoing by the Indemnitee, (C) such settlement does not involve any payment by the Indemnitee that is not indemnified hereunder, and (D) such settlement does not involve the imposition of any equitable relief against the Indemnitee. If a good faith and diligent defense is not being or ceases to be materially conducted by the Indemnitor, the Indemnitee shall have the right, at the expense of the Indemnitor, upon at least ten (10) Business Days' prior written notice to the Indemnitor of its intent to do so, to undertake the defense of such Third Party Claim for the account of the Indemnitor (with counsel reasonably selected by the Indemnitee). If the Indemnitee is defending such Third Party Claim, the Indemnitee shall keep the Indemnitor apprised of all material developments with respect to such Third Party Claim and promptly provide the Indemnitor with copies of all material correspondence and documents exchanged by the Indemnitee and the opposing party(ies) to such litigation. If the Indemnitor has elected to defend such Third Party Claim or if the Indemnitor has otherwise acknowledged in writing its responsibility for indemnifying a Third Party Claim, the Indemnitee may not compromise or settle such Third Party Claim without the prior written consent of the Indemnitor, such consent not to be unreasonably withheld or delayed, and the Indemnitor shall have no liability in respect of any such compromise or settlement to which the Indemnitor has not provided its consent, unless such consent is unreasonably withheld or delayed.

(e) The Indemnitee may participate in, but not control, any defense or settlement of any Third Party Claim controlled by the Indemnitor pursuant to this Section 8.02 and shall bear its own costs and expenses with respect to such participation; *provided*, *however*, that the Indemnitor shall bear such costs and expenses (i) if counsel for the Indemnitor or counsel for the Indemnitee shall have reasonably determined that counsel for the Indemnitor may not properly represent both the Indemnitor and the Indemnitee or (ii) if such participation is requested by the Indemnitor.

Section 8.03 Limitations on Indemnification. Notwithstanding anything to the contrary contained in this Agreement, the maximum aggregate amount of indemnifiable Damages that may be recovered from (a) Seller pursuant to Section 8.01(a) shall equal the Purchase Price, and (b) Buyer pursuant to Section 8.01(b) shall equal the Purchase Price. Notwithstanding anything to the contrary set forth herein, except to the extent actually awarded against an Indemnitee pursuant to an Order with respect to a Third Party Claim and except for a Party's Fraud, no Party shall have any liability under any provision of this Agreement (including this Article VIII) for any punitive, special or indirect damages or damages for or otherwise based on business interruption, diminution of value, loss of future revenue, profits or income, or loss of business reputation or opportunity. Nothing in this Section 8.03 shall operate to limit or exclude in any way Seller's liability for any and all Excluded Liabilities.

Section 8.04 Additional Indemnification Matters. The right of indemnification provided under this Article VIII shall not be affected by any knowledge acquired (or capable of being acquired) at any time, whether before or after the Closing, with respect to the accuracy or inaccuracy of, or compliance or noncompliance with, any representation, warranty, covenant, or agreement contained herein. Notwithstanding anything to the contrary herein, for the purposes of this Article VIII, for the purposes of determining the amount of Damages, each representation or warranty made by a Party will be deemed made without any qualifications or limitations as to materiality and, without limiting the foregoing, the word "material" and words of similar import will be deemed deleted from any such representation or warranty.

Section 8.05 Exclusive Remedy. From and after the Closing, except in the case of Fraud and as otherwise provided in Section 11.09, the sole and exclusive remedy of any Indemnitee for any Damages that such Indemnitee may at any time suffer or incur, or become subject to, as a result of, or in connection with this Agreement, including any inaccuracy, violation or breach of any representation and warranty contained in this Agreement by any Party, or any failure by any Party to perform or comply with any covenant or agreement that, by its terms, was to have been performed, or complied with, under this Agreement, shall be indemnification in accordance with this Article VIII (subject to the applicable qualifications and limitations set forth in this Agreement).

ARTICLE IX.
TERMINATION

Section 9.01 Termination Prior to Closing. Notwithstanding any contrary provisions of this Agreement, this Agreement and the respective obligations of the Parties to consummate the transactions contemplated by this Agreement may be terminated and abandoned at any time before the Closing only as follows:

(a) upon the mutual written consent of Buyer and Seller; or

(b) by either Party, by written notice to the other Party if the Closing has not occurred on or before 11:59 p.m., Eastern Standard Time, on the date that is ninety (90) days following the Effective Date (the "***Outside Date***"); *provided*, *however*, that the right to terminate this Agreement under this Section 9.01(b) shall not be available to any Party whose material breach of any provision set forth in this Agreement is the primary cause of the failure of the Closing to occur on or before such date;

(c) by Buyer or Seller, if (i) any Legal Requirement having the effect referred to in Section 6.01(b) has been enacted, issued, promulgated, enforced or entered or (ii) any order, injunction or decree having the effect referred to in Section 6.01(b) is in effect and has become final and non-appealable;

(d) by Buyer, if Buyer is not in material breach of its obligations under this Agreement and there has been a violation or breach by Seller of any of its representations, warranties, covenants or other agreements contained in this Agreement, which has prevented or would prevent the satisfaction of any condition to the obligations of Buyer at the Closing set forth in Section 6.02, and (i) such violation or breach has not been waived by Buyer, (ii) Buyer has provided written notice to Seller of such violation or breach setting forth the allegations of violation or breach in reasonable detail, and (iii) such violation or breach cannot be or has not been cured by Seller within twenty (20) Business Days after receiving written notice thereof from Buyer (*provided*, that in no event shall such twenty (20) Business Day extend beyond the Outside Date); or

(e) by Seller, if Seller is not in material breach of its obligations under this Agreement and there has been a violation or breach by Buyer of any of its representations, warranties, covenants or other agreements contained in this Agreement, which has prevented or would prevent the satisfaction of any

condition to the obligations of Seller at the Closing set forth in Section 6.03 and (i) such violation or breach has not been waived by Seller, (ii) Seller has provided written notice to Buyer of such violation or breach setting forth the allegations of violation or breach in reasonable detail, and (iii) such violation or breach cannot be or has not been cured by Buyer within twenty (20) Business Days after receiving written notice thereof from Seller (*provided*, that in no event shall such twenty (20) Business Day extend beyond the Outside Date).

Section 9.02 Effect of Termination. In the event of the termination of this Agreement as provided in Section 9.01: (a) written notice thereof shall forthwith be given to the other Party specifying the provision hereof pursuant to which such termination is made, (b) this Agreement shall forthwith become null and void (except for the provisions of this Section 9.02, Article I and Article XI, which shall survive any such termination), and (c) there shall be no liability on the part of Buyer or Seller except for damages resulting from any breach of this Agreement prior to termination of this Agreement by Buyer or Seller.

ARTICLE X.
ADDITIONAL COVENANTS

Section 10.01 Further Assurances.

(a) The Parties shall cooperate reasonably with each other in connection with any steps required to be taken as part of their respective obligations under this Agreement, including without limitation any notifications or filings required to be made to the FDA in connection with the transfer of the Purchased Assets, and shall (i) furnish upon request to each other such further information, (ii) execute and deliver to each other such other documents, and (iii) do such other acts and things, all as the other Party may reasonably request for the purpose of carrying out the intent of this Agreement and the transactions contemplated by this Agreement, including the use by Buyer, its Affiliates or their respective successors and assigns of the Priority Review Voucher in accordance with its terms and applicable Legal Requirements.

(b) Without limiting the foregoing, Buyer and Seller agree to cooperate and assist each other with respect to all filings or notifications to any Governmental Entity related to the transfer and assignment of the Purchased Assets.

Section 10.02 Compliance with Legal Requirements. Following the Effective Date, Seller shall, and shall cause its Affiliates and shall require any successor in interest to or assignee of the product approved under the Subject BLA (as applicable) to, at all times comply with all Legal Requirements applicable to the Purchased Assets, including any and all Legal Requirements applicable to the validity, use or transfer of the Priority Review Voucher. Seller shall promptly forward to Buyer any communications or notices it or its Affiliates receive from any Governmental Entity in respect of or otherwise impacting the Purchased Assets; *provided*, that Seller may redact any portion of such written communications or other notices that is not relevant to the Priority Review Voucher.

Section 10.03 Marketing. Seller shall, and shall cause its Affiliates and shall require any successor in interest to or assignee of the product approved under the Subject BLA (as applicable) to, within the three hundred and sixty-five (365) day period beginning on the date of the FDA approval of the Subject BLA, market in the United States the product approved under the Subject BLA to the extent and in a manner required under applicable Legal Requirements to preclude the FDA from exercising its authority to revoke the Priority Review Voucher pursuant to 21 U.S.C. § 360ff(e)(1).

Section 10.04 Nondisclosure.

(a) Subject to disclosures permitted or contemplated by Section 10.05, with respect to Confidential Information received from a Party, the other Party will (i) keep such Confidential Information confidential, (ii) not use any such Confidential Information for any reason other than to carry out the intent and purpose of this Agreement, and (iii) not disclose any such Confidential Information to any Person, except in each case as otherwise expressly permitted by this Agreement or with the prior written consent of the disclosing Party.

(b) Each Party may disclose Confidential Information of the other Party only to its Representatives on a need-to-know basis.

(c) Each Party will (i) enforce the terms of this Section 10.04 as to its Representatives, (ii) take such action to the extent necessary to cause its Representatives to comply with the terms and conditions of this Section 10.04, and (iii) be responsible and liable for any breach of this Section 10.04 by it or its Representatives.

(d) If a Party becomes compelled by a court or is requested by a Governmental Entity to make any disclosure that is prohibited or otherwise constrained by this Section 10.04, such Party shall provide the disclosing Party with prompt notice of such compulsion or request so that it may seek an appropriate protective order or other appropriate remedy or waive compliance with the provisions of this Section 10.04. In the absence of a protective order or other remedy, the Party subject to the requirement to disclose may disclose that portion (and only that portion) of the Confidential Information that, based upon advice of its counsel, it is legally compelled to disclose or that has been requested by such Governmental Entity; *provided, however*, that such Party shall use reasonable efforts to obtain reliable assurance that confidential treatment will be accorded by any Person to whom any Confidential Information is so disclosed.

(e) Nothing herein shall prohibit or otherwise restrict the disclosure of any Confidential Information by or on behalf of Buyer or its Affiliates to the FDA or other Governmental Entity to the extent required by the FDA or such other Governmental Entity to enable the use or transfer of the Priority Review Voucher; *provided*, that Buyer, its Affiliates and their respective Representatives shall use commercially reasonable efforts to obtain confidential treatment for any such disclosures.

(f) The Parties hereby agree that, effective as of the Closing, the Confidentiality Agreement shall automatically terminate and be of no further force and effect. For the avoidance of doubt, during the period between the signing of this Agreement and the Closing, if there exists any conflict between the terms of the Confidentiality Agreement and this Agreement, the terms of the Confidentiality Agreement shall control.

Section 10.05 Disclosures Concerning this Agreement. The press release with respect to the execution of this Agreement that is attached as Exhibit F hereto shall be issued by Seller within four (4) Business Days following the Effective Date. Buyer and Seller agree not to (and to ensure that their respective Affiliates do not) issue any other press releases or public announcements concerning this Agreement, or that identifies the other Party as party to this Agreement or the acquiror of the Priority Review Voucher, without the prior written consent of the other Party (which consent shall not be unreasonably withheld, conditioned or delayed), except as required by a Governmental Entity or applicable Legal Requirement (including the rules and regulations of any stock exchange or trading market on which a Party's (or its parent entity's) securities are traded); *provided*, that the Party intending to disclose such information shall use reasonable efforts to provide the other Party with advance notice of such required disclosure, and an opportunity to review and comment on such proposed disclosure (which comments shall be considered in good faith by the disclosing Party). Notwithstanding the foregoing, without prior

submission to or approval of the other Party, either Party may issue press releases or public announcements which incorporate only such information concerning this Agreement as was included in a press release or public disclosure which was previously disclosed under the terms of this Agreement or which contains only non-material factual information regarding this Agreement. Buyer acknowledges that Seller, as a publicly traded company is legally obligated to make timely disclosures of material events relating to its business. Buyer acknowledges that Seller may be obligated to file a copy of this Agreement with the United States Securities and Exchange Commission; *provided*, that if Seller is obligated to so file a copy of this Agreement, Seller shall prepare a proposed redacted version thereof and request confidential treatment thereof, and Buyer may promptly provide its comments and additional proposed redactions, if any, thereon, which comments and proposed redactions, if any, shall be considered in good faith by Seller.

Section 10.06 Expenses. Whether or not the Asset Purchase and the other transactions contemplated by this Agreement are consummated, and except as otherwise expressly set forth in this Agreement, each of the Parties shall bear its own fees and expenses incurred or owed in connection with the purchase and sale of the Purchased Assets, this Agreement and the transactions contemplated hereby.

ARTICLE XI.
GENERAL PROVISIONS

Section 11.01 Survival. The representations and warranties of Seller and Buyer contained in this Agreement, and liability for the breach thereof, shall survive the Closing and shall remain in full force and effect for a period of eighteen (18) months following the Closing Date; *provided*, *however*, that (a) all covenants that by their terms were to be performed at or prior to the Closing and all Fundamental Representations and any claims for Fraud shall survive the Closing Date and remain in full force and effect until the later of (i) the date that is six (6) years after the Closing Date, and (ii) the expiration of the applicable statute of limitations, and (b) covenants which are by their terms to be performed following the Closing shall survive the Closing and remain in full force and effect until performed in accordance with their terms. Notwithstanding the foregoing, if written notice of a claim has been given in the manner required by Section 8.02 prior to the expiration of the applicable survival period by the Party seeking indemnification for such claim, then the relevant covenants, representations and warranties of the other Party shall survive as to such claim until such claim has been finally resolved pursuant to Article VIII.

Section 11.02 Transfer Taxes and Fees. Any and all sales, excise, use, value-added and similar taxes, fees or duties assessed or incurred by reason of the sale by Seller and the purchase by Buyer of the Purchased Assets hereunder, excluding VAT ("***Transfer Taxes**") shall be borne fifty percent (50%) by Buyer on the one hand, and fifty percent (50%) by Seller on the other hand, regardless of which Party such taxes, fees or duties are assessed against. The Party that is primarily responsible for the filing of any Tax return or other documentation with respect to Transfer Taxes shall promptly prepare and file such Tax return or documentation, as applicable, and the other Party shall provide such cooperation in connection therewith as may be reasonably requested by the filing Party. Buyer, its Affiliates, or any Buyer transferee of the Priority Review Voucher shall be solely responsible for the payment of the priority review fee described in 21 U.S.C. § 360ff(c) (the "***Priority Review Fee***") and all other user fees applicable to the human drug application for which the Priority Review Voucher is redeemed, following the Closing. For the avoidance of doubt, following the Closing, Seller shall have no liability or obligation for any such fees. Notwithstanding anything in this Agreement to the contrary, all sums and other consideration payable under this Agreement are exclusive of VAT, and an amount in respect of VAT shall be payable by the Buyer in addition upon receipt of a valid VAT invoice. If Seller is legally obliged to account to a relevant Tax Authority for any VAT chargeable in respect of any supply made by Seller to Buyer, Buyer shall pay or reimburse (or procure that the relevant Affiliate of Buyer shall pay or reimburse) such VAT, in addition to the amounts of consideration otherwise due or payable, at the rate in force at the time of the relevant supply or such other time as is stipulated under the applicable Legal Requirements following the receipt of a valid

invoice in respect of any such indirect Tax issued in the appropriate form by Seller, such indirect Taxes to be payable on the due date of the payment to which such indirect Tax relates. If any VAT originally paid or otherwise borne by Buyer is in whole or in part subsequently determined by a Tax Authority in writing to either Seller or Buyer not to have been chargeable (and if such determination is received by Buyer, Buyer shall provide a copy of such determination to Seller), then Seller shall use commercially reasonable efforts to receive a refund of such undue VAT from the applicable Tax Authority and any amount of such undue VAT repaid by such authority to Seller, less Seller's reasonable costs incurred in connection with obtaining such refund, will be transferred to Buyer within forty-five (45) days of receipt by Seller.

Section 11.03 Notices. Any notice or other communication required or permitted to be delivered to any Party shall be in writing and shall be deemed properly delivered, given and received: (a) when delivered by hand; (b) on the date sent by e-mail of a PDF document (with confirmation of transmission) if sent prior to 5:00 p.m. in the time zone of the intended recipient on a Business Day, and otherwise on the next Business Day or (c) upon such Party's receipt after being sent by registered mail, by courier or express delivery service; in any case to the address set forth beneath the name of such Party below (or to such other address as such Party shall have specified in a written notice given to the other Party in accordance with this Section 11.03):

(a) if to Buyer, to:

[***]

with a copy (which shall not constitute notice) to:

Covington & Burling LLP
30 Hudson Yards
New York, NY 10001
Attention: Stephen A. Infante
Email: [***]

(b) if to Seller, to:

Rocket Pharmaceuticals, Inc.
9 Cedarbrook Drive
Cranbury, NJ 08512
Attention: Gaurav Shah, MD, Chief Executive Officer
Martin Wilson, General Counsel & Chief Corporate Officer
Email: [***]
[***]

with a copy (which shall not constitute notice) to:

K&L Gates LLP
599 Lexington Avenue
New York, NY 10022
Attention: Whitney John Smith and Christopher Bozydaj
Email: [***] and [***]

Section 11.04 Construction.

(a) The Parties agree that any rule of construction to the effect that ambiguities are to be resolved against the drafting Party shall not be applied in the construction or interpretation of this Agreement.

(b) As used in this Agreement, the words "include" and "including," and variations thereof, shall not be deemed to be terms of limitation, but rather shall be deemed to be followed by the words "without limitation" and the word "or" is not intended to be exclusive unless expressly indicated otherwise. The words "will" and "shall" have the same meaning. "Extent" in the phrase "to the extent" means the degree to which a subject or other thing extends, and such phrase does not mean simply "if."

(c) The words "hereof," "herein" and "hereunder" and words of like import used in this Agreement shall refer to this Agreement as a whole and not to any particular provision of this Agreement. Except as otherwise indicated, (i) all references in this Agreement to "Articles," "Sections," "Schedules" or "Exhibits" are intended to refer to Articles, Sections, Schedules or Exhibits of this Agreement, and (ii) references in any Section to any clause are references to such clause of such Section.

(d) Except where the context otherwise requires, wherever used, the singular shall include the plural, the plural the singular, the use of any gender shall be applicable to all genders and the word "or" is used in the inclusive sense (and/or).

(e) Whenever this Agreement refers to a number of days, unless otherwise specified, such number refers to calendar days.

(f) The captions, table of contents and headings in this Agreement are for convenience of reference only and in no way define, describe, extend, or limit the scope or intent of this Agreement or the intent of any provision contained in this Agreement.

(g) Unless otherwise specified, (i) references to any applicable law or other Legal Requirement shall be deemed to refer to such law or Legal Requirement as amended from time to time and to any rules, regulations or interpretations promulgated thereunder and (ii) references to any agreement or Contract are to that agreement or Contract as amended, modified, supplemented, extended or renewed from time to time in accordance with the terms hereof and thereof.

Section 11.05 Counterparts. This Agreement may be executed in two or more counterparts, all of which shall be considered one and the same instrument, and shall become effective when one or more counterparts have been signed by each of the Parties and delivered to the other Party, it being understood that all Parties need not sign the same counterpart. Each Party agrees that this Agreement and any other documents to be delivered in connection herewith may be electronically signed, and that any electronic signatures appearing on this Agreement or such other documents are the same as handwritten signatures for the purposes of validity, enforceability, and admissibility. The exchange of a fully executed Agreement (in counterparts or otherwise) by electronic transmission shall be sufficient to bind the Parties to the terms and conditions of this Agreement.

Section 11.06 Entire Agreement. This Agreement, including all exhibits and schedules attached hereto, and the Confidentiality Agreement set forth the entire understanding of the Parties relating to the subject matter hereof and supersede all prior agreements and understandings among or between the Parties relating to the subject matter hereof.

Section 11.07 Assignment. No Party will have the right to assign this Agreement, in whole or in part, by operation of law or otherwise, without the other Party's express prior written consent. Any attempt to assign this Agreement without such consent, will be null and void. Notwithstanding the foregoing, any Party may assign this Agreement, in whole or in part, without the consent of the other Party: (a) to a Third Party that succeeds to all or substantially all of its assets or business related to this Agreement (whether by sale, merger, operation of law or otherwise); or (b) to an Affiliate of such Party. Notwithstanding the foregoing, Buyer may assign this Agreement, in whole or in part, without Seller's consent, to any purchaser, transferee, or assignee of any of the Purchased Assets. For the avoidance of doubt, no assignment made pursuant to this Section 11.07 shall relieve the assigning Party of any of its obligations under this Agreement. Subject to the foregoing, this Agreement will bind and inure to the benefit of each Party's successors and permitted assigns.

Section 11.08 Severability. If any provision of this Agreement, or the application thereof, becomes or is declared by a court of competent jurisdiction to be illegal, void or unenforceable, the remainder of this Agreement shall continue in full force and effect and shall be interpreted so as reasonably to effect the intent of the Parties. The Parties shall use commercially reasonable efforts to replace such void or unenforceable provision of this Agreement with a valid and enforceable provision that shall achieve, to the extent possible, the economic, business and other purposes of such void or unenforceable provision.

Section 11.09 Remedies Cumulative. Except as otherwise provided herein, any and all remedies herein expressly conferred upon a Party shall be deemed cumulative with and not exclusive of any other remedy conferred hereby or by law or equity upon such Party, and the exercise by a Party of any one remedy shall not preclude the exercise of any other remedy and nothing in this Agreement shall be deemed a waiver by any Party of any right to specific performance or injunctive relief. The Parties agree that irreparable harm would occur in the event that the transactions contemplated hereby are not consummated in accordance with the terms of this Agreement, and that money damages or other legal remedies would not be an adequate remedy for any such harm. Accordingly, the Parties acknowledge and hereby covenant and agree that in the event of any breach or threatened breach of the covenants, agreements, or obligations set forth in this Agreement, then in addition to any other remedy available at law or in equity, the non-breaching Party will be entitled to seek an injunction or injunctions to prevent or restrain any breaches or threatened breaches of this Agreement, and to specifically enforce the terms and provisions of this Agreement to enforce compliance with the covenants, agreements, and obligations under this Agreement. Each Party hereby covenants and agrees not to raise, and irrevocably waives, any objections to the availability of such relief that a remedy at law would be adequate and that a bond or other security will be required.

Section 11.10 Governing Law. This Agreement shall be governed by, and construed in accordance with, the laws of New York, regardless of the laws that might otherwise govern under applicable principles of conflicts of law. The Parties irrevocably and unconditionally submit to the exclusive jurisdiction of the state and federal courts in New York solely and specifically for the purposes of any action or proceeding arising out of or in connection with this Agreement.

Section 11.11 WAIVER OF JURY TRIAL. EACH PARTY, TO THE EXTENT PERMITTED BY LAW, KNOWINGLY, VOLUNTARILY, AND INTENTIONALLY WAIVES ITS RIGHT TO A TRIAL BY JURY IN ANY ACTION OR OTHER LEGAL PROCEEDING ARISING OUT OF OR RELATING TO THIS AGREEMENT AND THE TRANSACTIONS IT CONTEMPLATES. THIS WAIVER APPLIES TO ANY ACTION OR LEGAL PROCEEDING, WHETHER SOUNDING IN CONTRACT, TORT, OR OTHERWISE.

Section 11.12 Amendment; Extension; Waiver. Subject to the provisions of applicable Legal Requirements, the Parties may amend this Agreement at any time pursuant to an instrument in writing signed on behalf of each of the Parties. At any time, any Party may, to the extent legally allowed, (a) extend

the time for the performance of any of the obligations or other acts of the other Party, (b) waive any inaccuracies in the representations and warranties made to such Party contained herein or (c) waive compliance with any of the agreements or conditions for the benefit of such Party contained herein. Any agreement on the part of a Party to any such extension or waiver shall be valid only if set forth in an instrument in writing signed on behalf of such Party. Without limiting the generality or effect of the preceding sentence, no delay in exercising any right under this Agreement shall constitute a waiver of such right, and no waiver of any breach or default shall be deemed a waiver of any other breach or default of the same or any other provision in this Agreement.

Section 11.13 Representation By Counsel; Interpretation. Seller and Buyer each acknowledge that it has been represented by its own legal counsel in connection with this Agreement and the transactions contemplated by this Agreement. Accordingly, any rule of law, or any legal decision that would require interpretation of any claimed ambiguities in this Agreement against the Party that drafted it, has no application and is expressly waived.

[SIGNATURE PAGE FOLLOWS]

IN WITNESS WHEREOF, this Agreement has been executed on behalf of each of the parties hereto as of the date first above written.

[***]

By: /s/ [***]
Name: [***]
Title: [***]

IN WITNESS WHEREOF, this Agreement has been executed on behalf of each of the parties hereto as of the date first above written.

ROCKET PHARMACEUTICALS, INC.

By: /s/ Martin Wilson
Name: Martin Wilson
Title: General Counsel and Chief Corporate Officer

EXHIBIT A

APPROVAL LETTER

[TO BE ADDED]

EXHIBIT B

FORM OF BILL OF SALE

EXHIBIT C

FDA COVER LETTER

EXHIBIT D

SELLER'S TRANSFER ACKNOWLEDGMENT LETTER

EXHIBIT E

BUYER'S TRANSFER ACKNOWLEDGMENT LETTER

EXHIBIT F

PRESS RELEASE

Schedule 4.11

Document Disclosure

Exhibit 31.1

CERTIFICATIONS

I, Gaurav Shah, MD, certify that:

1. I have reviewed this quarterly report on Form 10-Q for the period ended June 30, 2026 of Rocket Pharmaceuticals, Inc.;

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

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

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

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

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

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

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

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

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

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

Date: August 10, 2026

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 June 30, 2026 of Rocket Pharmaceuticals, Inc.;

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

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

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

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

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

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

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

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

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

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

Date: August 10, 2026

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

Exhibit 32.1

CERTIFICATION PURSUANT TO

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

SECTION 906 OF THE SARBANES-OXLEY ACT OF 2002

In connection with the quarterly report on Form 10-Q of Rocket Pharmaceuticals, Inc. (the "Company") for the period ended June 30, 2026, as filed with the United States Securities and Exchange Commission on the date hereof (the "Report"), each of the undersigned officers hereby certifies, pursuant to Section 906 of the Sarbanes-Oxley Act of 2002, 18 U.S.C. Section 1350, that to his knowledge:

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

Date: August 10, 2026

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

Date: August 10, 2026

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