Rocket Pharmaceuticals Reports Second Quarter 2024 Financial Results and Highlights Recent Progress

August 5, 2024

Enrollment of patients ongoing in the pivotal Phase 2 study of RP-A501 for the treatment of Danon disease and the Phase 1 study of RP-A601 to treat PKP2 arrhythmogenic cardiomyopathy

Working toward FDA-approval of KRESLADI for severe LAD-I; Commercial infrastructure and capabilities in place for launch

Presented long-term KRESLADI ™ follow-up data from the global Phase 1/2 study for severe LAD-I, results from the global Phase 1/2 study of RP-L102 for Fanconi Anemia, and data from the Phase 1 study of RP-L301 for PKD at ASGCT in May

Cash, cash equivalents and investments of approximately $278.8M; expected operational runway into 2026

CRANBURY, N.J.--(BUSINESS WIRE)--Aug. 5, 2024-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a fully integrated, late-stage biotechnology company advancing a sustainable pipeline of genetic therapies for rare disorders with high unmet need, today reported financial and recent operational results for the quarter ending June 30, 2024.

“Over the quarter, Rocket has been advancing its clinical pipeline as we progressed our RP-A501 and RP-A601 cardiac programs, targeting Danon disease and PKP2-ACM, and continued to actively enroll patients,” said Gaurav Shah, M.D., Chief Executive Officer, Rocket Pharmaceuticals. “At ASGCT, we shared follow-up data from across our hematology portfolio including 4-year follow-up data for KRESLADI to treat patients with severe LAD-I, demonstrating a 100% survival rate. In parallel, we have been preparing for the anticipated FDA approval of KRESLADI.”

Recent Pipeline and Operational Updates

- **Continued advancement of Phase 2 pivotal study of RP-A501 for Danon Disease**.
  - Enrollment in the Phase 2 pivotal study of RP-A501 to treat Danon Disease is actively progressing. Details of the Phase 2 study can be found at www.ClinicalTrials.gov under NCT identifier NCT06092034.
- **Granted orphan medicinal product designation from the European Commission (EC) for RP-A601 for PKP2 arrhythmogenic cardiomyopathy (ACM).**
  - In May, Rocket announced orphan medicinal product designation from the EC for RP-A601 for the treatment of PKP2-ACM. Enrollment in the Phase 1 study is ongoing. Details of the study can be found at www.ClinicalTrials.gov under NCT identifier NCT05885412.
  - Orphan medicinal product designation by the EC is available to novel therapeutics that prevent or treat life-threatening or chronically debilitating conditions that affect fewer than five in 10,000 persons in the European Union (EU). The designation qualifies for financial and regulatory benefits including protocol assistance from the European Medicines Agency during clinical development, access to centralized marketing authorization, and a 10-year period of marketing exclusivity after product approval.
- **U.S. Food and Drug Administration (FDA) review of limited additional Chemistry Manufacturing and Controls (CMC) information underway for KRESLADI ™ for the treatment of severe leukocyte adhesion deficiency-I (LAD-I).**
  - In June, Rocket announced that the FDA requested limited additional CMC information to complete its review of KRESLADI ™ (marnetegragene autotemcel; marne-cel) to treat severe LAD-I. The Company is working with senior leaders and reviewers from the FDA’s Center for Biologics Evaluation and Research to support the approval of KRESLADI ™.
  - Long-term KRESLADI ™ follow-up data from the global Phase 1/2 study were presented at the American Society of

Gene and Cell Therapy (ASGCT) 27th Annual Meeting. Data demonstrated survival of 100% in the absence of allogeneic hematopoietic stem cell transplantation from 18 to 45 months with a well-tolerated safety profile in all nine patients with severe LAD-I.

- **Progressed Fanconi Anemia (FA) program through regulatory and clinical milestones.**
  - Regulatory filings and review for RP-L102 for the treatment of FA are on track with health authorities in the U.S. and Europe.
  - Results from the global Phase 1/2 study of RP-L102 were presented at the ASGCT 27th Annual Meeting. Previously disclosed data demonstrated genetic and phenotypic correction combined with hematologic stabilization extending to 42 months with polyclonal integration patterns.

- **Furthered Pyruvate Kinase Deficiency (PKD) program through clinical milestones.**
  - Updated data from the Phase 1 study of RP-L301 for PKD were presented at the ASGCT 27th Annual Meeting. Sustained and clinically meaningful hemoglobin improvement and well-tolerated safety profile were observed in PKD patients up to 36 months after RP-L301 treatment.
  - The global Phase 2 pivotal study of RP-L301 for PKD has been initiated. Details of the Phase 2 study can be found at www.ClinicalTrials.gov under NCT identifier NCT06422351.

- **Progressing BAG3-associated dilated cardiomyopathy preclinical program.**
  - Nonclinical, IND-enabling studies are ongoing.

Second Quarter Financial Results

- **Cash position.** Cash, cash equivalents and investments as of June 30, 2024, were $278.8 million.
- **R&D expenses.** Research and development expenses were $91.6 million for the six months ended June 30, 2024, compared to $97.8 million for the six months ended June 30, 2023. The decrease of $6.2 million in R&D expenses was driven by decreases in manufacturing and development and direct costs of $14.9 million. The decreases were partially offset by increases in the costs for compensation and benefits of $1.2 million due to increased R&D headcount, professional fees of $3.4 million, laboratory supplies of $0.6 million, non-cash stock compensation expense of $1.1 million, and clinical trial costs of $1.4 million.
- **G&A expenses.** General and administrative expenses were $49.5 million for the six months ended June 30, 2024, compared to $33.2 million for the six months ended June 30, 2023. The increase in G&A expenses was primarily driven by increased commercial preparation expenses which consists of commercial strategy, medical affairs, market development and pricing analysis of $9.5 million, legal expenses of $3.3 million, and non-cash stock compensation expense of $1.4 million.
- **Net loss.** Net loss was $131.7 million or $1.40 per share (basic and diluted) for the six months ended June 30, 2024, compared to $124.0 million or $1.55 (basic and diluted) for the six months ended June 30, 2023.
- **Shares outstanding.** 90,956,613 shares of common stock were outstanding as of June 30, 2024.

Financial Guidance

- **Cash position.** As of June 30, 2024, Rocket had cash, cash equivalents and investments of $278.8 million. Rocket expects such resources will be sufficient to fund its operations into 2026, including producing AAV cGMP batches at the Company's Cranbury, N.J. R&D and manufacturing facility and continued development of its six clinical and/or preclinical programs.

About Rocket Pharmaceuticals, Inc.
Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) is a fully integrated, late-stage biotechnology company advancing a sustainable pipeline of investigational genetic therapies designed to correct the root cause of complex and rare disorders. Rocket's innovative multi-platform approach allows us to design the optimal gene therapy for each indication, creating potentially transformative options that enable people living with devastating rare diseases to experience long and full lives.

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

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

For more information about Rocket, please visit www.rocketpharma.com and follow us on LinkedIn, YouTube, and X.

Rocket Cautionary Statement Regarding Forward-Looking Statements
This press release contains forward-looking statements concerning Rocket's future expectations, plans and prospects 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 release are forward-looking statements. You should not place reliance on these forward-looking statements, which often include words such as "could," "believe," "expect," "anticipate," "intend," "plan," "will give," "estimate," "seek," "will," "may," "suggest" or similar terms, variations of such terms or the negative of those terms. These forward-looking statements include, but are not limited to, statements concerning Rocket's expectations regarding the safety and effectiveness of product candidates that Rocket is developing to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Danon Disease (DD) and other diseases, the expected timing and data readouts of Rocket's ongoing and planned clinical trials, the expected timing and outcome of Rocket's regulatory interactions and planned submissions, including the timing and outcome of the FDA's review of the additional CMC information that Rocket will provide in response to the FDA's request, the safety, effectiveness and timing of pre-clinical studies and clinical trials, Rocket's ability to establish key collaborations and vendor relationships for its product candidates, Rocket's ability to develop sales and marketing capabilities or enter into agreements with third parties to sell and market its product candidates, Rocket's ability to expand its pipeline to target additional indications that are compatible with its gene therapy technologies, Rocket's ability to transition to a commercial stage pharmaceutical company, and Rocket's expectation that its cash, cash equivalents and investments will be sufficient to funds its operations into 2026. Although Rocket believes that the expectations reflected in the forward-looking statements are reasonable, Rocket cannot guarantee such outcomes. Actual results may differ materially from those indicated by these forward-looking statements as a result of various important factors, including, without limitation, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, unexpected expenditures, Rocket's competitors' activities, including decisions as to the timing of competing product launches, pricing and discounting, Rocket's ability to develop, acquire and advance product candidates into, enroll a sufficient number of patients into, and successfully complete, clinical studies, the integration of new executive team members and the effectiveness of the newly configured corporate leadership team, Rocket's ability to acquire additional businesses, form strategic alliances or create joint ventures and its ability to realize the benefit of such acquisitions, alliances or joint ventures, Rocket's ability to obtain and enforce patents to protect its product candidates, and its ability to successfully defend against unforeseen third-party infringement claims, as well as those risks more fully discussed in the section entitled "Risk Factors" in Rocket's Annual Report on Form 10-K for the year ended December 31, 2023, filed February 27, 2024 with the SEC and subsequent filings with the SEC including our Quarterly Reports on Form 10-Q. Accordingly, you should not place undue reliance on these forward-looking statements. All such statements speak only as of the date made, and Rocket undertakes no obligation to update or revise publicly any forward-looking statements, whether as a result of new information, future events or otherwise.

	Three Months Ended June 30,		Six Months Ended June 30,	
	2024	2023	2024	2023
Operating expenses:				
Research and development	$ 46,345	$ 51,383	$ 91,572	$ 97,754
General and administrative	27,367	17,374	49,515	33,197
Total operating expenses	73,712	68,757	141,087	130,951
Loss from operations	(73,712)	(68,757)	(141,087)	(130,951)
Interest expense	(471)	(468)	(942)	(936)
Interest and other income, net	2,294	846	5,323	2,754
Accretion of discount on investments, net	2,243	2,678	5,006	5,097
Net loss	$ (69,646)	$ (65,701)	$ (131,700)	$ (124,036)
Net loss per share - basic and diluted	$ (0.74)	$ (0.82)	$ (1.40)	$ (1.55)
Weighted-average common shares outstanding - basic and diluted	93,746,243	80,472,362	93,759,894	79,965,755

	June 30, 2024	December 31, 2023
Cash, cash equivalents, and investments	$ 278,825	$ 407,495
Total assets	446,411	566,341
Total liabilities	61,776	73,767
Total stockholders' equity	384,635	492,574

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