Rocket Pharmaceuticals Reports Third Quarter 2023 Financial Results and Highlights Recent Progress

November 6, 2023

Agreement reached with FDA on Phase 2 pivotal study design of RP-L301 for PKD to support accelerated approval; Initiating single-arm, 10-patient pivotal study with primary endpoint of ≥1.5 point Hgb improvement at 12 months

Initiated Phase 2 pivotal trial of RP-A501 for Danon Disease following FDA alignment

Initiated Phase 1 study of RP-A601 for PKP2-ACM

FDA accepted BLA with Priority Review for KRESLADI™ (marnetegragene autotemcel) for severe LAD-I; PDUFA target action date is March 31, 2024

Product filings for RP-L102 for Fanconi Anemia anticipated in the first half of 2024 in the U.S. and Europe

Cash, cash equivalents and investments of approximately $437.2M; expected operational runway through 2025

CRANBURY, N.J.--(BUSINESS WIRE)--Nov. 6, 2023-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a leading late-stage biotechnology company advancing an integrated and sustainable pipeline of genetic therapies for rare disorders with high unmet need, today reported financial results for the quarter ending September 30, 2023, and updates from the Company's key pipeline developments, business operations and upcoming milestones.

"I'm very pleased with Rocket's results this quarter, as we realized significant regulatory milestones across our pipeline, including reaching FDA alignment on the study design for the 10-patient Phase 2 pivotal trial of RP-L301 for the treatment of severe PKD and securing the FDA's acceptance of Rocket's BLA for RP-L201, now named KRESLADI ™, for the treatment of severe LAD-I," said Gaurav Shah, M.D., Chief Executive Officer, Rocket Pharma. "At the same time, we initiated the Phase 2 pivotal trial of RP-A501 for Danon Disease following FDA alignment and the Phase 1 study of RP-A601 for PKP2-ACM, which move us substantially closer to delivering potential one-time, curative therapies to patients facing these two devastating cardiac diseases."

Dr. Shah continued, "As we bring 2023 to a close, we have made remarkable progress across the breadth of our AAV cardiovascular and LV hematology portfolios. Four programs have now reached pivotal studies, one of which is under review for approval, thanks to strong collaboration with health authorities and scientific partners, as we collectively seek to fulfill the high unmet needs of patients with rare and life-threatening diseases."

Key Pipeline and Operational Updates

AAV Cardiovascular Portfolio

Danon Disease

- **Initiated Phase 2 pivotal trial of RP-A501 for Danon Disease following FDA alignment.** The global Phase 2 multi-center trial is a single arm study with an external comparator arm and includes 12 male patients with Danon Disease. The trial is evaluating the safety and efficacy of RP-A501 at a dose level of 6.7 x 10^{13} GC/kg and includes a pediatric safety run-in (n=2). The co-primary endpoint is composed of *LAMP2* protein expression and left ventricular (LV) mass index and will be assessed at 12 months for accelerated approval.

- **Supported peer-reviewed expert consensus paper to increase awareness of diagnosis and clinical management of patients with Danon Disease**. "International Consensus on Differential Diagnosis and Management of Patients with Danon Disease: JACC State-of-the-Art Review" was published in the *Journal of the American College of Cardiology*. Highlights of the publication include a review of diagnosing Danon Disease emphasizing the importance of genetic testing upon clinical suspicion, natural history, management recommendations and recent advances in potential gene therapy treatment.

- **Filed Clinical Trial Application (CTA)/Investigational Medicinal Product Dossier (IMPD) for RP-A501 to EMA and MHRA**. Rocket is working towards initiation of Phase 2 pivotal trial activities in Europe and the UK.

PKP2 Arrhythmogenic Cardiomyopathy (PKP2-ACM)

- **Initiated Phase 1 trial of RP-A601.** The multi-center Phase 1, dose escalation trial will evaluate the safety and preliminary efficacy of RP-A601 in at least six adult PKP2-ACM patients with ICDs and overall high risk for arrhythmias. The study will assess the impact of RP-A601 on PKP2 myocardial protein expression, cardiac biomarkers, and clinical predictors of life-threatening ventricular arrhythmias and sudden cardiac death. Patients in the dose-escalation trial will receive a single dose of RP-A601. The starting dose will be 8 x 10^{13} GC/kg.

LV Hematology Portfolio

Leukocyte Adhesion Deficiency-I (LAD-I)

- **BLA accepted by FDA with Priority Review for KRESLADI™ (marnetegragene autotemcel).** Based on the positive top-line efficacy and safety data from the global Phase 1/2 study, the FDA accepted the BLA and also granted Priority Review for KRESLADI™, also known as RP-L201. This represents Rocket's first product filing and is a significant milestone for both the Company and patients whose only current available treatment option is bone marrow transplant, which has substantial morbidity and mortality, and may not be available in time. The PDUFA date set by the FDA is March 31, 2024.

Fanconi Anemia (FA)

- **Product filings for RP-L102 anticipated in the first half of 2024 in the U.S. and Europe**. Rocket is finalizing the CMC package with the FDA.

Pyruvate Kinase Deficiency (PKD)

- **Reached agreement with FDA on study design of Phase 2 pivotal trial of RP-L301.** Based on positive safety and efficacy data from the Phase 1 study, Rocket has aligned with the FDA on the pivotal study design to support accelerated approval and is initiating a 10-patient, single-arm Phase 2 pivotal trial with a primary endpoint of ≥1.5 point Hgb improvement at 12 months.

- **Presented positive updated clinical data from Phase 1 trial of RP-L301 at the ESGCT Annual Meeting.** Updated data demonstrated sustained efficacy in both adult patients with up to 36 months follow-up. Hemoglobin improvement relative to pre-treatment baseline was observed in both patients in the pediatric cohort with up to six months of available follow-up. Results also indicated favorable safety profile for RP-L301 in all four patients treated in the Phase 1 study.

Operational Updates

- **Published peer-reviewed risk assessment demonstrating low incidence of busulfan-related secondary malignancies in pediatric transplant recipients**. "Busulfan and Subsequent Malignancy: An Evidence-based Risk Assessment" was published in *Pediatric Blood & Cancer*. Busulfan is utilized as conditioning prior to gene therapy in nonmalignant hematologic and related disorders. Researchers conducted a literature-based assessment of busulfan and subsequent late effects, concluding the incidence of busulfan-related secondary malignancies has been less than 1% in pediatric transplant recipients, and that the incidence of secondary cancers in pediatric patients receiving single-agent busulfan in non-malignant settings is likely to be substantially lower than 1%.

- **Raised net proceeds of $188.9M through public offering**. On September 15, 2023, the Company completed a public offering of approximately 9.5 million shares of our common stock at a public offering price of $16.00 per share and pre-funded warrants to purchase 3.1 million shares of common stock at a price of $15.99 per warrant. The gross proceeds to Rocket from the public offering were approximately $201.3 million, net of $12.4 million of offering costs, commissions, legal and other expenses for net proceeds from the offering of $188.9 million.

Upcoming Investor Conferences

- Stifel Healthcare Conference 2023, November 15
- 6th Annual Evercore ISI HealthCONx Conference 2023, November 30

Third Quarter Financial Results

- **Cash position**. Cash, cash equivalents and investments as of September 30, 2023, were $437.2 million.
- **R&D expenses**. Research and development expenses were $46.8 million for the three months ended September 30, 2023, compared to $43.4 million for the three months ended September 30, 2022. The increase in R&D expenses was

primarily driven by increases in compensation and benefits expenses of $4.5 million due to increased R&D headcount, clinical trial expenses of $7.4 million, license expenses of $2.2 million, and non-cash stock-based compensation of $1.6 million. Increases noted were offset by decreases in manufacturing and development costs of $9.0 million and cost of direct materials of $3.3 million.

- **G&A expenses**. General and administrative expenses were $18.6 million for the three months ended September 30, 2023, compared to $15.1 million for the three months ended September 30, 2022. The increase in G&A expenses was primarily driven by increases in commercial preparation expenses which consists of commercial strategy, medical affairs, market development and pricing analysis of $2.5 million and non-cash stock compensation expense of $1.0 million, partially offset by reduction in acquisition related expenses of $1.3 million.
- **Net loss**. Net loss was $61.9 million or $0.75 per share (basic and diluted) for the three months ended September 30, 2023, compared to $57.8 million or $0.87 per share (basic and diluted) for the three months ended September 30, 2022.
- **Shares outstanding**. 90,146,602 shares of common stock were outstanding as of September 30, 2023.

Financial Guidance

- **Cash position.** As of September 30, 2023, Rocket had cash, cash equivalents and investments of $437.2 million. Rocket expects such resources will be sufficient to fund its operations through 2025, 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 advancing an integrated and sustainable pipeline of investigational genetic therapies designed to correct the root cause of complex and rare disorders. The Company's platform-agnostic approach enables it to design the best therapy for each indication, creating potentially transformative options for patients afflicted with rare genetic diseases. Rocket's clinical programs using lentiviral (LV) vector-based gene therapy are for the treatment of Fanconi Anemia (FA), a difficult to treat genetic disease that leads to bone marrow failure 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 rare, monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia. Rocket's first clinical program using adeno-associated virus (AAV)-based gene therapy is for Danon Disease, a devastating, pediatric heart failure condition. Rocket also is developing AAV-based gene therapy programs in PKP2-arrhythmogenic cardiomyopathy (ACM) and BAG3-associated dilated cardiomyopathy (DCM). For more information about Rocket, please visit www.rocketpharma.com.

Rocket Cautionary Statement Regarding Forward-Looking Statements

Various statements in this release concerning Rocket's future expectations, plans and prospects, including without limitation, 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, Rocket's plans for the advancement of its Danon Disease program, including its planned pivotal trial, and the safety, effectiveness and timing of related pre-clinical studies and clinical trials, may constitute forward-looking statements for the purposes of the safe harbor provisions under the Private Securities Litigation Reform Act of 1995 and other federal securities laws and are subject to substantial risks, uncertainties and assumptions. You should not place reliance on these forward-looking statements, which often include words such as "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. 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 ability to monitor the impact of COVID-19 on its business operations and take steps to ensure the safety of patients, families and employees, the interest from patients and families for participation in each of Rocket's ongoing trials, our expectations regarding the delays and impact of COVID-19 on clinical sites, patient enrollment, trial timelines and data readouts, our expectations regarding our drug supply for our ongoing and anticipated trials, actions of regulatory agencies, which may affect the initiation, timing and progress of pre-clinical studies and clinical trials of its product candidates, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, and unexpected expenditures, 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, 2022, filed February 28, 2023 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 September 30,		Nine Months Ended September 30,	
	2023	2022	2023	2022
Operating expenses:				
Research and development	$ 46,844	$ 43,383	$ 144,598	$ 115,533
General and administrative	18,585	15,105	51,782	39,728
Total operating expenses	65,429	58,488	196,380	155,261
Loss from operations	(65,429)	(58,488)	(196,380)	(155,261)
Interest expense	(469)	(465)	(1,405)	(1,395)
Interest and other income, net	1,720	1,353	4,474	2,644
Accretion of discount and amortization of premium on investments, net	2,279	(156)	7,376	(1,128)

Net loss	$ (61,899)	$ (57,756)	$ (185,935)	$ (155,140)
Net loss per share - basic and diluted	$ (0.75)	$ (0.87)	$ (2.30)	$ (2.37)
Weighted-average common shares outstanding - basic and diluted	82,636,120	66,215,535	80,865,658	65,406,844

	September 30, 2023	December 31, 2022
Cash, cash equivalents, and investments	$ 437,171	$ 399,670
Total assets	598,762	551,807
Total liabilities	57,676	62,121
Total stockholders' equity	541,086	489,686

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