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

August 10, 2023

Initiating enrollment of two-patient pediatric safety run-in for pivotal single-arm Phase 2 study of RP-A501 (Danon Disease); finalizing primary composite endpoint with FDA to support accelerated approval

RP-L201 (LAD-I) BLA submitted to FDA; represents Rocket's first product filing

RP-A601 (PKP2-ACM) advancing towards first patient treatment in Phase 1 study following FDA clearance of IND; received FDA Fast Track and Orphan Drug designations

RP-L102 (Fanconi Anemia) U.S. pivotal Phase 2 study completed; product filing on track

RP-L301 (PKD) granted EMA PRIME and FDA RMAT designations

Cash, cash equivalents and investments of approximately $307M; expected operational runway into the first half of 2025

CRANBURY, N.J.--(BUSINESS WIRE)--Aug. 10, 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 June 30, 2023, and updates from the Company's key pipeline developments, business operations and upcoming milestones.

"The second quarter of 2023 marked a period of strong forward momentum across our cardiovascular AAV and hematology LV portfolios highlighted by the BLA submission for our LAD-I program, Rocket's first product filing. In addition, we are in advanced discussions with the FDA to finalize the components of the primary composite endpoint for the single-arm, pivotal Phase 2 Danon Disease study. In parallel, we are initiating enrollment in the trial following recent alignment with the FDA on our proposed two-patient pediatric safety run-in," said Gaurav Shah, M.D., Chief Executive Officer, Rocket Pharma. "We appreciate the strong and positive collaboration with the FDA afforded by our RMAT designation, which we believe will lead to the most optimal global development path forward for the first pivotal gene therapy trial for a cardiac condition. Simultaneously, in our second cardiac program, RP-A601 for the treatment of PKP2-ACM, we are rapidly moving towards first patient treatment following IND clearance and have also received FDA Fast Track and Orphan Drug designations."

Dr. Shah continued, "Building on the BLA submission for LAD-I, we remain on track to submit the BLA for our LV-based Fanconi Anemia program in the fourth quarter. Taken together, I am very pleased with our progress across our entire pipeline of AAV and LV assets and look forward to continuing our momentum through the remainder of the year and beyond."

Key Pipeline and Operational Updates

AAV Cardiovascular Portfolio

Danon Disease

- **Received European designations including Priority Medicines (PRIME) and Advanced Therapy Medicinal Products (ATMP) for RP-A501.** PRIME designation offers the benefits of early and enhanced support from the European Medicines Agency (EMA) and the opportunity for accelerated marketing application review. It is granted to medicines that target an unmet need for which no treatment option exists and with data showing a meaningful improvement of clinical outcomes. ATMP designation is intended for medicines that offer groundbreaking new opportunities and allows for a single evaluation and authorization procedure.
- **Positive clinical data from the Phase 1 trial of RP-A501 presented at the ASGCT Annual Meeting.** As of May 2023, all six optimally enrolled patients in the Danon Disease Phase 1 trial continued to demonstrate improvement or stabilization in ongoing follow-up of six to 36 months. Patients would have typically experienced progressive disease or death without treatment.

- **Initiating two-patient pediatric safety run-in for RP-A501 pivotal study; approaching final alignment with FDA on primary composite endpoint to support accelerated approval.** Rocket has obtained IRB approvals and begun screening activities. Initial clinical sites are the University of California, San Diego (UCSD) and Children's Hospital of Philadelphia.

PKP2 Arrhythmogenic Cardiomyopathy (PKP2-ACM)

- **Received FDA Fast Track and Orphan Drug designations for RP-A601**. Fast Track designation facilitates the development and expedited review of medicines that treat serious conditions and fill an unmet medical need. It also enables increased communication with the FDA, offering the potential for accelerated approval and priority review if criteria are met, and permits a rolling Biologics License Application (BLA) review. Orphan Drug designation is granted to support the development of medicines for rare disorders and provides certain benefits, including market exclusivity upon regulatory approval, exemption of FDA application fees, and tax credits for qualified clinical trials.
- **Positive proof of concept from preclinical studies of RP-A601 presented at the ASGCT Annual Meeting.** Data demonstrated decreased arrhythmias and increased survival in the PKP2 knockout mouse model. Based on these data and the completion of Investigational New Drug (IND)-enabling toxicology studies, Rocket received IND clearance from the FDA for a Phase 1 study of RP-A601 that 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.
- **Phase 1 start-up activities for RP-A601 are well progressed following IND clearance to allow for first patient treatment.** Rocket has gained IRB approval at UCSD, initiated Phase 1 study start up activities and is rapidly advancing toward first patient treatment.

LV Hematology Portfolio

Leukocyte Adhesion Deficiency-I (LAD-I)

- **Positive clinical data from pivotal Phase 2 trial of RP-L201 presented at the ASGCT Annual Meeting.** Positive, updated top-line data demonstrated 100% overall survival at 12 months post-infusion (and for entire duration of follow-up) via Kaplan Meier estimate for all nine LAD-I patients with 12 to 24 months of available follow-up. Data also showed evidence of resolution of LAD-I-related skin rash and restoration of wound repair capabilities. The safety profile was highly favorable in all patients with no RP-L201-related serious adverse events.
- **BLA submitted to FDA for RP-L201.** Based on the positive efficacy and safety data from the pivotal study of RP-L201, Rocket submitted the BLA to the FDA, as its first product filing.

Fanconi Anemia (FA)

- **Positive clinical data from pivotal Phase 2 trial of RP-L102 presented at the ASGCT Annual Meeting.** Positive, updated top-line data showed sustained genetic correction in eight of 12 evaluable patients and comprehensive phenotypic correction in seven of 12 evaluable patients with ≥12 months of follow up as demonstrated by increased resistance to mitomycin-C (MMC) in bone marrow (BM)-derived colony forming cells and hematologic stabilization. The safety profile was highly favorable with no significant safety signals, and the treatment, administered without any cytotoxic conditioning, was well tolerated.
- **BLA submission for RP-L102 anticipated in Q4 2023.** Based on the positive efficacy and safety data from the pivotal study of RP-L102, Rocket is on track to submit the BLA during the fourth quarter of 2023 and anticipates providing an update following FDA acceptance.

Pyruvate Kinase Deficiency (PKD)

- **Presented positive clinical data from Phase 1 trial of RP-L301 at the ASGCT Annual Meeting.** Data showed robust and sustained efficacy in both adult patients for up to 30 months. Results from the first pediatric patient indicate preliminary efficacy and favorable safety.
- **Received EMA Priority Medicines (PRIME) designation for RP-L301 in July**. PRIME designation offers the benefits of early and enhanced support from the European Medicines Agency (EMA) and the opportunity for accelerated marketing application review. It is granted to medicines that target an unmet need for which no treatment option exists and with data showing a meaningful improvement of clinical outcomes.
- **Received FDA Regenerative Medicine Advanced Therapy (RMAT) designation for RP-L301.** RMAT designation was granted based on robust safety and efficacy data from the ongoing Phase 1 study and its potential to cure a life-threatening disease for which no curative therapies currently exist. The designation will provide the benefits of added FDA guidance and expedited review through the program's development. All four Rocket-sponsored programs with clinical data have received RMAT designation.
- **Adult and pediatric enrollment are completed in the Phase 1 study**. Phase 2 pivotal trial initiation activities are anticipated in the fourth quarter of 2023.

Operational Update

- **Entered into data licensing agreement with Invitae to support timely access to genetic testing for patients who may have genetic forms of cardiomyopathy.** The Invitae Unlock™ Cardiomyopathy and Arrhythmia genetic testing program evaluates causal mutations in approximately 170 genes, including *LAMP2*, *PKP2* and *BAG3*, that result in Danon Disease, PKP2-ACM and BAG3-DCM, respectively. As part of the agreement, Rocket is raising awareness of this testing program and educating healthcare professionals across the U.S. on the benefits of screening for genetic forms of cardiomyopathy.

Second Quarter Financial Results

- **Cash position**. Cash, cash equivalents and investments as of June 30, 2023, were $307.0 million.
- **R&D expenses**. Research and development expenses were $51.4 million for the three months ended June 30, 2023, compared to $41.4 million for the three months ended June 30, 2022. The increase in R&D expenses was primarily driven by increases in compensation and benefits expense of $5.7 million due to increased R&D headcount, clinical trial expenses of $2.7 million, and non-cash stock-based compensation of $1.7 million.
- **G&A expenses**. General and administrative expenses were $17.4 million for the three months ended June 30, 2023, compared to $12.9 million for the three months ended June 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 $1.3 million, compensation and benefits of $0.8 million due to increased G&A headcount and non-cash stock compensation expense of $1.1 million.
- **Net loss**. Net loss was $65.7 million or $0.82 per share (basic and diluted) for the three months ended June 30, 2023, compared to $54.4 million or $0.83 (basic and diluted) for the three months ended June 30, 2022.
- **Shares outstanding**. 80,521,415 shares of common stock were outstanding as of June 30, 2023.

Financial Guidance

- **Cash position.** As of June 30, 2023, Rocket had cash, cash equivalents and investments of $307.0 million. Rocket expects such resources will be sufficient to fund its operations into the first half of 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 childhood 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 vector (LV)-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 has received IND clearance for the AAV-based gene therapy program for PKP2-arrhythmogenic cardiomyopathy (ACM), a heart failure condition associated with life-threatening arrhythmias, cardiac structural abnormalities, and sudden cardiac death, and is advancing a preclinical program for 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), PKP2-ACM 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 "aim," "anticipate," "believe," "can," "continue," "design," "estimate," "expect," "intend," "may," "plan," "potential," "will give," "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, 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, our ability to submit regulatory filings with the U.S. Food and Drug Administration (FDA) and to obtain and maintain FDA or other regulatory authority approval of our product candidates, Rocket's dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, our competitors' activities, including decisions as to the timing of competing product launches, pricing and discounting, our integration of an acquired business, which involves a number of risks, including the possibility that the integration process could result in the loss of key employees, the disruption of our ongoing business, or inconsistencies in standards, controls, procedures, or policies, our ability to successfully

develop and commercialize any technology that we may in-license or products we may acquire and any 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 June 30,		Six Months Ended June 30,	
	2023	2022	2023	2022
Operating expenses:				
Research and development	$ 51,383	$ 41,356	$ 97,754	$ 72,150
General and administrative	17,374	12,854	33,197	24,624
Total operating expenses	68,757	54,210	130,951	96,774
Loss from operations	(68,757)	(54,210)	(130,951)	(96,774)
Interest expense	(468)	(465)	(936)	(928)
Interest and other income, net	846	669	2,754	1,291
Accretion of discount and amortization of premium on investments, net	2,678	(396)	5,097	(973)
Net loss	$ (65,701)	$ (54,402)	$ (124,036)	$ (97,384)
Net loss per share - basic and diluted	$ (0.82)	$ (0.83)	$ (1.55)	$ (1.50)
Weighted-average common shares outstanding - basic and diluted	80,472,362	65,476,531	79,965,755	64,995,797

	June 30, 2023	December 31, 2022
Cash, cash equivalents, and investments	$ 307,040	$ 399,670
Total assets	461,781	551,807
Total liabilities	58,811	62,121
Total stockholders' equity	402,970	489,686

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