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

May 4, 2023

Continued to advance leading pipeline of AAV cardiology and LV hematology assets; all 2023 milestones remain on track

Updates on all four clinical programs and PKP2-ACM preclinical program to be presented at the 26th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT)

Mark White, MB.ChB, named Chief Medical Officer following more than 25 years at AstraZeneca; Jonathan Schwartz, M.D., appointed Chief Gene Therapy Officer

Became founding consortium member of Rady Children's Institute for Genomic Medicine's BeginNGS™ newborn screening program to increase speed of diagnosis for genetic diseases

Cash, cash equivalents and investments of approximately $360M; expected operational runway into the first half of 2025

CRANBURY, N.J.--(BUSINESS WIRE)--May 4, 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 March 31, 2023, and updates from the Company's key pipeline developments, business operations and upcoming milestones.

"I am very pleased with our first quarter progress, as we continued to advance our industry leading pipeline of now six programs across both AAV and LV platforms addressing life threatening cardiac and hematologic rare diseases, including two LV programs nearing filings and four programs either demonstrating robust clinical results or strong preclinical proof of concept, "said Gaurav Shah, M.D., Chief Executive Officer, Rocket Pharma. "Corporate and program milestones remain on track, including the planned initiation of the Phase 2 pivotal trial in Danon Disease this second quarter and regulatory filings for LAD-I and Fanconi Anemia this second quarter and fourth quarter, respectively."

Dr. Shah continued, "In addition, we look forward to presenting data across all four of our clinical programs at the upcoming ASGCT meeting this month, as well as our recently announced preclinical program for PKP2-ACM, one of the most prevalent and devastating forms of heart disease impacting nearly 50,000 adults and children in the U.S. and EU. Further, I am proud to announce that we became a Founding Member of BeginNGS™, a national program that advocates for and facilitates newborn screening for approximately 400 genetic diseases. As we diligently work towards cures for rare diseases, encouraging early genetic screening is essential in providing rare disease patients and their families with an accurate and early diagnosis so they can identify and activate a disease management plan with their healthcare providers as soon as possible."

"Lastly, following a recent ATM sale of $17.2M in net proceeds and in-house manufacturing efficiencies, I am pleased to announce that we have extended our cash runway into the first half of 2025. We look forward to leveraging this strong cash position, which will take us through planned launches in 2024," said Dr. Shah. "Taken together, I am incredibly proud of the tremendous progress we have made in expeditiously developing one of the broadest and deepest pipeline of assets in gene therapy and building integrated capabilities spanning discovery through manufacturing and commercial to help address the unmet needs of patients facing these rare and devastating diseases. We look forward to continuing this progress into the next quarter and year."

Key Pipeline and Operational Updates

- **Danon, FA, LAD-I, PKD trials and plans for PKP2-ACM and BAG3-DCM preclinical programs remain on track.** All 2023 milestones remain on track including anticipated initiation of the Phase 2 pivotal trial for Danon Disease during this second quarter and Biologics License Application (BLA) filing for LAD-I in Q2 2023 and BLA filing for FA in Q4 2023.
- **Updated data across all clinical trials and PKP2-ACM preclinical program to be presented at ASGCT.** Updates anticipated at the 26th Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) at the Los Angeles Convention Center, May 16-20.

Details for oral presentations are as follows:

Title: Danon Disease Phase 1 RP-A501 Results: The First Single-Dose Intravenous (IV) Gene Therapy with Recombinant Adeno-Associated Virus (AAV9:LAMP2B) for a Monogenic Cardiomyopathy
Session: Clinical Trials Spotlight Symposium
Presenter: Joseph Rossano, M.D., M.S., FAAP, FACC, Co-Director of the Cardiac Center and Chief of the Division of Cardiology at Children's Hospital of Philadelphia
Session date and time: Thursday, May 18, 2023, 8:00 a.m. – 9:45 a.m. PT
Location: Concourse Hall 152 & 153
Presentation number: 9

Title: Lentiviral-Mediated Gene Therapy for Fanconi Anemia [Group A]: Results From Global RP-L102 Clinical Trials
Session: Hematologic and Immunologic Diseases
Presenter: Agnieszka Czechowicz, M.D., Ph.D., Department of Pediatrics, Division of Hematology/ Oncology, Stem Cell Transplantation and Regenerative Medicine, Stanford University School of Medicine
Session date and time: Thursday, May 18, 2023, 3:45 p.m. – 5:30 p.m. PT
Location: Room 501 ABC
Presentation number: 217

Title: Global Phase 1 Study Results of Lentiviral Mediated Gene Therapy for Severe Pyruvate Kinase Deficiency (PKD)
Session: Hematologic and Immunologic Diseases
Presenter: Ami J. Shah, M.D., Clinical Professor of Pediatrics, Division of Hematology/ Oncology, Stem Cell Transplantation and Regenerative Medicine, Stanford University School of Medicine
Session date and time: Thursday, May 18, 2023, 3:45 p.m. – 5:30 p.m. PT
Location: Room 501 ABC
Presentation number: 218

Title: Preclinical Efficacy of AAVrh.74-PKP2a (RP-A601): Gene Therapy for PKP2-associated Arrhythmogenic Cardiomyopathy
Session: Late-Breaking Abstracts 1
Presenter: Christopher Herzog, Ph.D., Associate Vice President, AAV R&D, Rocket Pharma
Session date and time: Friday, May 19, 2023, 8:00 a.m. – 9:45 a.m. PT
Location: Room 515AB
Presentation number: 2

Details for the poster presentation are as follows:

Title: Autologous Ex-Vivo Lentiviral Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I): Interim Results from an Ongoing Phase 1/2 Study
Session: Friday Poster Session
Presenter: Donald B. Kohn, M.D., Distinguished Professor of Microbiology, Immunology & Molecular Genetics (MIMG), Pediatrics and Molecular & Medical Pharmacology; Director of the UCLA Human Gene and Cell Therapy Program, University of California, Los Angeles
Session date and time: Friday, May 19, 2023, 12:00 p.m. PT
Location: Exhibit Hall/West Hall A
Presentation number: 1547

- **Expanded leadership team to support evolution towards Commercial stage**. Jonathan Schwartz, M.D., Rocket's founding Chief Medical Officer, was appointed Chief Gene Therapy Officer to enhance the Company's focus on the strategic application of gene therapy technologies to current and future therapeutic areas. Dr. Schwartz oversees research, deepens relationships with external collaborators, and offers a pointed focus on clinical strategy and pipeline expansion. In addition, Mark White, MB.ChB, was named Chief Medical Officer following more than 25 years at AstraZeneca and brings expertise in clinical development, global regulatory submissions, and commercial and business strategy. At AstraZeneca, Dr. White most recently served as Global Franchise Head, Respiratory and Inflammation and has been the program lead for multiple innovative medicines guiding them through late-stage development, approvals and launches around the globe.
- **Became founding consortium member of a diagnostic and precision medicine guidance tool to accelerate early diagnosis of rare genetic diseases.** Rocket entered into a collaboration with Rady Children's Institute for Genomic Medicine to advance BeginNGS™, a national program that advocates for and facilitates newborn screening for approximately 400 genetic diseases using rapid Whole Genome Sequencing (rWGS®). As part of the collaboration, Rocket joins the BeginNGS™ consortium, which serves as a think tank across public and private institutions and participates in the Frontiers in Pediatric Genomic Medicine Conference. As a founding consortium member, Rocket will play a leading role advocating for early diagnosis of patients with genetic rare diseases.
- **Celebrated annual Rare Disease Day with multi-faceted awareness campaign.** On February 28, 2023, Rocket hosted its annual Rare Disease Day recognition program highlighting the theme, "Stories That Need To Be Shared: The Human Side of Rare Disease." More than 300 members of the global rare disease community gathered in person and virtually at the Make-A-Wish New Jersey Samuel & Josephine Plumeri Wishing Place to hear inspirational stories from patients living with rare diseases, caregivers and patient advocates. The Company also continued to build upon its Light Up for Rare initiative in collaboration with global partners to light up buildings and landmarks in more than 100 countries across the globe in the Rare Disease Day colors, including the Empire State Building and Niagara Falls. Rocket remains committed to supporting the rare disease community through patient-focused events, education and advancing science to bring potential treatments to patients with unmet needs.

First Quarter Financial Results

- **Cash position**. Cash, cash equivalents and investments as of March 31, 2023, were $360.0 million.
- **R&D expenses**. Research and development expenses were $46.4 million for the three months ended March 31, 2023, compared to $30.8 million for the three months ended March 31, 2022. The increase in R&D expenses was primarily driven by increases in compensation and benefits expense of $6.6 million due to increased R&D headcount, manufacturing and development costs of $2.8 million, direct materials of $0.9 million, and laboratory supplies of $0.9 million.
- **G&A expenses**. General and administrative expenses were $15.8 million for the three months ended March 31, 2023, compared to $11.8 million for the three months ended March 31, 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 $1.1 million, compensation and benefits of $0.7 million due to increased G&A headcount and non-cash stock compensation expense of $1.1 million.
- **Net loss**. Net loss was $58.3 million or $0.73 per share (basic and diluted) for the three months ended March 31, 2023, compared to $43.0 million or $0.67 (basic and diluted) for the three months ended March 31, 2022.
- **Shares outstanding**. 80,412,194 shares of common stock were outstanding as of March 31, 2023.

Financial Guidance

- **Cash position.** As of March 31, 2023, Rocket had cash, cash equivalents and investments of $360.0 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 our 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 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 preclinical 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 "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 March 31,	
	2023	2022
Operating expenses:		
Research and development	$ 46,371	$ 30,794

General and administrative	15,823	11,770
Total operating expenses	62,194	42,564
Loss from operations	(62,194)	(42,564)
Interest expense	(468)	(464)
Interest and other income, net	1,908	623
Accretion of discount and amortization of premium on investments, net	2,419	(577)
Net loss	$ (58,335)	$ (42,982)
Net loss per share - basic and diluted	$ (0.73)	$ (0.67)
Weighted-average common shares outstanding - basic and diluted	79,453,519	64,509,721

	March 31, 2023	December 31, 2022
Cash, cash equivalents, and investments	$ 360,041	$ 399,670
Total assets	514,757	551,807
Total liabilities	55,881	62,121
Total stockholders' equity	458,876	489,686

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