Rocket Pharmaceuticals Reports First Quarter 2022 Financial Results and Highlights Recent Progress

May 5, 2022

—Continued to advance clinical gene therapy programs for the treatment of Danon Disease, Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I) and Pyruvate Kinase Deficiency (PKD) —

—Updates on all four programs to be presented at the 2022 Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) this month; trials on track for 2022 data readouts —

—Appointed internationally recognized cardiovascular physician-scientist and biopharmaceutical executive Fady Malik, M.D., Ph.D., to Board of Directors –

—Named experienced commercial leader Carlos Martin to Chief Commercial Officer and proven biotech executive Jessie Yeung to Vice President of Investor Relations and Corporate Finance —

— Cash position of $346.6M; operational runway extended into first half of 2024 —

CRANBURY, N.J.--(BUSINESS WIRE)--May 5, 2022-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a leading late-stage, clinical biotechnology company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders with high unmet need, today reports financial results for the quarter ending March 31, 2022, and updates from the Company's key pipeline developments, business operations and upcoming milestones.

"I am pleased with our first quarter progress as we maintained our strong focus on execution across each of our clinical programs, in-house AAV manufacturing readiness and overall business operations," said Gaurav Shah, M.D., Chief Executive Officer of Rocket Pharma. "We are looking forward to this month's ASGCT meeting, where we will present updates from all four clinical programs. We also remain on track to deliver full data readouts through the rest of the year as planned."

Dr. Shah continued, "In the first quarter, we also bolstered our Company leadership team with the appointments of Fady Malik, M.D., Ph.D., an internationally recognized physician-scientist, to our Board of Directors, global commercial executive Carlos Martin to Chief Commercial Officer and capital markets expert Jessie Yeung to Vice President, Investor Relations and Corporate Finance. Furthermore, we continued to scale up our in-house manufacturing facility and remain on track for AAV cGMP manufacturing initiation in Q2."

"Finally, we extended our cash runway from the second half of 2023 into the first half of 2024 and have a strong balance sheet with liquidity through potential regulatory filings and approvals," said Dr. Shah. "Taken together, we have generated tremendous momentum with our best-in-class gene therapies, utilizing both ex-vivo lentiviral and in-vivo AAV platforms, in our pursuit of cures for patients facing these rare and truly devastating diseases."

Key Pipeline and Operational Updates

- **Danon, FA, LAD-I and PKD trials have continued to progress and remain on track.** All 2022 milestones remain on track including potential top-line readouts for Phase 2 trials in LAD-I and FA in Q2 and Q3, respectively, pediatric efficacy readout for the Phase 1 Danon Disease trial in Q3, and the Phase 1 readout for PKD in Q4.
- **Updated clinical data to be presented at ASGCT**. Updates from all four clinical programs to be presented at the 2022 Annual Meeting of the American Society of Gene and Cell Therapy (ASGCT) at the Walter E. Washington Convention Center in Washington, D.C., May 16-19.

Details for oral presentations are as follows:

Title: Extended Results from First-In-Human Clinical Trial of RP-A501 (AAV9:LAMP2B) Gene Therapy Treatment For Danon Disease
Session: Cardiovascular and Pulmonary Diseases

Presenter: Barry Greenberg, M.D., FHFSA, University of California, San Diego Medical Center, La Jolla, CA
Date: Monday, May 16, 2022
Session Time: 10:15 a.m. – 12:00 p.m. ET
Presentation Time: 10:45 a.m. – 11:00 a.m. ET
Location: Room 206
Abstract Number: 24

Title: Ex vivo Lentiviral-mediated Gene Therapy for Patients with Fanconi Anemia [Group A]: Updated Results from Global RP-L102 Clinical Trials
Session: Hematopoietic Stem Cell Gene Therapy
Presenter: Agnieszka Czechowicz, M.D., Ph.D., Center for Definitive and Curative Medicine, Stanford University School of Medicine, Stanford, CA
Date: Monday, May 16, 2022
Session Time: 3:45 p.m. – 5:30 p.m. ET
Presentation Time: 4:15 p.m. – 4:30 p.m. ET
Location: Room 202
Abstract Number: 108

Title: Interim Results from an ongoing Phase 1/2 Study of Lentiviral-Mediated Ex-Vivo Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I)
Session: Clinical Trials Spotlight Symposium
Presenter: Donald B. Kohn, M.D., Broad Stem Cell Research Center, UCLA, Los Angeles and recipient of ASGCT's 2022 Outstanding Achievement Award
Date: Thursday, May 19, 2022
Session Time: 8:00 a.m. – 9:45 a.m. ET
Presentation Time: 8:00 a.m. – 8:15 a.m. ET
Location: Ballroom C
Abstract Number: 1188

Details for the poster presentation are as follows:

Title: Changing the Treatment Paradigm for Pyruvate Kinase Deficiency with Lentiviral Mediated Gene Therapy: Interim Results from an Ongoing Global Phase 1 Study
Presenter: Ami Shah, M.D., Center for Definitive and Curative Medicine, Stanford University School of Medicine, Stanford, CA
Date: Monday, May 16, 2022
Time: 5:30 p.m. – 6:30 p.m. ET
Location: Hall D
Abstract Number: 357

- **In-house AAV current Good Manufacturing Practice (cGMP) manufacturing initiation anticipated in Q2.** The Company's state-of-the-art, 103,720 ft ^{2} manufacturing facility in Cranbury, N.J. is being scaled up to manufacture AAV drug product for a planned Phase 2 study in Danon Disease.
- **Broadened cardiovascular experience of Rocket's Board of Directors.** The Company announced the appointment of Fady Malik, M.D., Ph.D. to its Board of Directors. Dr. Malik brings nearly 25 years of experience as an internationally recognized cardiovascular physician-scientist and highly successful biopharmaceutical executive. Dr. Malik is Executive Vice President of Research and Development at Cytokinetics, a late-stage biopharmaceutical company, where he has worked in a variety of positions since he joined the founders to launch the company.
- **Named Chief Commercial Officer**. In March 2022, Carlos Martin was appointed to Senior Vice President, Chief Commercial Officer. Mr. Martin brings over 20 years of global commercial leadership gained at Novartis, Schering Plough and Eli Lilly. Mr. Martin will lead the go-to-market entry models in the U.S. and EU and build-out of key commercial capabilities to pave the way for potential patient access, reimbursement and treatment of Rocket's gene therapies.
- **Appointed Vice President, Investor Relations & Corporate Finance**. In March 2022, Jessie Yeung joined the Company as Vice President, Investor Relations. Ms. Yeung brings more than 15 years of investor relations, corporate finance and capital market experience across industries including the biopharmaceutical and financial sectors. Prior to Rocket, Ms. Yeung was the Head of Corporate Finance and Investor Relations at Legend Biotech. Ms. Yeung also has experience as an equity research analyst at Bank of America Merrill Lynch, Wells Fargo, and J.P. Morgan.
- **Published peer-reviewed expanded categorization of severe PKD**. "Who should be eligible for gene therapy clinical trials in red blood cell Pyruvate Kinase Deficiency (PKD)?: Toward an expanded definition of severe PKD" was published in the *American Journal of Hematology*. The publication summarizes the natural history of the disease and proposes an expanded categorization of severe PKD to help identify the most optimal patients for ongoing gene- and cell-based evaluations.
- **Recognized Rare Disease Day with an event at NASDAQ Tower in New York City**. On February 28, 2022, Rocket hosted its annual Rare Disease Day celebration highlighting the theme, "Rare, But Not Alone." More than 250 members of the global rare disease community and Rocket team gathered in person and virtually to hear about the impact of rare disease and clinical research from patients, families, advocacy groups and scientific collaborators and innovators – including Dr. Moris Danon, founder of Danon Disease. The event concluded with the lighting of the Empire State Building, as well as other global landmarks, in Rare Disease Day colors.

Anticipated 2022 Milestones

RP-A501 for Danon Disease (AAV)

- Report data from pediatric patient cohort of Phase 1 trial – Q3 2022
- Initiate pivotal Phase 2 trial activities – Q4 2022

RP-L201 for Leukocyte Adhesion Deficiency-I (LVV)

- Report top-line data from pivotal Phase 2 trial – Q2 2022

RP-L102 for Fanconi Anemia (LVV)

- Report top-line data from pivotal Phase 2 trial – Q3 2022

RP-L301 for Pyruvate Kinase Deficiency (LVV)

- Report preliminary Phase 1 data – Q4 2022
- Initiate pivotal Phase 2 trial activities – Q4 2022

Manufacturing Facility in Cranbury, New Jersey

- Achieve in-house AAV current Good Manufacturing Practice (cGMP) manufacturing readiness – Q2 2022

Upcoming Investor Conference

- Kinnari Patel, Pharm.D., MBA, President and Chief Operating Officer, will deliver an in-person company presentation at the Bank of America Securities Healthcare Conference on Wednesday, May 11 at 2:20 p.m. ET at the Encore Hotel in Las Vegas.

First Quarter Financial Results

- **Cash position.** Cash, cash equivalents and investments as of March 31, 2022, were $346.6 million.
- **R&D expenses.** Research and development expenses were $30.8 million for the three months ended March 31, 2022, compared to $28.3 million for the three months ended March 31, 2021. The increase in research and development expense was primarily driven by an increase in compensation and benefits expense due to increased R&D headcount, an increase in laboratory supplies and manufacturing development expenses, offset by a decrease in non-cash stock compensation expense.
- **G&A expenses.** General and administrative expenses were $11.7 million for the three months ended March 31, 2022, compared to $10.9 million for the three months ended March 31, 2021. The increase in general and administrative expenses was primarily driven by an increase in compensation and benefits expense due to increased G&A headcount, and an increase in commercial preparation expenses, offset by a decrease in non-cash stock compensation expense.
- **Net loss.** Net loss was $43.0 million or $0.67 per share (basic and diluted) for the three months ended March 31, 2022, compared to $40.2 million or $0.65 per share (basic and diluted) for the three months ended March 31, 2021
- **Shares outstanding.** 64,522,057 shares of common stock were outstanding as of March 31, 2022

Financial Guidance

- **Cash position.** As of March 31, 2022, we had cash, cash equivalents and investments of $346.6 million. In April 2022, the Company sold 1.3 million shares of common stock for net proceeds of $17.3 million under the at-the-market facility. With the at-the-market facility proceeds and other efficiencies, the Company expects such resources will be sufficient to fund its operating expenses and capital expenditure requirements into the first half of 2024, including the continued buildout and initiation of AAV cGMP manufacturing capabilities at our Cranbury, New Jersey R&D and manufacturing facility and continued development of our four clinical programs as well as future pipeline programs.

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) is advancing an integrated and sustainable pipeline of genetic therapies that 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 (LVV)-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. 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 its guidance for 2022 in light of COVID-19, 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), and Danon Disease, the expected timing and data readouts of Rocket's ongoing and planned clinical trials, Rocket's plans for the advancement of its Danon Disease program following the lifting of the FDA's clinical hold 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, 2021, filed February 28, 2022 with the SEC. 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,	
	2022	**2021**
Operating expenses:		
Research and development	$ 30,794	$ 28,309
General and administrative	11,770	10,913
Total operating expenses	42,564	39,222
Loss from operations	(42,564)	(39,222)
Research and development incentives	-	500
Interest expense	(464)	(1,729)
Interest and other income net	623	911
(Amortization of premium) accretion of discount on investments - net	(577)	(639)
Total other expense, net	(418)	(957)
Net loss	$ (42,982)	$ (40,179)
Net loss per share attributable to common stockholders - basic and diluted	$ (0.67)	$ (0.65)
Weighted-average common shares outstanding - basic and diluted	64,509,721	61,574,405

	March 31, 2022	**December 31, 2021**
Cash, cash equivalents and investments	346,593	388,740
Total assets	460,150	497,020
Total liabilities	42,530	42,296
Total stockholders' equity	417,620	454,724

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