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

May 6, 2024

Advanced RP-L102 for Fanconi Anemia towards regulatory reviews; EMA accepted MAA for review and BLA submission anticipated in the first half of 2024

Continued preparations to launch LV portfolio beginning with KRESLADI™ (marnetegragene autotemcel) for severe LAD-I; PDUFA date of June 30, 2024

Appointed Aaron Ondrey as Chief Financial Officer, bringing seasoned leadership experience in commercial-stage financial management, strategic planning, and capital allocation

Cash, cash equivalents and investments of approximately $330.3M; expected operational runway into 2026

CRANBURY, N.J.--(BUSINESS WIRE)--May 6, 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 March 31, 2024.

"Rocket has had a strong start to 2024. This quarter has been marked by meaningful progress across all our clinical programs, most notably with the EMA's review of RP-L102 for Fanconi Anemia, commercial preparations for the potential approval of KRESLADI for severe LAD-I, and ongoing enrollment in the Phase 2, pivotal study of RP-A501 for Danon Disease and the Phase 1 study of RP-A601 for PKP2-ACM," said Gaurav Shah, M.D., Chief Executive Officer, Rocket Pharmaceuticals. "As we build on the advancements made this quarter, we remain focused on execution for patients with rare and devastating diseases with limited treatment options."

Recent Pipeline and Operational Updates

- **Advanced Fanconi Anemia (FA) program through key regulatory milestones.**
  - In April, Rocket announced that the European Medicines Agency (EMA) accepted the Marketing Authorization Application (MAA) for RP-L102 for the treatment of FA. MAA acceptance was based on positive, previously disclosed data from the global RP-L102 Phase 1/2 clinical trial.
  - The Company remains on track to submit the Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) in the first half of 2024.

- **KRESLADI™ on track for June 30, 2024, PDUFA date.**
  - Rocket continues to ramp up enabling activities to support the launch of its lentiviral (LV) vector portfolio beginning with KRESLADI™ for severe Leukocyte Adhesion Deficiency-I (LAD-I).Qualified Treatment Center initiation, disease education, payer engagement, and field team build-out are all underway.

- **Bolstered finance, investor relations, and corporate communications expertise of company leadership to support evolution towards commercial stage.**
  - Rocket appointed Aaron Ondrey as Chief Financial Officer (CFO). Mr. Ondrey brings over 20 years of experience leading commercial-stage financial management, strategic planning, and capital allocation. Mr. Ondrey was previously the CFO at Mirati Therapeutics and has held multiple senior finance leadership positions at Arena Pharmaceuticals, Alexion Pharmaceuticals, and Regeneron Pharmaceuticals.
  - In addition, Meg Dodge was appointed Vice President to lead Investor Relations & Corporate Communications. Ms. Dodge is experienced with engaging stakeholders across investors, media, and other communities in the biotech and financial sectors. Prior to joining Rocket, Ms. Dodge was Head of Investor Relations and Corporate

Communications at Krystal Biotech.

- **Celebrated annual Rare Disease Day with multi-faceted awareness campaign**.
  - On February 29, 2024, Rocket hosted its annual Rare Disease Day recognition program highlighting the theme, "Leap into Action for Rare." Several hundred attendees gathered at the Liberty Science Center and virtually to hear inspirational stories from the community. Rocket also continued its Light Up for Rare initiative in collaboration with global partners to light up buildings and landmarks in Rare Disease Day colors, including the Empire State Building.
  - Rocket remains highly committed to supporting the rare disease community through patient-focused events, education and advancing science to bring potential treatments to patients with unmet needs.

- **Milestones in 2024 are on track across its pipeline of gene therapies for rare and devastating diseases.**
  - Rocket continues to advance three disclosed programs from its adeno-associated virus (AAV) cardiovascular portfolio, including:
    - Phase 2 pivotal study of RP-A501 for Danon Disease,
    - Phase 1 study of RP-A601 for PKP2-arrhythmogenic cardiomyopathy (ACM), and
    - IND-enabling studies for BAG3-associated dilated cardiomyopathy (DCM)
  - In its late-stage LV portfolio, Rocket is working towards initiation of the Phase 2 pivotal study of RP-L301 for Pyruvate Kinase Deficiency (PKD).

- **Data from Rocket's LV hematology portfolio to be presented at the American Society of Gene and Cell Therapy (ASGCT) 27th Annual Meeting.**
  - Updated data across Rocket's LV hematology programs will be highlighted as oral presentations at the ASGCT 27 th Annual Meeting taking place May 7-11, 2024, in Baltimore, MD.
  - Incremental updates include longer-term data demonstrating the safety and efficacy of Rocket's Phase 1/2 pivotal studies of KRESLADI™ for severe LAD-I and RP-L102 for FA, in addition to the Phase 1 study of RP-L301 for PKD.
  - Details for oral presentations are as follows:

    Title: Gene Therapy for Adult and Pediatric Patients with Severe Pyruvate Kinase Deficiency: Results from a Global Study of RP-L301
    Session: Clinical Trials Spotlight Symposium
    Presenter: Julián Sevilla, M.D., Ph.D., Clinical Investigator, Hematología y Hemoterapia, Hematología y Oncología Pediátricas, Hospital Infantil Universitario Niño Jesús
    Presentation date and time: Wednesday, May 8, 2024, 8:00 a.m. – 8:15 a.m. ET
    Location: Ballroom 1
    Presentation number: 4

    Title: Lentiviral-Mediated Gene Therapy (RP-L102) for Fanconi Anemia [Group A] is Associated with Polyclonal Integration Patterns in the Absence of Conditioning
    Session: Cell Therapy and Cell-Based Gene Therapy Trials
    Presenter: Agnieszka Czechowicz, M.D., Ph.D., Center for Definitive and Curative Medicine, Department of Pediatrics, Division of Hematology/ Oncology, Stem Cell Transplantation and Regenerative Medicine, Stanford University School of Medicine, Lucile Packard Children's Hospital Stanford
    Presentation date and time: Friday, May 10, 2024, 2:45 p.m. – 3:00 p.m. ET
    Location: Ballroom 1
    Presentation number: 245

    Title: Autologous *Ex-Vivo* Lentiviral Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I Provides Sustained Efficacy with a Favorable Safety Profile
    Session: Cell Therapy and Cell-Based Gene Therapy Trials
    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
    Presentation date and time: Friday, May 10, 2024, 3:00 p.m. – 3:15 p.m. ET
    Location: Ballroom 1
    Presentation number: 246

Upcoming Investor Conference

- Bank of America Global Healthcare Conference 2024: May 16, 2024

First Quarter Financial Results

- **Cash position.** Cash, cash equivalents and investments as of March 31, 2024, were $330.3 million.
- **R&D expenses.** Research and development expenses were $45.2 million for the three months ended March 31, 2024, compared to $46.4 million for the three months ended March 31, 2023. The decrease in R&D expenses was primarily driven by decreases in manufacturing and development costs and direct materials of $5.8 million. Decreases were partially

offset by increases in costs for compensation and benefits expense of $1.4 million due to increased R&D headcount, professional fees of $1.1 million, laboratory supplies of $0.9 million, non-cash stock compensation expense of $0.8 million, and clinical trial costs of $0.6 million.

- **G&A expenses.** General and administrative expenses were $22.1 million for the three months ended March 31, 2024, compared to $15.8 million for the three months ended March 31, 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 $3.3 million, legal expenses of $1.5 million, and non-cash stock compensation expense of $0.5 million.
- **Net loss.** Net loss was $62.1 million or $0.66 per share (basic and diluted) for the three months ended March 31, 2024, compared to $58.3 million or $0.73 (basic and diluted) for the three months ended March 31, 2023.
- **Shares outstanding.** 90,646,590 shares of common stock were outstanding as of March 31, 2024.

Financial Guidance

- **Cash position.** As of March 31, 2024, Rocket had cash, cash equivalents and investments of $330.3 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 "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, Rocket's plans for the advancement of its DD program, including its planned pivotal trial, and the safety, effectiveness and timing of related 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 and Rocket's ability to expand its pipeline to target additional indications that are compatible with its gene therapy technologies. 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, 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 March 31,	
	2024	2023
Operating expenses:		
Research and development	$ 45,227	$ 46,371
General and administrative	22,148	15,823

Total operating expenses	67,375	62,194
Loss from operations	(67,375)	(62,194)
Interest expense	(471)	(468)
Interest and other income, net	3,029	1,908
Accretion of discount on investments, net	2,763	2,419
Net loss	$ (62,054)	$ (58,335)
Net loss per share - basic and diluted	$ (0.66)	$ (0.73)
Weighted-average common shares outstanding - basic and diluted	93,549,884	79,453,519

	March 31, 2024	December 31, 2023
Cash, cash equivalents, and investments	$ 330,313	$ 407,495
Total assets	499,442	566,341
Total liabilities	57,940	73,767
Total stockholders' equity	441,502	492,574

View source version on businesswire.com: https://www.businesswire.com/news/home/20240506660250/en/

Media & Investors
Meg Dodge

Media
Kevin Giordano
media@rocketpharma.com

Investors
Brooks Rahmer
investors@rocketpharma.com

Source: Rocket Pharmaceuticals, Inc.