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

November 7, 2024

Enrollment completed in September and dosing ongoing in the Phase 2 pivotal study of RP-A501 for Danon disease

Updated Phase 1 data from RP-A501 for Danon disease anticipated at American Heart Association's 2024 Late-Breaking Scientific Sessions

Enrollment completed in low dose cohort of Phase 1 study of RP-A601 for PKP2-ACM; Preliminary data expected in the first half of 2025

Rolling BLA submission initiated for RP-L102 for Fanconi Anemia

Progress on FDA-approval of KRESLADI for severe LAD-I; Approval anticipated in 2025

Appointed Mikael Dolsten, M.D., Ph.D., to Board of Directors

Cash, cash equivalents and investments of approximately $235.7M; expected operational runway into 2026

CRANBURY, N.J.--(BUSINESS WIRE)--Nov. 7, 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 third quarter ending September 30, 2024.

"Rocket made meaningful progress during the third quarter, notably with the completion of enrollment in the RP-A501 program for Danon disease, low dose cohort enrollment completion in the RP-A601 program for PKP2-ACM, and appointment of seasoned pharmaceutical executive, Mikael Dolsten to our Board of Directors," said Gaurav Shah, M.D., Chief Executive Officer, Rocket Pharmaceuticals. "As we continue to pursue our mission of seeking gene therapy cures for patients with rare and devastating diseases, we remain focused on expediently advancing our deep pipeline of cardiovascular and hematology programs."

Recent Pipeline and Operational Updates

- **Continued advancement of Phase 2 pivotal study of RP-A501 for Danon Disease**.
  - In September, Rocket announced completion of enrollment in the Phase 2 pivotal study of RP-A501 to treat Danon Disease.
  - Dosing in the Phase 2 pivotal study is ongoing.
  - Updated data from the Phase 1 study to be presented at the American Heart Association's 2024 Late-Breaking Science sessions on November 18.
  - Details of the Phase 2 pivotal study can be found at www.ClinicalTrials.gov under NCT identifier NCT06092034.

- **Progressed the Phase 1 clinical study of RP-A601 for PKP2 arrhythmogenic cardiomyopathy (ACM).**
  - Completed patient enrollment in the low dose cohort.
  - Preliminary data from the Phase 1 study is expected in the first half of 2025.
  - Ongoing internal estimates confirm that PKP2-ACM affects approximately 50,000 people in the U.S. and Europe, representing the largest market opportunity in Rocket's pipeline of disclosed programs.
  - Details of the Phase 1 study can be found at www.ClinicalTrials.gov under the NCT identifier NCT05885412.

- **Progressed BAG3-associated dilated cardiomyopathy preclinical program.**
  - Nonclinical, IND-enabling studies are ongoing.
  - Submission of the IND is anticipated in the first half of 2025.

- **Progressed RP-L102 investigational gene therapy for Fanconi Anemia (FA).**

- Initiated rolling Biologics License Application (BLA) for RP-L102.
- Secured an ICD-10 code from the Centers for Medicare and Medicaid Services to document patients with FA.
- Regulatory review for RP-L102 for the treatment of FA by the European Medicines Authority is underway.

- **U.S. Food and Drug Administration (FDA) review of limited additional Chemistry Manufacturing and Controls (CMC) information ongoing for KRESLADI™ (marnetegragene autotemcel; marne-cel) for the treatment of severe leukocyte adhesion deficiency-I (LAD-I).**
  - Rocket previously disclosed that the FDA requested limited additional CMC information to complete its review of KRESLADI to treat severe LAD-I.
  - The Company continues to work with senior leaders and reviewers from the FDA's Center for Biologics Evaluation and Research.
  - Approval of KRESLADI anticipated in 2025.

- **Initiated global Phase 2 pivotal study of RP-L301 for Pyruvate Kinase Deficiency.**
  - Details of the Phase 2 study can be found at www.ClinicalTrials.gov under NCT identifier NCT06422351.

- **Appointed Mikael Dolsten, M.D., Ph.D., to Board of Directors**.
  - Dr. Dolsten is an accomplished industry executive with extensive global pharmaceutical management experience. He currently serves as the Chief Scientific Officer, President, Research & Development at Pfizer Inc., focused on advancing gene therapies, small-molecule medicines, biotherapeutics, and vaccines.

Third Quarter Financial Results

- **Cash position.** Cash, cash equivalents and investments as of September 30, 2024, were $235.7 million.
- **R&D expenses.** Research and development expenses were $133.9 million for the nine months ended September 30, 2024, compared to $144.6 million for the nine months ended September 30, 2023. The decrease of $10.7 million in R&D expenses was primarily driven by decreases in manufacturing and development and direct material costs of $17.4 million and costs for research agreements of $1.2 million. The decreases were partially offset by increases in the costs for professional fees and consultants of $4.9 million, non-cash stock compensation expenses of $1.2 million, and compensation and benefit expenses of $1.0 million.
- **G&A expenses.** General and administrative expenses were $76.6 million for the nine months ended September 30, 2024, compared to $51.8 million for the nine months ended September 30, 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 $15.7 million, legal expenses of $4.0 million, non-cash stock compensation expense of $2.2 million, and compensation and benefit expenses of $1.9 million.
- **Net loss.** Net loss was $198.4 million or $2.11 per share (basic and diluted) for the nine months ended September 30, 2024, compared to $185.9 million or $2.30 (basic and diluted) for the nine months ended September 30, 2023.
- **Shares outstanding.** 91,116,692 shares of common stock were outstanding as of September 30, 2024.

Financial Guidance

- **Cash position.** As of September 30, 2024, Rocket had cash, cash equivalents and investments of $235.7 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 "could," "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, including the timing and outcome of the FDA's review of the additional CMC information that Rocket will provide in response to the FDA's request, the safety, effectiveness and timing of 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, Rocket's ability to expand its pipeline to target additional indications that are compatible with its gene therapy technologies, Rocket's ability to transition to a commercial stage pharmaceutical company, and Rocket's expectation that its cash, cash equivalents and investments will be sufficient to funds its operations into 2026. 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, the integration of new executive team members and the effectiveness of the newly configured corporate leadership team, 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 September 30,		Nine Months Ended September 30,	
	2024	2023	2024	2023
Operating expenses:				
Research and development	$ 42,315	$ 46,844	$ 133,887	$ 144,598
General and administrative	27,109	18,585	76,624	51,782
Total operating expenses	69,424	65,429	210,511	196,380
Loss from operations	(69,424)	(65,429)	(210,511)	(196,380)
Interest expense	(471)	(469)	(1,413)	(1,405)
Interest and other income, net	1,327	1,720	6,650	4,474
Accretion of discount on investments, net	1,849	2,279	6,855	7,376
Net loss	$ (66,719)	$ (61,899)	$ (198,419)	$ (185,935)
Net loss per share - basic and diluted	$ (0.71)	$ (0.75)	$ (2.11)	$ (2.30)
Weighted-average common shares outstanding - basic and diluted	94,158,491	82,636,120	93,893,729	80,865,658

	September 30, 2024	December 31, 2023
Cash, cash equivalents, and investments	$ 235,662	$ 407,495
Total assets	393,688	566,341
Total liabilities	63,917	73,767
Total stockholders' equity	329,771	492,574

View source version on businesswire.com: https://www.businesswire.com/news/home/20241107375632/en/

Media & Investors
Meg Dodge
mdodge@rocketpharma.com

Media
Kevin Giordano
media@rocketpharma.com

Investors

Brooks Rahmer
investors@rocketpharma.com

Source: Rocket Pharmaceuticals, Inc.