Rocket Pharmaceuticals Reports Fourth Quarter and Full Year 2023 Financial Results and Highlights Recent Progress

February 26, 2024

Advancing leading pipeline of six disclosed programs across AAV cardiovascular and LV hematology portfolios; all milestones remain on track for 2024

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

Cash, cash equivalents and investments of approximately $407.5M; expected operational runway into 2026

CRANBURY, N.J.--(BUSINESS WIRE)--Feb. 26, 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 operational results for the fourth quarter and year ended December 31, 2023.

"I am pleased with the strong results Rocket delivered in 2023, as we closed another successful year of progress across all six disclosed gene therapy programs spanning our AAV cardiovascular and LV hematology portfolios," said Gaurav Shah, M.D., Chief Executive Officer, Rocket Pharma. "We are well poised to make further strides in 2024, notably expanding our commercial and operational capabilities to support the anticipated launch of KRESLADI ™ (marnetegragene autotemcel) for severe LAD-I, representing Rocket's first step in making our gene therapies available to patients who need them most. At the same time, we are laser focused on regulatory filings in Fanconi Anemia and continuing to advance our clinical programs in Danon Disease, PKP2-ACM and PKD in the year ahead. As we close the quarter, Rocket continues to solidify our industry leadership in gene therapy across multiple therapeutic areas and platforms as we seek to meet the needs of patients living with rare and devastating diseases."

Recent Pipeline and Operational Updates

- **Milestones remain on track for 2024 across pipeline of adeno-associated virus (AAV) cardiovascular and lentiviral (LV) vector hematology portfolios.** The Marketing Authorisation Application (MAA) and Biologics License Application (BLA) for Fanconi Anemia remain on track for filing with the European Medicines Agency (EMA) and U.S. Food and Drug Administration (FDA), respectively, in the first half of 2024. Rocket is also progressing its Phase 2 pivotal study of RP-A501 for Danon Disease, Phase 2 pivotal study of RP-L301 for Pyruvate Kinase Deficiency (PKD), Phase 1 study of RP-A601 for PKP2-arrhythmogenic cardiomyopathy (ACM), and IND-enabling studies for BAG3-associated dilated cardiomyopathy (DCM).
- **KRESLADI ™ for severe Leukocyte Adhesion Deficiency-I (LAD-I) on track for PDUFA date of June 30, 2024**. Based on the positive top-line efficacy and safety data from the global Phase 1/2 study and high unmet need in patients with severe LAD-I, the FDA accepted the BLA and granted Priority Review for KRESLADI ™. On February 13, 2024, Rocket announced the FDA extended the Priority Review period by three months, to June 30, 2024, to allow additional time to review clarifying Chemistry, Manufacturing, and Controls (CMC) information submitted by Rocket in response to FDA information requests. The FDA further confirmed that an advisory committee meeting is not needed. In support of its first product launch, Rocket continues to expand its commercial and operational infrastructure, including Qualified Treatment Center initiation, channel strategy, disease education, and payer engagement.
- **Published data from preclinical study of RP-A601 in PKP2-ACM.** "AAV-Mediated Delivery of Plakophilin-2a Arrests Progression of Arrhythmogenic Right Ventricular Cardiomyopathy in Murine Hearts: Preclinical Evidence Supporting Gene Therapy in Humans" was published in *Circulation: Genomic and Precision Medicine.* Results from the study demonstrated the potential of RP-A601 as a gene therapy for patients with PKP2-ACM.
- **Bolstered commercial and operational expertise of Board of Directors with appointment of R. Keith Woods.** Mr. Woods is a seasoned executive with more than 30 years of experience spanning commercialization, global operations,

business strategy and supply chain. He recently served as Chief Operating Officer of argenx, where he led its global commercial organization, including marketing, market access, medical affairs, program management, and supply chain, during the company's successful transition to commercial stage. Mr. Woods launched Vyvgart, a treatment for a rare autoimmune condition that causes muscle weakness, generating more than $1 billion in sales.

Upcoming Investor Conferences

- Cowen's 44th Annual Health Care Conference: March 5, 2024
- Leerink Partners Global Biopharma Conference: March 12, 2024
- Needham 23rd Annual Virtual Healthcare Conference: April 9, 2024

Fourth Quarter and Full Year 2023 Financial Results

- **Cash position**. Cash, cash equivalents and investments as of December 31, 2023, were $407.5 million.
- **R&D expenses**. Research and development expenses were $41.7 million and $186.3 million for the three and twelve months ended December 31, 2023, respectively, compared to $50.0 million and $165.6 million for the three and twelve months ended December 31, 2022, respectively. The increase in R&D expenses for the twelve months ended December 31, 2023, was primarily driven by increased compensation and benefits expense of $16.9 million due to increased R&D headcount, increased clinical trial costs of $14.5 million, and increased non-cash stock compensation expense of $5.0 million, offset by a decrease in manufacturing and development costs of $17.0 million.
- **G&A expenses**. General and administrative expenses were $21.5 million and $73.3 million for the three and twelve months ended December 31, 2023, respectively, compared to $19.0 million and $58.8 million for the three and twelve months ended December 31, 2022, respectively. The increase in G&A expenses for the twelve months ended December 31, 2023, was primarily driven by increases in commercial preparation expenses which consists of commercial strategy, medical affairs, market development and pricing analysis of $8.4 million and increased non-cash stock compensation expense of $3.4 million.
- **Net loss**. Net loss was $59.7 million and $245.6 million or $0.64 and $2.92 per share (basic and diluted) for the three and twelve months ended December 31, 2023, compared to $66.7 million and $221.9 million or $0.92 and $3.26 per share (basic and diluted) for the three and twelve months ended December 31, 2022.
- **Shares outstanding**. 90,282,267 shares of common stock were outstanding as of December 31, 2023.

Financial Guidance

- **Cash position.** As of December 31, 2023, Rocket had cash, cash equivalents and investments of $407.5 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 gene therapies target hematologic diseases and consist of late-stage programs for Fanconi Anemia, 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 monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia.

Our adeno-associated virus (AAV)-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, 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 December 31,		Twelve Months Ended December 31,	
	2023	2022	2023	2022
Operating expenses:				
Research and development	$ 41,744	$ 50,037	$ 186,342	$ 165,570
General and administrative	21,535	19,044	73,317	58,773
Total operating expenses	63,279	69,081	259,659	224,343
Loss from operations	(63,279)	(69,081)	(259,659)	(224,343)
Research and development incentives	-	500	-	500
Interest expense	(470)	(467)	(1,875)	(1,862)
Interest and other income, net	814	1,245	5,288	3,889
Accretion of discount and amortization of premium on investments, net	3,275	1,081	10,651	(47)
Net loss	$ (59,660)	$ (66,722)	$ (245,595)	$ (221,863)
Net loss per share - basic and diluted	$ (0.64)	$ (0.92)	$ (2.92)	$ (3.26)
Weighted-average common shares outstanding - basic and diluted	93,336,541	72,889,548	84,009,004	68,148,925

	December 31, 2023	December 31, 2022
Cash, cash equivalents, and investments	$ 407,495	$ 399,670
Total assets	566,341	551,807
Total liabilities	73,767	62,121
Total stockholders' equity	492,574	489,686

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