Rocket Pharmaceuticals Announces Buildout of R&D and Manufacturing Facility to Support Development of Innovative Gene Therapy Pipeline

January 11, 2021

—New 103,720 ft^2 Facility to Serve as Headquarters and House 150 Employees in Cranbury, NJ—

—State of the Art R&D Facility to Support Manufacturing Including AAV Drug Product—

—cGMP Production to be Initiated in 2021 for Planned Phase 2 Study of First AAV-based Gene Therapy, RP-A501 for the Treatment of Danon Disease—

CRANBURY, N.J.--(BUSINESS WIRE)--Jan. 11, 2021-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders, today announces the Company's plans for the buildout of its new Research and Development (R&D) and Chemistry, Manufacturing and Controls (CMC) operation which will also serve as the Company's new headquarters in Cranbury, New Jersey. This new 103,720 ft^2 facility will support clinical development of Rocket's growing pipeline of lentivirus (LV) and adeno-associated virus (AAV) gene therapies from discovery through pivotal trials, with space for potential future expansion and commercialization. This buildout comes on the heels of the Company's recent successful capital raise of approximately $300 million that provides a cash runway into the second half of 2023.

This press release features multimedia. View the full release here: https://www.businesswire.com/news/home/20210111005331/en/

Rocket Pharmaceuticals Headquarters (Photo: Business Wire)

"Investing in R&D and manufacturing innovation, talent, and capacity through this new world-class facility will allow us to deliver on our mission of bringing five curative gene therapies to rare disease patients by 2025," said Gaurav Shah, M.D., President and Chief Executive Officer of Rocket. "With data on five clinical programs expected this year, including two that are in registration-enabling trials, these new capabilities will enable us to work with continued urgency and purpose towards bringing transformational therapies to patients."

"We are collaborating with some of the best scientists and innovators worldwide. This new facility is instrumental in bolstering Rocket's gene therapy capabilities to rapidly advance multiple platforms and programs efficiently and effectively. Producing clinical drug product will enable greater control of supply, cost, quality, and timing to pave a smoother path toward commercialization," added Kinnari Patel, Pharm.D., MBA, Chief Operating Officer and Head of Development.

Approximately one-half of the facility is being scaled for AAV Current Good Manufacturing Practice (cGMP) production. The other half features state-of-the-art R&D labs to support the expanding pipeline and Quality Control (QC) laboratories to support CMC development for process and analytics.

Rocket recently reported positive interim Phase 1 results for its first AAV-based gene therapy, RP-A501 for the treatment of Danon Disease. The first cGMP production at this facility will be initiated in 2021 and will be used in a planned Phase 2 registrational study evaluating RP-A501, following the completion of the current Phase 1 trial.

Approximately $300 million secured in public equity offering

On December 14, 2020, Rocket closed an upsized underwritten public offering of 6,035,714 shares of its common stock, inclusive of greenshoe, at the public offering price of $56.00 per share. The offering was ~7.3x oversubscribed based on the initial deal size of $175 million. Rocket intends to use the net proceeds from this offering to further fund the development of its pipeline of gene therapies for rare diseases, including filing for marketing authorization for RP-L201 in the U.S. and Europe, accelerate the buildout of in-house manufacturing capabilities, and for general corporate purposes. This capital raise extends Rocket's cash runway to the second half of 2023.

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, Pyruvate Kinase Deficiency (PKD) a rare, monogenic red blood cell disorder resulting in increased red cell destruction and mild to life-threatening anemia, and Infantile Malignant Osteopetrosis (IMO), a bone marrow-derived disorder. 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 the safety, effectiveness and timing of product candidates that Rocket may develop, to treat Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Pyruvate Kinase Deficiency (PKD), Infantile Malignant Osteopetrosis (IMO) and Danon Disease, 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 successfully demonstrate the efficacy and safety of such products and pre-clinical studies and clinical trials, its gene therapy programs, the preclinical and clinical results for its product candidates, which may not support further development and marketing approval, the potential advantages of Rocket's product candidates, 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 and its licensors’ ability to obtain, maintain and protect its and their respective intellectual property, the timing, cost or other aspects of a potential commercial launch of Rocket's product candidates, Rocket's ability to manage operating expenses, Rocket's ability to obtain additional funding to support its business activities and establish and maintain strategic business alliances and new business initiatives, 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 Quarterly Report on Form 10-Q for the quarter ended September 30, 2019, filed November 8, 2019 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.

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