Rocket Pharmaceuticals to Present at William Blair Biotech Focus Conference

July 8, 2022

CRANBURY, N.J.--(BUSINESS WIRE)--Jul. 8, 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 announces that Gaurav Shah, M.D., Chief Executive Officer, will participate in a fireside chat at the William Blair Biotech Focus Conference 2022 on July 12-13, 2022, at the St. Regis Hotel in New York City. Management will also be hosting one-on-one meetings with investors.

The pre-recorded fireside chat will be available beginning Monday, July 11, 2022, at 9:00 a.m. ET. A link to the recording will be available under "Events" in the Investors section of the Company's website at https://ir.rocketpharma.com. The webcast replay will be available to view for 30 days via the Rocket website following the conference.

The presentation will highlight the Company's pipeline of first-in-class gene therapies that incorporate both adeno-associated viral vector (AAV) and lentiviral vector (LVV) approaches to gene therapy. The Company recently announced positive top-line safety and efficacy data from its Phase 2 pivotal trial for severe Leukocyte Adhesion Deficiency-I (LAD-I), as well positive clinical updates from its Phase 2 pivotal trial for Fanconi Anemia (FA) and Phase 1 trials for Danon Disease and Pyruvate Kinase Deficiency (PKD).

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 that 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, and Rocket's plans for the advancement of its LAD-I program based on the data presented at ASGCT and the potential for therapeutic benefit related thereto, 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.

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