Rocket Pharmaceuticals Highlights Anticipated 2022 Milestones Across Lentiviral and AAV Gene Therapy Clinical Programs

January 11, 2022

— Top-line data from pivotal LAD-I and Fanconi Anemia trials anticipated in Q2 and Q3, respectively —

— Danon Disease pediatric patient cohort data expected Q3 —

— In-house AAV GMP manufacturing initiation anticipated in Q2 —

— Danon Disease pivotal Phase 2 trial initiation planned for Q4 —

— PKD data from Phase 1 and pivotal Phase 2 trial initiation planned for Q4 —

CRANBURY, N.J.--(BUSINESS WIRE)--Jan. 11, 2022-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders, today shares expected milestones for 2022, which were discussed yesterday during the Company's virtual presentation at the 40 th Annual J.P. Morgan Healthcare Conference.

"The Rocket team together with our partners made significant progress across our pipeline of first-and best-in-class lentiviral and AAV gene therapies in 2021," said Kinnari Patel, Pharm.D., MBA, President and Chief Operating Officer of Rocket Pharma. "We are excited to share our expected milestones for 2022 as we progress toward potential near-term U.S. and EU regulatory filings for our Leukocyte Adhesion Deficiency-I (LAD-I) and Fanconi Anemia programs as well as continue our strong progress across our pipeline. Notably, with the most recently treated patient now engrafting with neutrophil CD18 expression of 61% at three months, all nine severe LAD-I patients who have been treated with RP-L201 in our pivotal Phase 2 trial with at least three months of follow-up have engrafted with CD18 expression ranging from 26% to 87% of normal."

Dr. Patel continued, "As our AAV-based gene therapy for Danon Disease advances in the clinic including anticipated data from our pediatric cohort in Q3, we continue to ready our in-house AAV manufacturing capabilities in our Cranbury, New Jersey facility to support commercial product. We are excited for the year ahead and look forward to continuing to leverage Rocket's strong team and resources to drive our vision of seeking and delivering gene therapy cures for patients facing such rare and devastating diseases."

Anticipated 2022 Milestones

RP-A501 for Danon Disease (AAV)

- Report data from pediatric patient cohort of Phase 1 trial – Q3 2022
- Initiate Phase 2 pivotal study activities – Q4 2022

RP-L102 for Fanconi Anemia (LVV)

- Report top-line data from pivotal Phase 2 trial – Q3 2022

RP-L201 for Leukocyte Adhesion Deficiency-I (LVV)

- Report top-line data from pivotal Phase 2 trial – Q2 2022

RP-L301 for Pyruvate Kinase Deficiency (LVV)

- Report data from Phase 1 trial – Q4 2022
- Initiate pivotal Phase 2 trial – Q4 2022

Manufacturing Facility in Cranbury, New Jersey

- Achieve in-house AAV Good Manufacturing Practice (GMP) manufacturing – Q2 2022

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, 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, 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, 2020, filed March 1, 2021 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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