Rocket Pharmaceuticals Provides Business Update in Light of COVID-19 Pandemic

April 2, 2020

—Continue to Believe We Will Achieve 2020 Milestones—

— Balance Sheet is Strong; Company Capital Will Be Sufficient into 2022 —

NEW YORK--(BUSINESS WIRE)--Apr. 2, 2020-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a clinical-stage company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders, today provides a business update in light of the COVID-19 pandemic. The Company has taken steps to ensure the safety of all patients, families and employees during this challenging time and continues to monitor potential impact on business operations. While there have been operational delays due to the pandemic, Rocket believes that it will achieve its 2020 milestones.

There is strong interest from patients and families for participation in each of Rocket's ongoing trials. Although many clinical trial sites have cut back non-essential activities, Rocket's programs are structured such that resumption of normal operations within a few months at hospitals is not anticipated to result in meaningful delays to guidance regarding both anticipated data readouts and overall enrollment and registration plans. In addition, Rocket currently has sufficient vector inventory from its suppliers for each of the ongoing lentiviral programs, and cell processing is currently operating without interruption. Similarly, the Company has already manufactured Phase 1 study drug product for its adeno-associated virus (AAV) program through contract manufacturers.

"In the face of many uncertainties, ensuring patient health and safety is our utmost priority. We are working creatively with our partners to drive our pipeline forward in the face of evolving COVID-19 related logistical challenges," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "We remain cautious during this challenging period since none of us know the precise interval required to resume normal business activities. Assuming things return to normal within a few months, we believe our milestones will remain on track. Should it take longer, there may be risk to our timelines. We look forward to updating you as we learn more."

Rocket is tracking the global coronavirus pandemic closely and will respond accordingly with respect to the safety of our patients and the continuity of business operations. As the state of affairs continues to evolve, Rocket will provide updates as appropriate.

Rocket Guidance

At this time, Rocket maintains its current guidance. We expect to update this guidance on May 6, 2020 when the Company reports financial results for the first quarter of 2020.

Anticipated Milestones

- **FA (RP-L102)**
  - Additional data update (2Q)
  - Preliminary Phase 2 data (2H)
- **Danon Disease (RP-A501)**
  - Advancing to next cohort (2Q)
  - Phase 1 data (2H)
- **LAD-I (RP-L201)**
  - Phase 1 data update (2H)
  - Initiate Phase 2 study (2H)
- **PKD (RP-L301)**
  - First patient treatment (2Q)
  - Preliminary Phase 1 data (2H)
- **IMO (RP-L401)**
  - Initiation of clinical study (2H)

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) (“Rocket”) 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 contending 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. Rocket’s pre-clinical pipeline program is for Infantile Malignant Osteopetrosis (IMO), a bone marrow-derived disorder. 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 2020 in light of COVID-19, 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 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 when clinical trial sites will resume normal business operations, 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, 2019, filed March 6, 2020 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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Source: Rocket Pharmaceuticals, Inc.