Rocket Pharmaceuticals Expands Clinical Sites for its FA, Danon, LAD-I and IMO Trials, Adding to its Global Centers of Excellence

August 26, 2020

—Four Clinical Trial Sites Added Across Multiple Clinical Programs Including Great Ormond Street Hospital, Children's Hospital of Philadelphia, University of Minnesota and University of California, Los Angeles—

—New Sites Expand Opportunities for Patient Access, Enrollment and Overall Presence Globally—

NEW YORK--(BUSINESS WIRE)--Aug. 26, 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 announces the addition of four additional clinical trial sites across four clinical programs for Fanconi Anemia (FA), Leukocyte Adhesion Deficiency-I (LAD-I), Danon Disease and Infantile Malignant Osteopetrosis (IMO). The newly added centers, which include some of the leading gene therapy research programs in the world, are: Great Ormond Street Hospital (GOSH), Children's Hospital of Philadelphia (CHOP), the University of Minnesota and the University of California, Los Angeles (UCLA).

"Rocket is pleased to add these world-class sites which represents another meaningful step in our journey and vision of having Centers of Excellence worldwide," said Kinnari Patel, Pharm.D., MBA, Chief Operating Officer and Head of Development of Rocket. "We have been working with prominent and experienced research centers to broaden our footprint to new geographies for these trials and the entire pipeline overall. These sites represent not only world-class partners for expanding patient enrollment but ideal partners for any future potential commercialization. We are confident that the exceptional principal investigators at each of these sites will strengthen our already robust clinical trial activities and expand access for patients contending with rare and devastating disorders."

Great Ormond Street Hospital in London has opened as a clinical trial site for Rocket's FA and LAD-I programs. Dr. Claire Booth, Mahboubian Associate Professor in Gene Therapy University College London Great Ormond Street Institute of Child Health (UCL GOS ICH) and Honorary Consultant in Pediatric Immunology, GOSH, will serve as principal investigator at the site. GOSH, together with its academic partner the UCL GOS ICH forms the largest concentration of children's health research in Europe. The LAD-I trial first opened at the University of California, Los Angeles, and two patients have already been treated in the Phase 1 trial.

As Rocket moves into the next cohort in its clinical trial of RP-A501 for the treatment of Danon Disease, the world-renowned Children's Hospital of Philadelphia will join the University of California San Diego (UCSD) Health as a trial site under the leadership of Joseph Rossano, M.D., M.S., FAAP, FACC, Chief of the Division of Cardiology at CHOP and Professor of Pediatrics at Perelman School of Medicine at the University of Pennsylvania. With this addition, Rocket brings an East Coast presence to the trial, facilitating broader patient access.

Rocket's global registrational trial of RP-L102 for FA continues to progress and will expand to the University of Minnesota under the leadership of Margaret MacMillan, M.D., MSc., FRCPC, Professor in Pediatrics in the Division of Blood and Marrow Transplantation at the University of Minnesota Medical School.

In addition, the trial site for the recently launched Phase 1 clinical trial of RP-L401 for IMO will be University of California, Los Angeles led by principal investigator Donald B. Kohn, M.D., Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics (Hematology/Oncology), Molecular and Medical Pharmacology, member of the Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research at the University of California, Los Angeles and principal investigator of RP-L201 for LAD-I. The presence of UCLA and addition of Dr. Kohn are major steps forward in Rocket's continued evaluation of RP-L401 as a treatment for IMO.

The collaborations with GOSH/ICH and University of Minnesota further leverage the ongoing consultative expertise of Adrian Thrasher, Ph.D., M.D. and John Wagner, M.D., respectively. Professor Thrasher is Professor of Pediatric Immunology and Wellcome Trust Principal Research Fellow at the ICH. Dr. Wagner is Director of the Institute of Cell, Gene and Immunotherapeutics at the University of Minnesota. Since 2016, Professor Thrasher has collaborated with Rocket regarding multiple lentiviral programs and lends valuable insight through his role at the largest concentration of children's health research in Europe. Dr. Wagner is an internationally recognized expert in FA, stem cell and transplantation biology, and umbilical cord transplant. Dr. Wagner has been closely involved in Rocket's FA program since 2018.

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 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 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-Q for the quarter ended June 30, 2020, filed August 5, 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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