Rocket Pharmaceuticals Appoints Gayatri R. Rao, M.D., J.D., Former U.S. Food and Drug Administration Director of the Office of Orphan Products Development, as Vice President, Regulatory Policy and Patient Advocacy

May 15, 2018

NEW YORK--(BUSINESS WIRE)--May 15, 2018-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a leading U.S.-based multi-platform gene therapy company, today announced the appointment of Gayatri R. Rao, M.D., J.D., as Vice President, Regulatory Policy and Patient Advocacy. In this new role, Dr. Rao will provide critical direction to global regulatory policies and strategies for Rocket programs to bring multiple therapies to patients with rare and devastating diseases. This will include oversight of regulatory strategy, patient advocacy initiatives, and rare disease natural history studies supporting Rocket's programs.

Dr. Rao most recently served as Director of the Office of Orphan Products Development (OOPD) within the U.S. Food and Drug Administration (FDA) where she was responsible for implementing statutory programs focused on promoting the development of medical products for rare diseases. As Director, Dr. Rao served as the FDA's rare disease lead for interactions with Congress, international regulatory counterparts, other federal agencies, press, sponsors, and patient advocacy groups. Dr. Rao previously was an Associate Chief Counsel in FDA's Office of Chief Counsel where she advised on a variety of issues related to clinical trials, medical devices, and combination products. She began her career at an international law firm in Washington, D.C., where she focused on healthcare and food and drug law related matters. She holds a J.D. from the University of Pennsylvania Law School, a Master of Bioethics from the University of Pennsylvania School of Medicine, and an M.D. from Rutgers New Jersey Medical School.

"Rocket is pleased to welcome Dr. Rao during a particularly exciting time as we look forward to presenting updated data from our Fanconi Anemia (FA) program later this week at the American Society of Gene & Cell Therapy (ASGCT) Annual Meeting," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "Dr. Rao's accomplished track record of working at the FDA within the Office of Orphan Products Development will be very valuable for Rocket in shaping the strategies for the multiple regulatory filings that we have planned, including the global registration trial for FA."

Kinnari Patel, Pharm.D., MBA, Chief Operating Officer & Head of Development of Rocket commented, "Dr. Rao's commitment to rare disease product development aligns strongly with Rocket's mission of finding curative therapies for patients with devastating diseases. In addition, Dr. Rao's extensive experience in patient advocacy will be particularly important for building the natural history studies, which constitute an essential scientific foundation for effective clinical development programs. This is especially the case in drug development for rare diseases, for which comprehensive understanding of clinical pathogenesis is often lacking."

About Rocket Pharmaceuticals, Inc.
Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket") is an emerging, clinical-stage biotechnology company focused on developing first-in-class gene therapy treatment options for rare, devastating diseases. Rocket's multi-platform development approach applies the well-established lentiviral vector (LVV) and adeno-associated viral vector (AAV) gene therapy platforms. Rocket's lead clinical program is a LVV-based gene therapy for the treatment of Fanconi Anemia (FA), a difficult to treat genetic disease that leads to bone marrow failure and potentially cancer. Preclinical studies of additional bone marrow-derived disorders are ongoing and target Pyruvate Kinase Deficiency (PKD), Leukocyte Adhesion Deficiency-I (LAD-I) and Infantile Malignant Osteopetrosis (IMO). Rocket is also developing an AAV-based gene therapy program for an undisclosed rare pediatric disease. For more information about Rocket, please visit www.rocketpharma.com.

View source version on businesswire.com: https://www.businesswire.com/news/home/20180515005165/en/

Source: Rocket Pharmaceuticals, Inc.

Claudine Prowse, Ph.D.
SVP Corporate Development and IRO
Rocket Pharmaceuticals, Inc.
The Alexandria Center for Life Science
430 East 29 Street, Suite 1040
New York, NY 10016
cp@rocketpharma.com