Rocket Pharmaceuticals Announces Presentations Highlighting AAV and Lentiviral Gene Therapies at Upcoming Scientific Congresses

November 3, 2022

CRANBURY, N.J.--(BUSINESS WIRE)--Nov. 3, 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 upcoming data presentations at the 75th American Heart Association (AHA) Annual Meeting, taking place in Chicago, Illinois, and virtually, from Nov. 5-7, 2022, and the 64th American Society of Hematology (ASH) Annual Meeting, taking place in New Orleans, Louisiana, and virtually, from Dec. 10-13, 2022. At AHA, the Company will share previously disclosed data from the Phase 1 trial of RP-A501, its adeno-associated virus (AAV)-based gene therapy for Danon Disease, in an oral presentation. During ASH, the Company will present poster presentations sharing data from its lentiviral vector (LV)-based gene therapy programs. Updated safety and efficacy data will be presented from the Phase 2 pivotal trial of RP-L102 for Fanconi Anemia and Phase 1 trial of RP-L301 for Pyruvate Kinase Deficiency (PKD). Previously disclosed data will be presented from the Phase 2 pivotal trial of RP-L201 for Leukocyte Adhesion Deficiency-I.

AHA Presentation Details

Title: Phase 1 Danon Disease Results: The First Single Dose Intravenous (IV) Gene Therapy (RP-A501) with Recombinant Adeno-associated Virus (AAV9:LAMP2B) for a Monogenic Cardiomyopathy
Session: Genetics and Mitochondria in Congenital Heart Disease and Pediatric Cardiology
Presenter: Joseph Rossano, M.D., M.S., FAAP, FACC, Co-Director of the Cardiac Center and Chief of the Division of Cardiology at Children's Hospital of Philadelphia
Session date and time: Saturday, November 5, 2022, 4:30 p.m. - 5:30 p.m. CT
Location: McCormick Place Convention Center, Chicago
Presentation number: 213

ASH Presentation Details

Title: Lentiviral-mediated Gene Therapy for Adults and Children with Severe Pyruvate Kinase Deficiency: Results from an Ongoing Global Phase 1 Study
Session: Gene Therapies: Poster I
Presenter: Ami J. Shah, M.D. - Clinical Professor of Pediatrics, Division of Hematology/ Oncology, Stem Cell Transplantation and Regenerative Medicine, Stanford University School of Medicine
Session date and time: Saturday, December 10, 2022, 5:30 p.m. - 7:30 p.m. CT
Location: Ernest N. Morial Convention Center, Hall D, New Orleans
Presentation number: 2138

Title: Interim Results from an Ongoing Phase 1/2 Study of Lentiviral-mediated *Ex-vivo* Gene Therapy for Pediatric Patients with Severe Leukocyte Adhesion Deficiency-I (LAD-I)
Session: Gene Therapies: Poster II
Presenter: Donald B. Kohn, M.D., Distinguished Professor of Microbiology, Immunology & Molecular Genetics (MIMG), Pediatrics and Molecular & Medical Pharmacology; Director of the UCLA Human Gene and Cell Therapy Program, University of California, Los Angeles
Session date and time: Sunday, December 11, 2022, 6:00 p.m. - 8:00 p.m. CT
Location: Ernest N. Morial Convention Center, Hall D, New Orleans
Presentation number: 3460

Title: Lentiviral-mediated Gene Therapy for Patients with Fanconi Anemia [Group A]: Updated Results from Global RP-L102 Clinical Trials
Session: Gene Therapies: Poster III
Presenter: Agnieszka Czechowicz, M.D., Ph.D., Department of Pediatrics, Division of Hematology/ Oncology, Stem Cell Transplantation and Regenerative Medicine, Stanford University School of Medicine
Session date and time: Monday, December 12, 2022, 6:00 p.m. - 8:00 p.m. CT
Location: Ernest N. Morial Convention Center, Hall D, New Orleans
Presentation number: 4775

Details for the poster presentation on a corollary study are as follows:

Title: Hematopoietic and Immunological Assessment in Fanconi Anemia after *Ex-vivo* Lentiviral FANCA Gene Therapy with RP-L102
Session: Gene Therapies: Poster II
Presenter: Rofida Nofal, M.D., Department of Pediatrics, Division of Hematology/Oncology, Stem Cell Transplantation and Regenerative Medicine, Stanford University School of Medicine
Session date and time: Sunday, December 11, 2022, 6:00 p.m. - 8:00 p.m. CT
Location: Ernest N. Morial Convention Center, Hall D, New Orleans

About Danon Disease

Danon Disease is a rare X-linked inherited disorder caused by mutations in the gene encoding lysosome-associated membrane protein 2 (LAMP-2), an important mediator of autophagy. This results in accumulation of autophagosomes and glycogen, particularly in cardiac muscle and other tissues, which ultimately leads to heart failure, and for male patients, frequent death during adolescence or early adulthood. It is estimated to have a prevalence of 15,000 to 30,000 patients in the U.S. and Europe. The only available treatment option for Danon Disease is cardiac transplantation, which is associated with substantial complications and is not considered curative. There are no specific therapies available for the treatment of Danon Disease.

About Fanconi Anemia

Fanconi Anemia (FA) is a rare pediatric disease characterized by bone marrow failure, malformations and cancer predisposition. The primary cause of death among patients with FA is bone marrow failure, which typically occurs during the first decade of life. Allogeneic hematopoietic stem cell transplantation (HSCT), when available, corrects the hematologic component of FA, but requires myeloablative conditioning. Graft-versus-host disease, a known complication of allogeneic HSCT, is associated with an increased risk of solid tumors, mainly squamous cell carcinomas of the head and neck region. Approximately 60-70% of patients with FA have a Fanconi Anemia complementation group A (*FANCA*) gene mutation, which encodes for a protein essential for DNA repair. Mutations in the *FANCA* gene leads to chromosomal breakage and increased sensitivity to oxidative and environmental stress. Increased sensitivity to DNA-alkylating agents such as mitomycin-C (MMC) or diepoxybutane (DEB) is a ‘gold standard’ test for FA diagnosis. Somatic mosaicism occurs when there is a spontaneous correction of the mutated gene that can lead to stabilization or correction of a FA patient’s blood counts in the absence of any administered therapy. Somatic mosaicism, often referred to as ‘natural gene therapy’ provides a strong rationale for the development of FA gene therapy because of the selective growth advantage of gene-corrected hematopoietic stem cells over FA cells.

About Leukocyte Adhesion Deficiency-I

Severe Leukocyte Adhesion Deficiency-I (LAD-I) is a rare, autosomal recessive pediatric disease caused by mutations in the *ITGB2* gene encoding for the beta-2 integrin component CD18. CD18 is a key protein that facilitates leukocyte adhesion and extravasation from blood vessels to combat infections. As a result, children with severe LAD-I are often affected immediately after birth. During infancy, they suffer from recurrent life-threatening bacterial and fungal infections that respond poorly to antibiotics and require frequent hospitalizations. Children who survive infancy experience recurrent severe infections including pneumonia, gingival ulcers, necrotic skin ulcers, and septicemia. Without a successful bone marrow transplant, mortality in patients with severe LAD-I is 60-75% prior to the age of 2 and survival beyond the age of 5 is uncommon. There is a high unmet medical need for patients with severe LAD-I.

Rocket’s LAD-I research is made possible by a grant from the California Institute for Regenerative Medicine (Grant Number CLIN2-11480). The contents of this press release are solely the responsibility of Rocket and do not necessarily represent the official views of CIRM or any other agency of the State of California.

About Pyruvate Kinase Deficiency

Pyruvate kinase deficiency (PKD) is a rare, monogenic red blood cell disorder resulting from a mutation in the *PKLR* gene encoding for the pyruvate kinase enzyme, a key component of the red blood cell glycolytic pathway. Mutations in the *PKLR* gene result in increased red cell destruction and the disorder ranges from mild to life-threatening anemia. PKD has an estimated prevalence of 4,000 to 8,000 patients in the United States and the European Union. Children are the most commonly and severely affected subgroup of patients. Currently available treatments include splenectomy and red blood cell transfusions, which are associated with immune defects and chronic iron overload.

RP-L301 was in-licensed from the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT), Centro de Investigación Biomédica en Red de Enfermedades Raras (CIBERER) and Instituto de Investigación Sanitaria de la Fundación Jiménez Díaz (IIS-FJD).

About Rocket Pharmaceuticals, Inc.

Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) is advancing an integrated and sustainable pipeline of investigational genetic therapies designed to 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, the expected timing and outcome of Rocket’s regulatory interactions and planned submissions, Rocket’s plans for the advancement of its Danon Disease program 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, 2021, filed February 28, 2022 with the SEC and subsequent filings with the SEC including our Quarterly Reports on Form 10-Q. 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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Media
Kevin Giordano
Director, Corporate Communications
kgiordano@rocketpharma.com

Investors
Jessie Yeung, M.B.A.
Vice President, Investor Relations and Corporate Finance
investors@rocketpharma.com

Source: Rocket Pharmaceuticals, Inc.