Rocket Pharmaceuticals Announces Publication of Data from Phase 1/2 Trial of First-Generation RP-L102 for Fanconi Anemia in Nature Medicine

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—First Demonstration of Successful Engraftment of Gene Corrected Hematopoietic Stem Cells Without the Use of Conditioning—

NEW YORK--(BUSINESS WIRE)--Sep. 10, 2019-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT) ("Rocket"), a leading U.S.-based multi-platform clinical-stage gene therapy company, today announces the publication of long-term data from the ongoing Phase 1/2 trial of RP-L102, the Company's lentiviral vector (LVV)-based gene therapy for Fanconi Anemia (FA) in the journal *Nature Medicine*. The data included in the manuscript are from the first four patients treated with RP-L102 in the Phase 1/2 FANCOLEN-I trial that utilized first-generation "Process A" without the use of any conditioning regimen. Follow-up for each of the initial four patients was 18-30 months from administration of RP-L102.

"Data from our first trial of RP-L102 demonstrate increasing levels of bone marrow engraftment, leading to stabilization and restored bone marrow function. These data highlight the natural selective advantage that uniquely exists in FA for gene corrected stem cells over diseased stem cells, which potentially obviates the need for conditioning," said Jonathan Schwartz, M.D., Chief Medical Officer and Senior Vice President of Rocket. "At the end of the year, we will have a first look at initial data from our Phase 1 trial of 'Process B' RP-L102, which utilizes fresh cells and incorporates a modified stem cell enrichment process, transduction enhancers, and commercial-grade vector and final drug product. We are also excited by the prospect of starting our global registrational trial incorporating recent alignment on endpoints from both the U.S. Food and Drug Administration and European Medicines Agency."

"There is an increased and urgent need for new therapies for patients and families suffering from FA as current treatments are limited to toxic and burdensome bone marrow transplant," said Paula Río, Ph.D., Senior Scientist, División de Terapias Innovadoras en el Sistema Hematopoyético, CIEMAT/CIBERER Unidad Mixta de Terapias Avanzadas CIEMAT/IIS Fundación Jiménez Díaz, and co-first author of the manuscript. "We are very pleased to see long-term follow-up data that further support our thesis for RP-L102 gene therapy without any conditioning to serve as an innovative, low-toxicity treatment for the hematologic component of this devastating disease."

The data included in the manuscript are from four pediatric patients (ages 3-6 years) who received RP-L102 utilizing fresh or cryopreserved CD34+ cells that were collected and transduced. Patients 02002 and 02006 were treated with higher dose levels of RP-L102. Patients 02004 and 02005 received non-optimized and lower doses of RP-L102. Key highlights of the manuscript include:

- Follow-up data for the initial four patients 18-30 months post-infusion demonstrate progressively increased engraftment in peripheral blood leukocytes and in the bone marrow following administration of RP-L102 without the use of conditioning.
- In Patient 02002 at 30 months follow-up, approximately 44% of bone marrow CD34+ cells displayed gene marking, suggesting the engraftment of very primitive corrected hematopoietic stem cells (HSCs).
- Sequential increases in gene marking in peripheral blood and in the bone marrow for Patients 02004 and 02005 were also seen, but at more modest levels and after longer durations.
- Phenotypic correction of bone marrow cells was measured by resistance to mitomycin-C (MMC) in colony forming cells. The bone marrow resistance to MMC in Patient 02002 increased to 70% at 24 months, approaching the phenotype of a healthy donor. Patients 02004, 02005 and 02006 also displayed progressive increases in MMC resistance.
- Phenotypic correction of blood cells was measured by chromosomal stability of T-lymphocytes in the presence of diepoxybutane (DEB). DEB exposure resulted in a lower proportion of cells with aberrant chromosomes in Patients 02002, 02004 and 02006.
- Hematologic correction was measured by changes in previously declining pre-treatment blood count trajectories, which were evident in at least two peripheral blood lineages for each of the four patients. Patient 02002 demonstrated stabilized neutrophil counts and hemoglobin levels as early as six months post-administration of RP-L102. Similar trends were also seen in Patient 02006.
- Progressive increases in the total number of corrected leukocytes were observed shortly after the initial administration of RP-L102 in all treated patients.

- Favorable safety profile with no serious adverse events associated with infusion of the investigational product in these initial four patients.

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 *FANC-A* gene mutation, which encodes for a protein essential for DNA repair. Mutation in the *FANC-A* gene leads to chromosomal breakage and increased sensitivity to oxidative and environmental stress. Chromosome fragility induced by DNA-alkylating agents such as mitomycin-C (MMC) or diepoxybutane (DEB) is the '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 'nature's 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[1].

[1]Soulier, J.,et al. (2005) Detection of somatic mosaicism and classification of Fanconi anemia patients by analysis of the FA/BRCA pathway. Blood 105: 1329-1336

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 first two clinical programs using 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, and Leukocyte Adhesion Deficiency-I (LAD-I), a severe pediatric genetic disorder that causes recurrent and life-threatening infections which are frequently fatal. Rocket's first clinical program using AAV-based gene therapy is for Danon disease, a devastating, pediatric heart failure condition. Rocket's pre-clinical pipeline programs for bone marrow-derived disorders are for Pyruvate Kinase Deficiency (PKD) and Infantile Malignant Osteopetrosis (IMO). 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 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 successfully demonstrate the efficacy and safety of such products and pre-clinical studies and clinical trials, its gene therapy programs, the pre-clinical and clinical results for its product candidates, which may not support further development and marketing approval, the potential advantages of Rocket's product candidates, 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 and its licensors' ability to obtain, maintain and protect its and their respective intellectual property, the timing, cost or other aspects of a potential commercial launch of Rocket's product candidates, Rocket's ability to manage operating expenses, Rocket's ability to obtain additional funding to support its business activities and establish and maintain strategic business alliances and new business initiatives, 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, 2018. 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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