Rocket Pharmaceuticals Expands Cardiac Gene Therapy Portfolio with Addition of RP-A601 for PKP2-ACM and Announces Positive Updated Phase 1 Data for RP-A501 in Danon Disease

January 9, 2023

RP-A601 for Arrhythmogenic Cardiomyopathy due to PKP2 pathogenic variants (PKP2-ACM) represents meaningful commercial opportunity with estimated prevalence of 50,000 adults and children in the U.S. and EU; IND filing anticipated in Q2 2023

In support of Phase 2 pivotal study, latest positive Phase 1 data in Danon Disease recently shared with FDA demonstrate improvements in all biomarker and clinical endpoints across pediatric and adult patients with marked difference vs. natural history; two cGMP manufacturing runs completed with high product quality at in-house facility

BLA filings for LAD-I and FA on track for Q2 2023 and Q4 2023, respectively

Well-capitalized to develop full pipeline of assets with $401M (preliminary, unaudited) in cash and cash equivalents; operational runway now expected through 2024 (inclusive of PKP2-ACM program)

CRANBURY, N.J.--(BUSINESS WIRE)--Jan. 9, 2023-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a leading late-stage biotechnology company advancing an integrated and sustainable pipeline of genetic therapies for rare childhood disorders with high unmet need, today announces the addition of RP-A601 to Rocket's cardiac gene therapy portfolio as well as anticipated highlights for the year ahead across the Company's world-class pipeline of lentiviral and AAV gene therapy programs targeting rare hematologic and cardiovascular diseases. These announcements and anticipated highlights will be presented at the 41st Annual J.P. Morgan Healthcare Conference today at 2:15 p.m. PT by Gaurav Shah, M.D., Chief Executive Officer, Rocket Pharma.

"I am extremely excited to build on our significant progress in 2022 as we advance six programs with compelling clinical and/or preclinical proof of concept, including the addition of RP-A601 for the treatment of arrhythmogenic cardiomyopathy due to plakophilin 2 pathogenic variants (PKP2-ACM), expanding our industry-leading cardiovascular AAV gene therapy portfolio. Equally, we look forward to submitting our first regulatory filings, for Fanconi Anemia and Leukocyte Adhesion Deficiency (LAD-I), this year," said Dr. Shah. "RP-A601 for ACM aims to address the unmet needs of approximately 50,000 adults and children in the U.S. and EU who face this devastating genetic heart disease marked by arrhythmias and heart failure that often results in sudden cardiac death. We are moving this program forward into the clinic having demonstrated robust preclinical proof of concept using an rh74 serotype that has been associated with a favorable safety profile in the clinic for other diseases. We anticipate being first to clinic with a submission of an IND for RP-A601 in the second quarter of 2023 for a multi-center, dose-escalation study for the treatment of PKP2-ACM."

Dr. Shah continued, "Additionally, positive data updates today in Danon Disease include several months of additional Phase 1 results that demonstrate further robust improvements in all biomarkers, and in how both adult and pediatric patients function and feel, that diverge meaningfully from natural history patients of similar age and disease characteristics. We have shared these updates with the FDA as part of our pivotal Phase 2 study design discussions. Additionally, we have successfully produced two Danon AAV cGMP batches at our Cranbury, N.J. facility with improved product specifications versus Phase 1 thus taking another essential step towards commercial readiness."

"Clinical programs from our hematology portfolio, represented by our Phase 2 pivotal studies in Fanconi Anemia and LAD-I, remain on track. We are laser focused on regulatory submissions, with BLA filings anticipated for LAD-I and FA in the second quarter and fourth quarter of 2023, respectively," said Dr. Shah. "Lastly, I am pleased to note an extension of our cash runway, which we expect to fund operations through 2024. I am incredibly pleased with our progress, and excited about the catalyst-rich year ahead as we continue on our path to becoming a fully integrated rare disease and gene therapy company with capabilities extending from discovery through manufacturing and commercialization."

RP-A601 for PKP2 Arrhythmogenic Cardiomyopathy on Track for IND Submission in Q2 2023

- ACM, or arrhythmogenic right ventricular dysplasia (ARVC), due to plakophilin 2 pathogenic variants (PKP2-ACM), is a high-risk cardiomyopathy caused by autosomal dominant mutations in the *PKP2* gene. ACM is characterized by frequent and life-threatening ventricular arrhythmias and structural ventricular myopathy. Available treatments fail to address the underlying genetics and disease biology and do not alter disease progression. PKP2-ACM affects approximately 50,000 people in the U.S. and EU.

- Preclinical proof of concept from a translationally relevant animal model has been demonstrated following Rocket-sponsored studies with academic partners at NYU Grossman School of Medicine. The preclinical studies with a cardiomyocyte-specific PKP2 knockout mouse model of ACM evaluated initial proof of concept and dose-related effects of AAV vectors, including survival, functional and anatomic benefits. Notably, studies evaluated the delivery of AAV at seven and 14 days following induction of PKP2 knockout and subsequent disease onset.
- Results demonstrated increased survival and preserved cardiac function in the PKP2 knockout mouse model.
    - 100% of adult PKP2 knockout mice receiving RP-A601 seven days after knockout induction demonstrated survival to the five-month duration of the evaluation compared to 100% mortality by approximately day 50 in PKP2 knockout mice receiving formulation control. PKP2 knockout mice receiving RP-A601 were observed with preserved ejection fraction and right ventricular area at 28 days, sustained to five months.
    - Fourteen days following RP-A601 administration, PKP2 knockout mice demonstrated robust survival with a similar degree of cardiac benefit to five months. RP-A601 was also associated with mitigation of isoproterenol-induced PVCs and arrhythmias, which are major morbidity components of ACM.
- GMP drug product manufacturing is completed, and a potency assay has been developed. Based on the strength of the pharmacology and toxicology data, Rocket anticipates filing an IND in the second quarter of 2023 for a Phase 1 multi-center, dose-escalation study evaluating two doses.

RP-A501 for Danon Disease Moving Toward Global Phase 2 Pivotal Study

- Efficacy results from the Phase 1 study continue to demonstrate durable improvement or stabilization of clinical parameters in the Danon Disease patients treated to date. Notably, these patients' improvements and stabilization of brain natriuretic peptide (BNP) and New York Heart Association (NYHA) class are in stark contrast to BNP increases and NYHA class deterioration observed in a representative sample of pediatric and adolescent natural history patients. These data have been presented recently to the FDA.
- The Phase 2 pivotal trial remains on track for initiation in the second quarter of 2023 based on ongoing and productive FDA interactions.
- Robust Technical Operations capabilities are highlighted by the advancement of in-house AAV cGMP manufacturing at Rocket's state-of-the-art facility in Cranbury, N.J. Completion of two in-house production runs resulted in high-quality drug substance enabling an approximately threefold increase in the number of patients treatable per batch, a significantly improved full versus empty particle ratio, and promising product comparability data generated to date compared to the Phase 1 material manufactured externally.

J.P. Morgan Healthcare Conference Webcast

Gaurav Shah, M.D., Chief Executive Officer, Rocket Pharma, will be presenting at the 41st Annual J.P. Morgan Healthcare Conference today at 2:15 p.m. PT. A live audio webcast of the presentation is available under "Events" in the Investors section of the Company's website at https://ir.rocketpharma.com. The webcast replay will be available on the Rocket website following the conference.

Anticipated 2023 Milestones

Hematology (LV)

RP-L102 for Fanconi Anemia

- Product filing – Q4 2023
- Complementation Groups C & G Investigational New Drug (IND) submission – 2024

RP-L201 for Leukocyte Adhesion Deficiency-I (LAD-I)

- Product filing – Q2 2023
- LAD-I moderate study initiation – Q4 2023

RP-L301 for Pyruvate Kinase Deficiency (PKD)

- Phase 2 pivotal study initiation – Q4 2023

LV Platform Enhancements

- Non-genotoxic conditioning for LV – 2024

Cardiovascular (AAV)

RP-A501 for Danon Disease

- Completed two in-house cGMP batches – Q1 2023
- Planned Phase 2 pivotal study initiation – Q2 2023
- EU Investigational Medicinal Product Dossier (IMPD) filing – Q3 2023

- Danon female study initiation – Q4 2023

RP-A601 for PKP2-ACM

- IND filing – Q2 2023

BAG3-Associated DCM

- IND filing – 2024

Undisclosed Candidates

- Disclosure of additional Wave 2 assets – 2024

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. Rocket also has preclinical AAV-based gene therapy programs in PKP2-arrhythmogenic cardiomyopathy (ACM) and BAG3-associated dilated cardiomyopathy (DCM). 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 anticipated 2023 and 2024 milestones, goals and target, ability to develop its full pipeline of assets with its current cash and cash equivalents, the sufficiency of its cash and cash equivalents to fund operations through 2024, guidance for 2023 and 2024 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), Danon Disease (DD) and other diseases, 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, including its planned pivotal trial, 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20230109005768/en/

Media
Kevin Giordano
kgiordano@rocketpharma.com

Investors
Brooks Rahmer
investors@rocketpharma.com

Source: Rocket Pharmaceuticals, Inc.