Rocket Pharmaceuticals Announces Leadership Expansion to Support Company on Evolution Towards Commercial Stage

April 4, 2023

Jonathan Schwartz, M.D., appointed Chief Gene Therapy Officer to enhance the Company's focus on strategic application of gene therapy technologies to current and future therapeutic areas

Mark White, MB.ChB, named Chief Medical Officer; Dr. White brings proven track record in late-stage development and commercialization with more than 25 years of experience at AstraZeneca

CRANBURY, N.J.--(BUSINESS WIRE)--Apr. 4, 2023-- Rocket Pharmaceuticals, Inc. (NASDAQ: RCKT), a leading late-stage biotechnology company advancing an integrated and sustainable pipeline of genetic therapies for rare disorders with high unmet need, today announced expansion of its leadership team to support its growing and industry-leading pipeline of AAV and LV gene therapy assets and evolution towards commercial stage. Jonathan Schwartz, M.D., Rocket's founding Chief Medical Officer, has been appointed to the newly created position of Chief Gene Therapy Officer to expand the Company's focus on the strategic application of gene therapy technologies to current and future therapeutic areas. In addition, Mark White, MB.ChB, joins Rocket as Chief Medical Officer following more than 25 years at AstraZeneca where he gained deep expertise in late-stage drug development and commercialization across small and large molecules, vaccines and therapeutic areas including rare diseases.

"Today's news represents another exciting and important step forward in our leadership team as we make meaningful strides in our evolution towards commercial stage, adding deep late-stage development expertise and commercial experience to our team along with intensified focus on applying our technologies and successes to date across our pipeline," said Gaurav Shah, M.D, Chief Executive Officer, Rocket Pharma. "Jonathan's exceptional scientific and clinical thought leadership will help unlock the full potential of gene therapy platforms for our pipeline and help advance the industry at large. At the same time, I am excited to welcome Mark as CMO who brings an extensive track record of success and deep insight in leading late-stage drug development and execution; he arrives at the perfect time in our growth. Taken together, with Jonathan, Mark and the rest of our exceptionally experienced and talented leadership team, we are well positioned to actively usher in the next stage of Rocket Pharma."

Dr. Schwartz joined Rocket as Chief Medical Officer in 2016 and has been instrumental in crafting and advancing our six disclosed gene therapy programs with compelling clinical and/or preclinical proof of concept. As Chief Gene Therapy Officer, Dr. Schwartz will oversee research, deepen relationships with external collaborators, and offer a pointed focus on clinical strategy and pipeline expansion.

"With the great strides we have made at Rocket, we are at an opportune time to enhance our focus on gene therapy research and deepen our pipeline even further," said Dr. Schwartz. "As Chief Gene Therapy Officer, I am excited to reinforce Rocket's gene therapy leadership position and look forward to partnering closely with Mark and our extended team to advance current and future programs for the benefit of patients, the healthcare system, and the ability of science to enrich our world."

Dr. White most recently served as Global Franchise Head, Respiratory and Inflammation at AstraZeneca and brings expertise in clinical development, global regulatory submissions, and commercial and business strategy. Among his prior positions, Dr. White was the program lead for multiple innovative medicines leading them through late-stage development, approvals and launches around the globe, including Myalept (metreleptin) at the time of U.S. approval and launch for the ultra-rare condition of generalized lipodystrophy, Saphnelo (anifrolumab) for systemic lupus erythematosus (SLE), and the influenza vaccine portfolio at MedImmune. In prior roles, Dr. White held positions in global marketing, portfolio strategy, clinical development in the design and delivery of clinical pharmacology trials and as a late-stage clinical lead for multiple Phase 3 trials and regulatory submissions.

"Rocket has delivered tangible success, advancing its rich pipeline of hematology and cardiology assets, and I am eager to apply my experience in late-stage drug development to bring the remarkable clinical results to patients in a commercial setting," said Dr. White. "I look forward to joining the team at this exciting time as Rocket makes meaningful strides in realizing gene therapy's potential for rare disease patients around the world."

Dr. White obtained his MB.ChB at the University of Manchester School of Medicine and is a Member of the Royal College of Physicians in the UK, a Fellow of the Royal College of Anaesthetists in the UK and a Member of the Faculty of Pharmaceutical Medicine of the Royal College of Physicians.

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 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 (LV) 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 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 “aim,” “anticipate,” "believe," “can,” “continue,” “design,” “estimate,” "expect," "intend," “may,” "plan," “potential,” "will give," "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, 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, our ability to submit regulatory filings with the U.S. Food and Drug Administration (FDA) and to obtain and maintain FDA or other regulatory authority approval of our product candidates, Rocket’s dependence on third parties for development, manufacture, marketing, sales and distribution of product candidates, the outcome of litigation, our competitors’ activities, including decisions as to the timing of competing product launches, pricing and discounting, our integration of an acquired business, which involves a number of risks, including the possibility that the integration process could result in the loss of key employees, the disruption of our ongoing business, or inconsistencies in standards, controls, procedures, or policies, our ability to successfully develop and commercialize any technology that we may in-license or products we may acquire and any 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, 2022, filed February 28, 2023 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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