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Rocket Pharmaceuticals sponsors clinical trial at UCLA

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Donald B. Kohn, M.D., of UCLA to lead U.S. clinical development efforts for Leukocyte Adhesion Deficiency-I and Infantile Malignant Osteopetrosis programs

Rocket Pharmaceuticals, a leading U.S.-based multi-platform gene therapy company, has announced a research agreement to support the clinical development of Rocket's lentiviral vector (LVV)-based gene therapy programs in Leukocyte Adhesion Deficiency-I (LAD-I) and Infantile Malignant Osteopetrosis (IMO) towards registrational trials. Rocket's LVV-based gene therapy program for LAD-I, RP-L201, is in clinical development with Rocket's European partners at the Centro de Investigaciones Energéticas, Medioambientales y Tecnológicas (CIEMAT). The LVV-based gene therapy for IMO, RP-L401, is in preclinical development in Sweden with European partner Lund University.

UCLA and its Eli and Edythe Broad Center of Regenerative Medicine and Stem Cell Research will serve as the lead U.S. clinical research center for the planned registrational clinical trial for LAD-I and also a lead U.S. clinical site for IMO. Donald B. Kohn, M.D., Professor of Microbiology, Immunology and Molecular Genetics, Pediatrics (Hematology/Oncology), and Molecular and Medical Pharmacology at UCLA, will serve as the principal investigator for the planned trials and will oversee the management of the clinical trial site. Additional terms of the agreement were not disclosed.

"Rocket has put the building blocks in place to execute upon our strategy to transform our pipeline with precision and exigency. Dr. Kohn was chosen to lead the LAD-I and IMO programs as he shared our passion for gene and cellular therapy research for rare diseases," said Gaurav Shah, M.D., Chief Executive Officer and President of Rocket. "We look forward to initiating our first clinical trial for LAD-I in the coming months and advancing our IMO program towards the clinic in 2020."

Rocket's Investigation New Drug (IND) application for RP-L201 was cleared by the U.S. Food and Drug Administration (FDA) in 2018. The Company expects to initiate a Phase 1/2 clinical trial in support of registration in the second quarter of 2019. The Phase 1 portion of the trial will assess the safety and tolerability of RP-L201. The Phase 2 portion of the trial will evaluate overall survival.