Forma Therapeutics Presents Safety, Pharmacokinetic And Pharmacodynamic Data From Phase 1 Clinical Trial For Investigational Agent FT-4202 In Patients With Sickle Cell Disease

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– Findings indicate favorable tolerability profile and favorable pharmacokinetic and pharmacodynamic effects in patients with SCD following a single dose of FT-4202 –

WATERTOWN, Mass. – June 12, 2020 – Forma Therapeutics, Inc. ("Forma"), a clinical-stage biopharmaceutical company focused on rare hematologic diseases and cancers, today announced positive Phase 1 results from the sickle cell disease (SCD) patient arm of an ongoing study of FT-4202, Forma's lead investigational agent currently in clinical development as a potentially disease-modifying treatment for SCD. The data, presented today at the Virtual Edition of the 25th European Hematology Association Annual Congress, demonstrate a favorable tolerability profile and favorable pharmacokinetic/pharmacodynamic (PK/PD) effects of FT-4202 in patients with SCD.

"FT-4202 represents a potential foundational, disease-modifying therapy for people living with sickle cell disease," said Frank Lee, chief executive officer of Forma. "These early data are encouraging and demonstrate multi-modal activity in SCD patients. People living with SCD face exceptional challenges, including an average reduced life expectancy of 25 to 30 years; we hope to change this current reality."

"These data indicate that FT-4202 was well-tolerated and had favorable biologic effects in all patients receiving the investigational agent," said Patrick Kelly, M.D., chief medical officer of Forma. "We believe these initial findings – showing a positive hemoglobin response, increased oxygen affinity and improved membrane deformability of sickle red blood cells – support continued development of FT-4202 in a global Phase 2/3 trial in SCD patients later this year. We also look forward to continuing to report data from our ongoing Phase 1 trial throughout the year."

Forma is currently enrolling patients with SCD in the Phase 1 trial to further evaluate the safety and PK/PD of FT-4202. For more information on eligibility and study locations, please visit clinicaltrials.gov/NCT03815695.

Presentation Overview

Phase 1 Single (SAD) and Multiple Ascending Dose (MAD) Study of the Safety, Pharmacokinetics (PK) and Pharmacodynamics (PD) of FT-4202, a PKR Activator, in Healthy and Sickle Cell Disease Subjects

Poster presentation by Jeremie H. Estepp, M.D., St. Jude Children's Research Hospital

Results announced today are based on patients with SCD (n=7), all of whom had mild vaso-occlusive history, persistent anemia and ongoing hemolysis despite hydroxyurea therapy, randomly assigned to receive a single oral dose of 700 mg of FT-4202 or placebo. The findings indicate that FT-4202:

- Exhibits linear and time-independent PK leading to the multi-modal effects of decreased 2,3-DPG and increased ATP levels, and confirming the PKR enzyme is functional and responsive to activation in sickle RBCs;
- Demonstrates a favorable tolerability profile in SCD patients with only transient grade 1 treatment emergent adverse events;
- Elicits PD responses indicating the favorable biological effects in sickle RBC of improved oxygen affinity, improved deformability and improved membrane function;
- Improves hemoglobin, RBCs and reticulocyte counts which, if modulation is sustained following use of FT-4202, may potentially improve the hemolytic anemia and frequency of vaso-occlusive crises that characterize SCD.

About Sickle Cell Disease

Sickle cell disease (SCD) is one of the most common disorders caused by a single gene mutation. Prevalence of SCD is approximately 100,000 people in the U.S. and approximately 30,000 people in France, Germany, Italy, Spain and the UK. While reporting limitations complicate stating an exact number, the National Institutes of Health reports that prevalence is estimated at over 20 million individuals worldwide. In people living with SCD, red blood cells, or RBCs, spontaneously deform in low oxygen conditions, taking on a sickle-like shape. Sickle cells are stiff and have damaged membranes, causing the RBCs to clump and burst in small blood vessels, resulting in inflammation and vaso-occlusive crises. Repeated deformation also depletes the RBC energy supply, called ATP. One important consequence of this energy depletion is increased levels of a metabolite, 2,3-DPG, that further reduces the RBCs' affinity for oxygen and exacerbates the cycle of repeated deformation and anemia.

About FT-4202

FT-4202 is a novel, oral, once-daily pyruvate kinase-R (PKR) activator designed to be a disease-modifying therapy for the treatment of sickle cell disease (SCD). Early studies and trials have shown that FT-4202 works upstream by employing a multi-modal approach and activating the red blood cells' (RBC) natural PKR activity to decrease 2,3-DPG levels, which we believe leads hemoglobin to hold on to oxygen molecules longer to reduce RBC sickling. FT-4202 has also shown downstream activity by increasing ATP levels, the fuel that provides energy to cells, which we believe may improve RBC health and survival. Together, these effects have the potential to increase hemoglobin levels and decrease painful vaso-occlusive crises. In preclinical safety studies, FT-4202 did not inhibit aromatase activity, important biological processes responsible for sexual development.

About Forma Therapeutics

Forma Therapeutics is a clinical-stage biopharmaceutical company focused on the development and commercialization of novel therapeutics to transform the lives of patients with rare hematologic diseases and cancers. Our R&D engine combines deep biology insight, chemistry expertise and clinical development capabilities to create drug candidates with differentiated mechanisms of action focused on indications with high unmet need. Our work has generated a broad proprietary portfolio of programs with the potential to provide profound patient benefit. For more information, please visit www.FormaTherapeutics.com or follow us on Twitter @FORMAInc and LinkedIn.

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