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FORMA Therapeutics Announces Clinical Data For Sickle Cell Disease Candidate FT-4202, Demonstrating Multi-Modal Activity Of PKR Activation Increases Oxygen Affinity And Decreases Sickle Hemoglobin Polymerization, At 61st Annual ASH Meeting

– Findings Indicate Safety, Tolerability and Proof of Mechanism in Healthy Volunteers, Support the Potential of FT-4202 to Beneficially Impact Hemoglobin Levels and Vaso-occlusive Crises –

WATERTOWN, Mass. – December 9, 2019 – FORMA Therapeutics Inc., a clinical stage biopharmaceutical company focused on rare hematologic diseases and cancers, today announced positive Phase 1 results from the healthy volunteer arm of an ongoing study of FT-4202, a novel selective red blood cell (RBC) pyruvate kinase-R (PKR) activator in development as a potential disease-modifying therapy for sickle cell disease (SCD). The data, presented today at the 2019 American Society of Hematology Annual Meeting, demonstrate the safety, tolerability and proof of mechanism of FT-4202 in healthy subjects, as well as *in vitro* in SCD RBCs.

“The safety and tolerability profile observed in this study is exciting, as are the PK/PD data that confirm FT-4202 activates PKR with a simultaneous effect on hemoglobin oxygen affinity and ATP levels in healthy volunteers,” said Patrick Kelly, M.D., chief medical officer of FORMA Therapeutics. “These data correlate with findings from *in vitro* studies of blood from patients with sickle cell disease, and we have now identified a well-tolerated dose range with PKR activity lasting up to three days following the last dose.”

SCD is a progressive, debilitating genetic disease characterized by the sickling of RBCs. FT-4202 is designed to impact the pathogenesis of SCD with a multimodal approach. First, FT-4202 works upstream by activating the RBCs’ natural PKR activity to decrease 2,3-DPG levels, which lead hemoglobin to hold onto oxygen molecules longer, potentially reducing RBC sickling. Second, the downstream activity of FT-4202 increases ATP levels to potentially improve RBC health and survival. Together, these effects are anticipated to increase hemoglobin levels and decrease the painful vaso-occlusive crises that patients often endure.

“The growing pipeline of new SCD medicines, both recently approved and under investigation, is very encouraging for these patients, however there is still an unmet need to address the complexity of this disease,” said Frank Lee, chief executive officer of FORMA Therapeutics. “We are encouraged by the potential ability of FT-4202 to address multiple biological events in the cascade that ultimately leads to painful vaso-occlusive crises and organ damage.”

FORMA is evaluating FT-4202 in an ongoing Phase 1 study to characterize the safety, tolerability and the pharmacokinetics/pharmacodynamics (PK/PD) of a single ascending dose and multiple ascending doses of FT-4202, first in the recently completed arm in healthy volunteers, and now in patients with sickle cell disease. For more information and a list of investigative sites currently recruiting patients for this study, please visit clinicaltrials.gov, identifier number NCT03815695.

Presentation Overview

Phase 1 Single (SAD) and Multiple Ascending Dose (MAD) Studies of the Safety, Tolerability, Pharmacokinetics (PK) and Pharmacodynamics (PD) of FT-4202, an Allosteric Activator of Pyruvate Kinase-R, in Healthy and Sickle Cell Disease Subjects

Oral presentation by Theodosia A. Kalfa, M.D., Ph.D., Associate Professor, University of Cincinnati Department of Pediatrics, Cincinnati Children's Hospital Medical Center

Results announced today are based on healthy subjects (SAD n=32; MAD n=48) randomly assigned to receive a single oral dose up to 1000 mg of FT-4202 or placebo, or multiple oral doses up to 600 mg/day of FT-4202 or placebo for 14 days. The findings:

- Exhibit linear and time-independent pharmacokinetics demonstrating proof of mechanism based on the mean maximum percent of change in key pharmacodynamic measures from baseline, achieved within 24 hours of a single dose of FT-4202 and sustained for the MAD 14-day dose period;
- Demonstrate a favorable safety profile with predominantly Grade 1 treatment emergent adverse events and no QTc interval prolongation events observed;
- Demonstrate maximal ATP response at doses ≥ 50 mg twice daily or 150 mg once daily in healthy RBC; and
- Demonstrate maximal 2,3-DPG response at doses ≥ 150 mg twice daily or 400 mg once daily in HV RBCs.

About Sickle Cell Disease

In patients with sickle cell disease (SCD), red blood cells, or RBCs, spontaneously deform in low oxygen conditions. The deformed cells take on a sickle-like shape. RBC sickling stimulates production of 2,3-DPG, exacerbating the condition by further reducing the RBCs' affinity for oxygen. Because sickled RBCs have lower levels of ATP, the fuel essential for cell function and health, the sickled cells are stiff with damaged membranes, causing the RBCs to clump and hemolyze in small blood vessels, thereby resulting in inflammation and vaso-occlusive crises. SCD is the most common disorder caused by a single gene mutation; the disease affects approximately 100,000 people in the U.S. and millions globally.

About FT-4202

FT-4202 is a novel selective red blood cell (RBC) pyruvate kinase-R (PKR) activator designed to be a disease-modifying therapy for the treatment of sickle cell disease (SCD). Employing a multimodal approach, FT-4202 works upstream by activating the RBCs' natural PKR activity to decrease 2,3-DPG levels, which leads hemoglobin to hold on to oxygen molecules longer to reduce RBC sickling. The downstream activity of FT-4202 increases ATP levels, the fuel that provides energy to cells, to improve RBC health and survival. Together, these effects are anticipated to increase hemoglobin levels and decrease painful vaso-occlusive crises.

About FORMA Therapeutics

FORMA Therapeutics is focused on the discovery, development and commercialization of transformative medicines for patients with rare hematologic diseases and cancers. A fully-integrated

biopharmaceutical company, FORMA's validated, proprietary R&D engine combines deep biology insight, chemistry expertise and clinical development capabilities to create differentiated drug candidates with best-in-class or first-in-class potential. FORMA has delivered high-value clinical candidates to its partners and generated a broad proprietary portfolio of programs, ranging from preclinical to pivotal-stage, with the potential to provide profound patient benefit. For more information, please visit the company website at www.formatherapeutics.com or follow us on Twitter @FORMAInc and LinkedIn.

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