

PRESS RELEASES

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FORMA Therapeutics Announces FT-4202 Granted Fast Track Designation And Rare Pediatric Disease Designation For Treatment Of Sickle Cell Disease

WATERTOWN, Mass. – February 20, 2020 – FORMA Therapeutics, Inc., a clinical-stage biopharmaceutical company focused on rare hematologic diseases and cancers, today announced the U.S. Food and Drug Administration (FDA) has granted Fast Track designation and Rare Pediatric Disease designation for its lead clinical asset, FT-4202, in development as a disease-modifying treatment for sickle cell disease (SCD). FT-4202 is a selective pyruvate kinase-R (PKR) activator that exhibits the potential to beneficially impact both anemia and vaso-occlusive crises for people living with SCD.

“These designations for FORMA’s lead clinical asset underscore the FDA’s continued recognition of the needs of patients with SCD,” said Frank Lee, chief executive officer of FORMA Therapeutics. “With more than 100,000 individuals living with SCD in the U.S., and with relatively few treatment options, we believe significant unmet medical needs persist. These FDA designations give FORMA the opportunity to accelerate the development of a new treatment for people impacted by SCD.”

FORMA is currently enrolling patients with SCD in a Phase 1 study to evaluate the safety and pharmacokinetics/pharmacodynamics (PK/PD) of FT-4202. FORMA plans to initiate a registrational trial within the next year. For more information on eligibility and study sites for the open Phase 1 study, please visit clinicaltrials.gov/NCT03815695.

About Fast Track Designation

The FDA’s Fast Track designation is a process designed to facilitate the development and expedite the review of drugs to treat serious conditions and fill an unmet medical need. Fast Track designation allows for early and frequent communication with the FDA throughout the entire drug development and review process. It also enables eligibility for Accelerated Approval and Priority Review, as well as a rolling review of a company’s New Drug Application, if relevant criteria are met.¹

About Rare Pediatric Disease Designation

Under the FDA’s Rare Pediatric Disease (RPD) program, a sponsor who receives marketing approval for a product with an RPD designation may be eligible for a voucher that can be redeemed to obtain priority review for any subsequent marketing application. The FDA defines a “rare pediatric disease” as a rare disease in which the serious or life-threatening manifestations primarily affect individuals from age zero to 18.²

About Sickle Cell Disease

Sickle cell disease (SCD) is the most common disorder caused by a single gene mutation. Worldwide, an estimated 300,000 children are born with SCD each year. In the U.S., prevalence of SCD is approximately 100,000 people. In people living with SCD, red blood cells, or RBCs, spontaneously deform in low oxygen conditions, taking on a sickle-like shape. Sickled 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 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. In preclinical safety studies, FT-4202 did not inhibit aromatase activity or affect steroidogenesis, important biological processes responsible for sexual development.

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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