Forma Therapeutics Announces FT-4202 Receives Orphan Drug Designation in Europe for Treatment of Sickle Cell Disease

November 10, 2020

WATERTOWN, Mass.--(BUSINESS WIRE)--Nov. 10, 2020-- Forma Therapeutics Holdings, Inc. (Nasdaq: FMTX), a clinical-stage biopharmaceutical company focused on rare hematologic diseases and cancers, today announced the European Commission granted Orphan Drug designation to Forma's FT-4202 for the treatment of sickle cell disease (SCD), based on a positive opinion from the Committee for Orphan Medicinal Products of the European Medicines Agency (EMA). FT-4202 was previously granted Fast Track, Rare Pediatric Disease and Orphan Drug designations from the U.S. Food and Drug Administration (FDA) for the treatment of patients with SCD.

"I am pleased to see the regulatory community recognize the urgent need to bring therapies to people living with sickle cell disease," said Frank Lee, president and chief executive officer of Forma. "Without effective treatment, sickle cell can affect all organs over time and lead to substantial suffering for those born with this genetic disease. We embrace the potential this designation may provide as we prepare to initiate a global registrational Phase 2/3 trial with FT-4202 in the first quarter of 2021."

Forma is currently enrolling patients with SCD in a randomized, placebo-controlled, multi-center Phase 1 study to evaluate the safety and pharmacokinetics/pharmacodynamics (PK/PD) of FT-4202. For more information on eligibility and study sites for the open Phase 1 study, please visit clinicaltrials.gov/NCT03815695.

About Orphan Drug Designation in the European Union (EU)

The European Commission grants Orphan Drug designation (ODD) to investigational drugs intended to diagnose, prevent or treat a rare disease. To qualify for ODD, the potential therapeutic must target a life-threatening or chronically debilitating disease that affects fewer than five (5) in 10,000 persons in the EU. In addition, the investigational drug must either provide a significant benefit over existing therapies or provide a treatment for patients for whom existing therapies do not work or exist. The designation provides financial and regulatory incentives to the sponsor company such as reduced fees, tax waivers, dedicated funds to reimbursement and 10 years of market exclusivity.

About Sickle Cell Disease

Sickle cell disease (SCD) is one of the most common single-gene disorders and is estimated to affect more than 70,000 in the EU-27, as well as approximately 100,000 people in the United States. The National Institutes of Health (NIH) reports that prevalence is estimated at more than 20 million individuals globally. From 2010 to 2050, the annual number of newborns with SCD is expected to rise globally by approximately one-third.[1] Despite recent advances in treatment, most patients with SCD still suffer from pain crises, lifelong disability, significant morbidity and reduced quality of life.

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 a clinical-stage biopharmaceutical company focused on the research, 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.

Forward-looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, including, without limitation, express or implied statements regarding the potential regulatory, exclusivity and marketing advantages of Orphan Drug designation by the EU Commission and previously granted Fast Track, Rare Pediatric Disease and Orphan Drug designations from the U.S. Food and Drug Administration (FDA) of FT-4202 for the treatment of patients with SCD,the advancement of our sickle cell disease program, our expectations of the therapeutic benefits related thereto, the timing and success of ongoing clinical trials, and our growth as a company and the anticipated contribution of our executives and employees to our operations and progress. The words "may," "will," "could," "would," "should," "expect," "plan," "anticipate," "intend," "believe," "estimate," "predict," "project," "potential," "continue," "target" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

Any forward-looking statements in this press release are based on management's current expectations and beliefs and are subject to a number of risks, uncertainties and important factors that may cause actual events or results to differ materially from those expressed or implied by any forward-looking statements contained in this press release, including, without limitation, those risks and uncertainties related to the advancement of our clinical

programs and other risks identified in our SEC filings, including those risks discussed under the heading “Risk Factors” in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2020, as well as other risks detailed in our subsequent filings with the SEC. We caution you not to place undue reliance on any forward-looking statements, which speak only as of the date they are made. We disclaim any obligation to publicly update or revise any such statements to reflect any change in expectations or in events, conditions or circumstances on which any such statements may be based, or that may affect the likelihood that actual results will differ from those set forth in the forward-looking statements. Any forward-looking statements contained in this press release represent our views only as of the date hereof and should not be relied upon as representing its views as of any subsequent date. We explicitly disclaim any obligation to update any forward-looking statements.

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[1]Piel, F. B., Hay, S. I., Gupta, S., Weatherall, D. J., & Williams, T. N. (2013). Global burden of sickle cell anaemia in children under five, 2010-2015: Modelling based on demographics, excess mortality, and interventions. PLOS Medicine, 10(7). Retrieved from link.

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Source: Forma Therapeutics Holdings, Inc.