Disc Medicine Expands Hematology Pipeline with Worldwide Licensing Agreement for Bitopertin, a First-in-Class Modulator of Heme Synthesis

May 27, 2021

Clinical-Stage Program is Positioned to Enter Phase 2 Study in Erythropoietic Protoporphyria (EPP)

CAMBRIDGE, Mass. (May 27, 2021) – Disc Medicine, Inc. announced today that it has entered into an exclusive worldwide licensing agreement with F. Hoffmann-La Roche Ltd for the development and commercialization of bitopertin, an orally administered GlyT1 inhibitor with demonstrated effects on the heme biosynthesis pathway. Disc Medicine intends to develop bitopertin as a treatment for hematologic diseases, initially for erythropoietic porphyrias (EP), a family of rare, debilitating and potentially life-threatening disorders caused by dysregulated heme biosynthesis in developing red blood cells.

“This collaboration is a major milestone in establishing Disc Medicine as a leader in hematology and enables us to target heme synthesis, a fundamental biological pathway of red blood cells that is highly complementary to our existing programs in iron homeostasis,” said John Quisel, J.D., Ph.D., President and Chief Executive Officer of Disc Medicine. “Importantly, the well-established clinical safety profile of bitopertin will allow us to move rapidly into patient studies of several serious, hematologic diseases.”

Under the terms of the agreement, Disc Medicine will obtain exclusive, global rights to and be responsible for all development, manufacturing, and commercialization of bitopertin and related back up compounds. Roche will receive an upfront payment from Disc Medicine and potential development, regulatory, and commercial milestones totaling in excess of $200 million. Roche is also eligible to receive tiered royalties based on net revenues, at a rate ranging from the high single digits to high teens, and a proportion of proceeds from future transactions related to bitopertin. Disc will engage with regulatory authorities to initiate patient studies in 2022.

About Erythropoietic Porphyrias

Erythropoietic porphyrias (EPs) are a family of ultra-rare, debilitating and potentially life-threatening diseases caused by mutations that affect the heme synthesis pathway. This results in the toxic accumulation of porphyrins that are activated when patients are exposed to sunlight and cause oxidative damage to surrounding tissues. EPs are characterized by severe cutaneous sensitivity that manifests as attacks of intense, burning pain, associated with blistering, edema and disfigurement that often persist. Patients also develop gastrointestinal complications from accumulation of porphyrins, including gallstones and liver failure in 5-20% of patients. There is no approved disease-modifying therapy and the only cure is hematopoietic stem cell transplant. The current approach to patient care involves taking extreme measures to avoid daylight, such as restricting outdoor activities to nighttime or using protective clothing and opaque shields, and management of pain. The first signs typically manifest in early childhood and are lifelong, having a major impact on the well-being, psychosocial development and daily lives of patients and their caregivers. Erythropoietic porphyrias comprise three subtypes: Erythropoietic protoporphyria (EPP), X-linked protoporphyria (XLPP), and Congenital Erythropoietic Porphyria (CEP).

About Bitopertin

Bitopertin is a clinical-stage, orally administered small molecule inhibitor of glycine transporter 1 (GlyT1). Glycine is an essential precursor for heme biosynthesis and GlyT1 is required to maintain adequate levels of intracellular glycine in developing erythrocytes. As a modulator of heme synthesis, bitopertin has the potential to provide benefit for a range of disorders caused by imbalances in the production of heme and its pathway intermediates. Bitopertin has been evaluated in over 4,000 healthy volunteers and patients in over 30 clinical trials across multiple indications, including several Phase 2 and 3 trials in psychiatric disorders and in a rare blood cell disorder and has a well-defined safety profile. Although CNS efficacy was not established in these studies, bitopertin demonstrated marked effects on heme synthesis. Moreover, in preclinical studies conducted by Disc Medicine, inhibition of GlyT1 by bitopertin was shown to decrease levels of the metabolites that are the underlying cause of EP. Bitopertin is an experimental agent and is not approved for use as a therapy in any jurisdiction worldwide.

###

About Disc Medicine

Disc Medicine is a biopharmaceutical company dedicated to transforming the lives of patients with hematologic disorders. We are building a unique portfolio of innovative, first-in-class therapeutic candidates that affect fundamental pathways of red blood cell biology. We are committed to developing treatments that empower and bring hope to the many patients who suffer from hematologic diseases. For more information, please visit www.discmedicine.com.

Media Contact

Sarah Ellinwood, Ph.D.

Verge Scientific Communications
sellinwood@vergescientific.com