HemoShear Therapeutics Receives FDA Clearance of IND for Phase 2 Study of its Investigational Drug HST5040 for the Treatment of Methylmalonic Acidemia and Propionic Acidemia

Charlottesville, Va., June 24, 2020 – HemoShear Therapeutics, a clinical stage company developing treatments for rare metabolic disorders, has received clearance from the U.S. Food and Drug Administration (FDA) for its Investigational New Drug (IND) application to conduct a phase 2 clinical trial of HST5040, an oral small molecule drug for the treatment of patients with methylmalonic acidemia (MMA) and propionic acidemia (PA). MMA and PA are rare genetic disorders caused by the deficiency of certain enzymes required to metabolize amino acids. The diseases result in the rapid buildup of life-threatening metabolites that can lead to severe organ damage, seizures, developmental deficits, and premature death.

"There are currently no targeted pharmacologic treatments for MMA or PA that can improve quality of life or extend lifespan, and there is a lot of enthusiasm in the community for the potential of a daily oral treatment approach," said Marshall Summar, MD, Division Chief, Genetics and Metabolism and Director of the Rare Disease Institute at Children's National. "This is a very exciting time for patients with MMA or PA and their families."

HemoShear's phase 2 clinical study, HERO (HElp Reduce Organic Acids), is designed to enroll at least 12 patients with MMA and PA. The study, which will start with patients age 12 and over and then expand to age 2 and older, will be conducted at select leading children's hospitals in the United States. The HERO Study will include three stages: open-label dose escalation; followed by a randomized, double-blind, placebo-controlled crossover; and then an open-label long-term extension.

"The FDA clearance to move HST5040 forward into a Phase 2 clinical trial is an important milestone in developing treatments for these devastating diseases as well as evidence of the potential of our innovative human disease modeling platform," said Jim Powers, Chairman and CEO of HemoShear. "I am very proud of the accomplishments of our team. We will be working closely with the MMA and PA communities to enroll patients into our clinical trial of this novel and convenient oral therapy."

About HST5040

HST5040 is an investigational oral small molecule therapy developed by HemoShear to correct metabolic abnormalities associated with MMA and PA. Because HST5040 is a small molecule, it has the ability to distribute to multiple affected tissues and thus has the potential to be active throughout the body, including the brain, heart and muscles. HST5040 is designed for convenient daily administration at home as a liquid formulation taken either orally or through a gastric feeding tube.

About HemoShear Therapeutics

HemoShear Therapeutics is a privately held clinical stage company developing treatments for rare metabolic disorders with significant unmet patient need. HemoShear's drug discovery platform, *REVEAL-Tx™*, enables the Company's scientists to create best-in-class, biologically relevant human disease models to uncover the underlying mechanisms of disease, translate those discoveries into drug targets, and select candidates that may treat patients successfully. In addition to the Company's proprietary rare disease programs, HemoShear has exclusive partnerships to identify novel therapeutic approaches in nonalcoholic steatohepatitis (NASH) with Takeda and in gout with Horizon Therapeutics
. For more information visit www.HemoShear.com.