HemoShear Therapeutics Announces First Two Patients Dosed in Phase 2 Study of Oral Small Molecule HST5040 for Methylmalonic Acidemia (MMA) and Propionic Acidemia (PA)

Charlottesville, Va., July 22, 2021 – HemoShear Therapeutics, Inc., a clinical stage company developing treatments for rare metabolic disorders, today announced that the first two patients have been dosed in the HERO (HElp Reduce Organic Acids) Phase 2 clinical trial of HST5040, an oral small molecule drug being investigated for the treatment of patients with methylmalonic acidemia (MMA) and propionic acidemia (PA). This trial seeks to advance the first oral therapy designed to directly address underlying biochemical causes of these rare and life-threatening diseases.

HST5040 is being developed by HemoShear to lower toxic metabolites that build up in MMA and PA as a result of genetic deficiencies in key enzymes required to break down certain amino acids from protein. These toxins can result in severe organ damage, developmental deficits, and premature death. Despite the current standard of care involving dietary control, carnitine supplementation and in more severe cases, organ transplantation, there remains a high unmet medical need in MMA and PA. As a small molecule, HST5040 has the ability to distribute to all affected tissues and thus has the potential to be active throughout the body, including the liver, kidneys, 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. The FDA has granted HemoShear's HST5040 Orphan Drug, Fast Track and Rare Pediatric Disease designations to treat MMA and PA.

"There are currently no targeted pharmacologic treatments for MMA or PA that can improve quality of life or extend lifespan for these devastating diseases," said Kimberly Chapman, MD, PhD, genetic metabolist at Children's National Hospital in Washington, DC. "The patient community is excited for the possibility of a convenient, daily oral treatment approach."

The HERO study is enrolling at least 12 patients with MMA or PA aged 2 and older. The study will include three sequential treatment periods: an open-label, within-patient, dose escalation period; followed by a randomized, double-blind, placebo-controlled crossover period; and then an open-label, long-term extension period. If the data are encouraging, enrollment in the long-term extension period of the HERO study may be expanded to include patients who have had liver or kidney transplants and those with additional types of MMA.

The first study participants to receive HST5040 are pediatric PA patients under the care of Jennifer Gannon, MD, the principal investigator at Children's Mercy Kansas City. Additional study sites include Boston Children's Hospital, UMPC Children's Hospital of Pittsburgh, Children's National Hospital, Rady Children's Hospital of San Diego, University of Florida, University of Minnesota, University of Utah, Vanderbilt University Medical Center and Yale Center for Clinical Investigation. More information can be found at www.MMA-PAHero.com or ClinicalTrials.gov (NCT04732429).

"This is a tremendous milestone for patients and families who desperately need a treatment that can potentially target all organs affected by these diseases," said Jim Powers, Chairman and CEO of HemoShear. "We named this the HERO study because of the challenges brave families face every day living with MMA and PA. We look forward to monitoring the impact of HST5040 on participants in the study."

About MMA and PA

Methylmalonic acidemia (MMA) and propionic acidemia (PA) are rare genetic disorders caused by the deficiency of key enzymes required to metabolize certain amino acids from protein. MMA and PA are diagnosed through newborn screening in the United States and in select countries in Europe, the Middle East and the rest of the world. The diseases result in the buildup of toxic metabolites that can lead to frequent acute metabolic decompensations, severe organ damage, seizures, developmental deficits, and premature death. In the United States, about 1 in 70,000 newborns is diagnosed with MMA, and about 1 in 240,000 is diagnosed with PA. Both diseases are more common in the Middle East and North Africa. There are an estimated 4,000 MMA and PA patients in the US and Europe combined.

About HemoShear Therapeutics

HemoShear Therapeutics, Inc. 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) and an undisclosed rare liver disease with Takeda, and in gout with Horizon Therapeutics. For more information visit www.HemoShear.com.

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