HemoShear Therapeutics Receives FDA Fast Track and Rare Pediatric Disease Designations for HST5040 to Treat Methylmalonic Acidemia and Propionic Acidemia

~Designations Highlight the Urgent Need for Treatments for These Rare Diseases~

Charlottesville, Va., July 28, 2020 – HemoShear Therapeutics, a clinical stage company developing treatments for rare metabolic disorders, has received Fast Track and Rare Pediatric Disease designations from the U.S. Food and Drug Administration (FDA) for HST5040, a once-daily oral small molecule drug being developed to treat methylmalonic acidemia (MMA) and propionic acidemia (PA).

In June, HemoShear received clearance from the FDA for its Investigational New Drug application to conduct a phase 2 clinical study of HST5040 in patients with MMA and 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. HemoShear's phase 2 clinical study, HERO (HElp Reduce Organic Acids), is designed to enroll at least 12 patients with MMA and PA at select children's hospitals in the United States.

"It is very encouraging that the FDA has recognized the urgent need for developing therapies like HST5040 to improve the lives of patients with MMA and PA," said Nicola Longo, M.D., Ph.D., Chief of the Division of Medical Genetics at the University of Utah and Professor of Pediatrics at Primary Children's Medical Center. "Clinicians on the front lines of treating these devastating diseases have very limited options to help their patients."

Fast Track designation is intended to accelerate the development and review of therapies to treat serious conditions with unmet medical need. This award will enable HemoShear to have more frequent interactions with the FDA, as well as eligibility for accelerated approval, priority review, and rolling submission of a new drug application. Rare Pediatric Disease designation is awarded by the FDA for therapies being developed to treat serious or life-threatening diseases that primarily affect children ages 18 years or younger and fewer than 200,000 people in the U.S. Through this award, HemoShear may receive a voucher upon commercial approval of HST5040 that provides for priority review of a subsequent new drug application.

"Knowing that there are no effective treatments currently available for patients, we are working hard to initiate our HERO clinical trial," said Jim Powers, Chairman and CEO of HemoShear. "We will be working closely with the MMA and PA communities to enroll patients into our study of this novel and convenient oral therapy."

About HST5040

HST5040 is an investigational oral small molecule therapy developed by HemoShear to address 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.