



HemoShear Therapeutics' HST5040 Receives U.S. Orphan Drug Designations for Treatment of Methylmalonic Acidemia and Propionic Acidemia

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

Charlottesville, Va., December 16, 2020 – HemoShear Therapeutics, a clinical stage company developing treatments for rare metabolic disorders, is pleased to announce that the U.S. Food and Drug Administration (FDA) has granted Orphan Drug Designations to HemoShear's HST5040 oral small molecule for treatment of methylmalonic acidemia (MMA) and propionic acidemia (PA). HST5040 was granted Fast Track and Rare Pediatric Disease designations to treat MMA and PA earlier in 2020.

This past 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 buildup of toxic metabolites that can lead to frequent metabolic decompensations, 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.

"We are pleased that the FDA has recognized the need for developing therapies like HST5040 to improve the lives of patients with MMA and PA," said Jim Powers, Chairman and CEO of HemoShear. "With no effective treatments currently available to treat these rare and devastating diseases, we are moving rapidly to initiate our HERO clinical trial of this convenient oral therapy."

Orphan Drug Designation is awarded by the FDA for therapies to treat diseases that affect fewer than 200,000 people in the U.S. This award will enable HemoShear to receive incentives, such as tax credits towards the cost of clinical trials and prescription drug user fee waivers, as well as the potential for seven years of market exclusivity in the U.S. following approval.

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, liver, kidneys, 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) and an undisclosed rare liver disease with [Takeda](#), and in gout with [Horizon Therapeutics](#). For more information visit www.HemoShear.com.