SHEAR INSIGHTS

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Peer-Reviewed Journal Publishes the Discovery of HemoShear's HST5040 Small Molecule in Clinical Development for Methylmalonic Acidemia (MMA) and Propionic Acidemia (PA)

The *Journal of Medicinal Chemistry* has published a paper by HemoShear scientists describing the discovery and characterization of HST5040 as a promising investigational agent in clinical development for Methylmalonic Acidemia (MMA) and Propionic Acidemia (PA). MMA and PA are rare genetic diseases caused by deficiencies of certain metabolic enzymes that result in impaired energy production and the buildup of toxins that can cause severe organ damage, developmental deficits, and premature death. The publication elaborates on how HemoShear's *REVEAL-Tx*™ platform enabled the identification of a lead series of small molecules that were further optimized for potency and other important drug properties resulting in the discovery of the clinical candidate HST5040.

"We created disease models for MMA and PA from patients' liver cells using our *REVEAL-Tx*™ platform and were able to screen for compounds that reduced key disease biomarkers," said Brian Johns, Chief Scientific Officer of HemoShear. "We selected HST5040 for its activity in reducing our target biomarkers while also demonstrating excellent drug-like properties during our preclinical evaluations. We were encouraged when we determined that the compound was studied in clinical trials previously for unrelated diseases and had demonstrated a favorable safety profile at doses that we believe may be therapeutic for MMA and PA."

Laboratory and animal studies were also conducted to determine how the body processes HST5040, including the absorption, metabolism, distribution and excretion of the compound. "It was important not only for us to select a candidate, but also to understand what tissues the molecule reaches and how it is metabolized in the body," said Gerry Cox, MD, PhD, Acting Chief Medical Officer at HemoShear. "Clinicians want to know the pharmacological properties of the drug and the organs that may be impacted. We were pleased to see that the drug reached critical organs affected by the disease, such as the kidney, liver, heart, muscles and brain."

Clinical Trial with HST5040

The FDA has granted HemoShear's HST5040 Orphan Drug, Fast Track and Rare Pediatric Disease designations for the treatment of MMA and PA. HemoShear's HERO phase 2 clinical study of HST5040 is enrolling at least 12 patients aged 2 and older with MMA or PA at select children's hospitals in the United States.

HELP REDUCE ORGANIC ACIDS
HERO
STUDY

HEMOSHEAR THERAPEUTICS

HemoShear Therapeutics discovers novel biological targets and advances drug programs to treat metabolic disorders with significant unmet patient need.