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Charlottesville, Virginia

Journal Publishes HemoShear Models for Rare Metabolic Diseases

The peer-reviewed journal *Molecular Genetics and Metabolism* has published HemoShear's data on models of Methylmalonic Acidemia (MMA) and Propionic Acidemia (PA). These rare genetic diseases are caused by deficiencies of certain metabolic enzymes that result in the buildup of toxins that can cause severe organ damage, developmental deficits and premature death. The publication demonstrates that HemoShear's REVEAL-Tx™ platform can successfully recreate complex biochemistry in relevant models of human disease.

"HemoShear's work has led to a greater understanding of the physiology of these diseases and has accelerated the important process of discovering potential treatments that we can study in patients," said Kimberly Chapman, MD, PhD, medical geneticist at Children's National and a co-author of the study.

REVEAL-Tx applies principles of physiological blood flow to human tissue to restore the in vivo disease state. HemoShear researchers created models of MMA and PA by using cells from the livers of patients with these diseases who underwent transplant. The company's models, which more accurately reflect the human disease state than traditional MMA and PA animal models, enable the HemoShear team to understand the complex disease process, identify and validate promising treatment approaches, and study the effects of drug candidates.

HemoShear scientist works with REVEAL-Tx™ platform device that restores blood flow to tissue

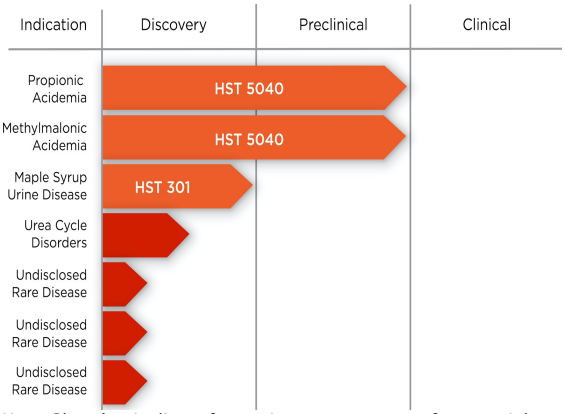
In MMA and PA, certain amino acids are not completely metabolized, leading to buildup of life-threatening toxins. HemoShear's research team was able to characterize how these amino acids are metabolized in MMA and PA patients and assess how different diets can affect patient outcomes. They also investigated a dietary supplement used for improving energy in patients and saw similar supporting results in REVEAL-Tx.

"REVEAL-Tx allows us to preserve the biochemical defects of these diseases. Because the models are derived from the patients and have their same genetic defects, we can use them to better understand the disease pathophysiology and the biochemical basis behind clinical manifestations," said Sol Collado, PhD, lead author of the paper and a rare disease program leader at HemoShear. "Our testing of key biomarkers aligns with what is seen in real patients and has been important in selecting an optimal drug candidate for clinical development."

New Drug Candidate Discovered

HemoShear has discovered a new drug candidate for MMA and PA using its validated disease models. REVEAL-Tx enabled the company to assess the efficacy and safety of different treatment approaches and led to the discovery of a new oral drug, called HST5040, that may reduce harmful toxins in patients.

"We are pleased that REVEAL-Tx has provided unprecedented insights into these complex disease pathways," said Brian Wamhoff, COO, Head of Innovation. "What is most exciting is that our research has led us to discover a potential new treatment for MMA and PA and we are making good progress advancing that candidate towards the clinic."



HemoShear's pipeline of proprietary treatments for rare inborn errors of metabolism