

HemoShear Therapeutics Launches Pilot Study to Evaluate Neuropsychological Assessments for MMA and PA

MMA & PA INSIGHT STUDY

HemoShear Therapeutics has begun a pilot study to evaluate how different neurocognitive assessments can be used for methylmalonic acidemia (MMA) and propionic acidemia (PA) patients.

Many children and adults with MMA and PA have difficulties learning, communicating, relating to others and participating in activities of daily living. As HemoShear's HST5040 enters into clinical studies, we will need effective ways to measure improvements in processing information, communicating, life skills and functioning in general.



[The MMA and PA Insight Study](#) is designed to identify which assessments might show changes in thinking or behavior when a potential new treatment, such as HST5040, is studied in clinical trials.

"Sometimes measuring an improvement can be hard in a clinical trial. I think that this study will help us make the right choices about which tests to use so that future therapeutic studies can demonstrate improvements when benefit has occurred," said Kimberly Chapman, MD, PhD, an expert in the diagnosis and management of MMA and PA at Children's National Hospital in Washington, DC.

The Insight Study will be conducted completely online and does not involve any visits to a clinic. Assessments will be done by Susan E. Waisbren, PhD, a leader in conducting neuropsychological evaluations in children with genetic disorders at Harvard Medical School and Boston Children's Hospital. She will conduct video calls with at least 25 English-speaking participants from around the United States and other countries.

"We are hopeful that HST5040 will improve the quality of life for patients and families impacted by MMA and PA," said Gerry Cox, MD, PhD, Chief Medical Officer at HemoShear. "The insights that we gain from this research will help us to better design clinical trials that can measure meaningful changes in abilities that may ultimately have a major impact on the day-to-day functioning of these vulnerable patients."