Dear MMA and PA Community,

We are excited to inform you that the US Food and Drug Administration has cleared HemoShear to proceed with a clinical trial of our investigational drug HST5040 for the treatment of MMA and PA.

HST5040 is an oral therapy developed by HemoShear that will be tested to correct metabolic abnormalities associated with MMA and PA. HST5040 has the potential to be active throughout the body, including the brain, heart and muscles. The drug can be taken conveniently at home as a daily liquid formulation by mouth or through a gastric feeding tube.

Our clinical study, HERO (Help Reduce Organic Acids), is designed to enroll at least 12 patients with MMA and PA. The study, which will start with patients age 12 and over and then expand to age 2 and older, will be conducted at select leading children’s hospitals in the United States.

We are in the process of contracting with study sites and plan to be ready to begin later this year. Our first priority is ensuring that all families are safe, so the timing of the first patient enrolled may be impacted by the COVID-19 pandemic.

You are a Hero!

Making medical progress to improve the quality of life for MMA and PA patients is going to be a collaborative process between industry, clinical researchers and families. We will be in touch with the community when the study is ready to begin enrolling participants. In the meantime, we invite you to visit our website and sign up if you want to receive updates from us.

We are humbled and excited to see if our drug can make a positive difference in patients’ lives.

On behalf of the entire HemoShear team, thank you for your interest and support.

Sincerely,

Brian Wamhoff, PhD
Co-Founder and Head of Innovation