SHEAR INSIGHTS

July 19, 2018

HemoShear Therapeutics Continues to Engage with Rare Disease Groups

HemoShear Gains Valuable Insight from Patients at Family Metabolic Disease Conferences

Representatives from HemoShear attended two recent metabolic rare disease family meetings to share the latest developments from the company and gain valuable insights into these diseases. The information gleaned from patients and caregivers will help the company to better understand the patient's experience and design future clinical trials to measure meaningful impacts on quality of life.

The company participated for the second time in the Organic Acidemia Association (OAA) International Metabolic Conference and for the first time in the Maple Syrup Urine Disease (MSUD) Family Support Group Symposium.

Understanding the Disease Experience from OAA Families

During the OAA meeting, HemoShear sponsored a gathering of 10 parents of children and young adults with propionic acidemia (PA) and methylmalonic acidemia (MMA) for a day-long session to share their experience with the diseases. The session was moderated by a skilled facilitator and included the following topics: Personal Experience, Living with MMA and PA, Caring for MMA and PA, Family Perceptions and Communication. The often-emotionally charged discussions included how their child was diagnosed, their treatment regimens, triggers that lead to metabolic crises, the impact on their child's development and growth and managing family dynamics. Below are a few quotes from the discussions:

- "I don't think I grasped the severity of the diagnosis. I heard diet, the protein thing. But then I Googled it. It's horrible."
- "I try to get to the hospital early when I see signs. You don't know. You could go home in an hour after getting [supplements], or you could be there weeks. But at least your child is not going to stay home and die."
- "Sometimes my daughter gets kind of sad, and sometimes kind of overjoyed. I think her moods are side effects. She generally has a good disposition. She's loving and happy and she's still alive."
- "My son got treated so differently at school. He didn't want to get singled out. I ended up home schooling."
- "Our older daughter gets stressed out with her sister. She protects her, trying not to get in the way when her sister's sick. She's constantly worried."

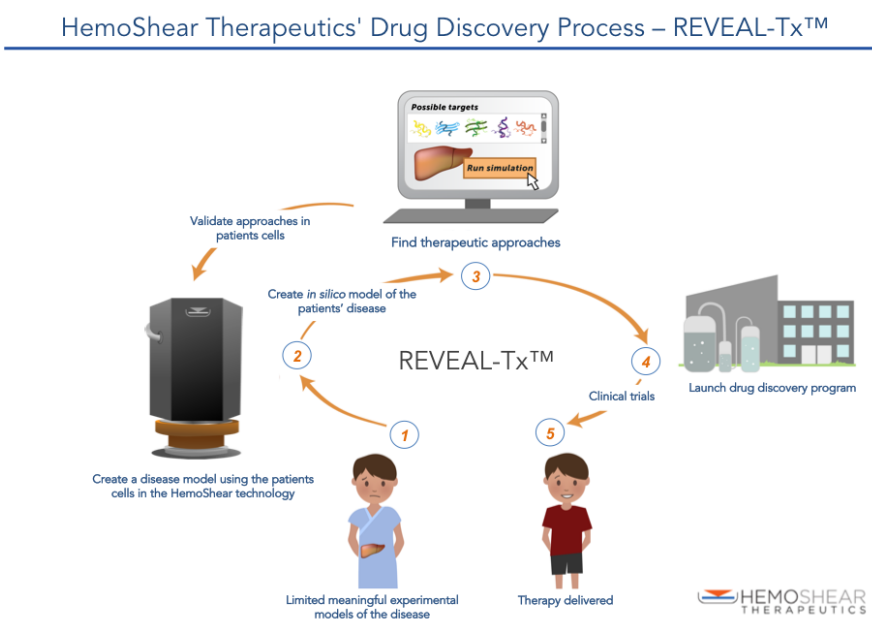
The participants were grateful for the opportunity to share their experience and learn from others with similar circumstances. A talented graphic recorder captured their insights on each topic at the meeting (see below example).

"While we have spent a lot of time in the laboratory understanding the pathophysiology of these diseases, it is critical to pair our knowledge with the day-to-day experience of patients," says John Reardon, PhD, Head of Research & Development at HemoShear. "After listening to the parents, we have a much better understanding of what would be truly meaningful improvements in their children's quality of life that we must consider when designing clinical trials."

In addition to gathering patient insights, HemoShear had the opportunity to explain the drug development process to families. Dr. Reardon gave a talk at the conference on the preclinical research steps, clinical trials and regulatory requirements for developing new therapies. He explained that patients can play an important role in advancing new treatments by sharing their medical history with researchers through natural disease registries and participating in future clinical trials. Dr. Reardon's talk was widely praised by attendees and served to strengthen HemoShear's relationship with the families and with OAA.

Sharing Progress with the MSUD Community

Brian Wamhoff, PhD, HemoShear co-founder and Head of Innovation, also attended the MSUD Family Support Group Symposium to explain how the company has made great strides in modeling MSUD and identifying new treatment targets through the REVEAL-Tx™ platform thanks to livers donated by patients (below image). Dr. Wamhoff's live talk can be viewed here.



"The MSUD Family Support Group was established in 1965 and there were more than 50 patients in attendance ranging from the ages of 1 to 50. MSUD patients and caregivers are extremely knowledgeable not only about their disorder but much of the cutting-edge science in search of a therapy. This was our first face-to-face experience with this group and it was truly a gift," said Dr. Wamhoff.

HEMOSHEAR THERAPEUTICS

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