MODELING MSUD TO ADVANCE PROGRESS TOWARDS NEW TREATMENTS

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New treatments are greatly needed to improve the quality of life for people with maple syrup urine disease (MSUD). In order for progress to be made, however, we need to really understand how the disease works and identify opportunities to disrupt and correct the faulty metabolic process of MSUD.

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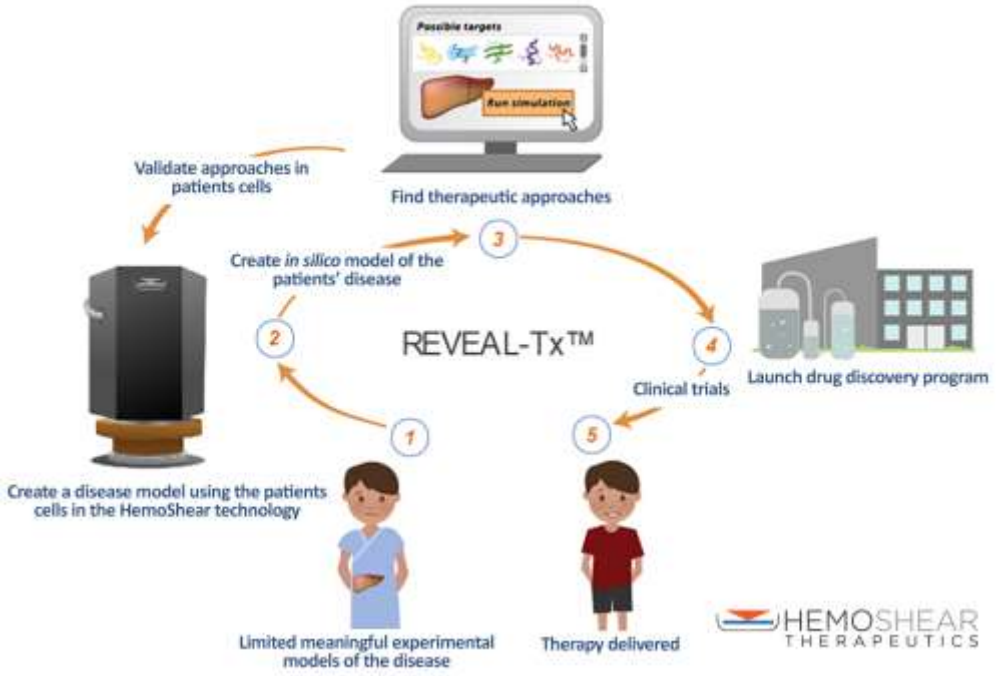

HemoShear Therapeutics' Drug Discovery Process - REVEAL-Tx™

I am head of innovation at HemoShear Therapeutics, a biotechnology company dedicated to generating the important insights that may lead to new therapies for MSUD and other rare inborn errors of metabolism.

As I shared at the MSUD Family Support Symposium in June, our company is recreating the human disease biology of MSUD. We are working beyond the traditional approach of examining static cells in a petri dish. Our innovative platform, called REVEAL-Tx,TM combines the power of dynamic human biology and computational science to discover new pathways for treating MSUD.

We have established a global network to collect the donated livers of MSUD patients who have undergone liver transplants. We have harvested the cells from these diseased livers and used our proprietary technology to build a dynamic bioreactor that recreates MSUD in our laboratories. This enables us to understand MSUD better than ever before.

We model MSUD in a computer, turning genes on and off to see if they fix the disease. We then use our bioreactor that mimics the human biology of MSUD to validate the approach.

We are making good and steady progress. Through the insights generated from our bioreactor, we have identified a novel therapeutic target for MSUD. We are now building molecules that address this target and are validating them in our system, with the goal of developing a drug to treat this disease.

This process can be painstaking and expensive. We work with a great sense of urgency as we focus on building partnerships and raising money to move a drug forward. The drug development field learns through failure. Our goal is to fail fast, so we can learn faster and maintain momentum toward developing a safe and effective treatment for MSUD.

We could not have gotten to where we are now without the support of the patients and their families who donated their livers to our research. They inspire us to continue our important work.

We will continue to keep the MSUD community aware of our progress and hope to one day move into the clinic with a promising new treatment.

See my presentation at the 2018 MSUD Family Support Meeting (https://www.msud-support.org/featured/news/718- 2018-msud-symposium-presentations)