Second Annual Million Dollar Bike Ride Grant Recipient Announced

NTSAD congratulates Marlene Jacobson, PhD, awardee of the 2015 Million Dollar Bike Ride grant made on behalf of Team NTSAD for her proposal entitled "Patient-Derived Phenotypic Assay to Discover Treatments for Tay-Sachs Disease."

Dr. Jacobson is Associate Professor in the Department of Pharmaceutical Sciences, Temple University School of Pharmacy and Associate Director of the Moulder Center for Drug Discovery Research. Dr. Jacobson's current research interests include identification of treatments for neuropathic lysosomal storage disorders and application of novel screening assay methodologies for drug discovery.

Specifically for this project funded by NTSAD and the Orphan Disease Center, Dr. Jacobson will use cells from Tay-Sachs patients to screen a collection of small molecules that could serve as treatment for this and other lysosomal storage disorders. Dr. Jacobson has developed a novel "disease in a dish" assay which will enable exploration of small molecules including a collection of known FDA-approved drugs in which a "hit" has the potential for rapid progression into clinical trials. The research will be performed in a fully integrated academic drug discovery center, The Moulder Center for Drug Discovery Research, composed of investigators with extensive experience in pre-clinical drug discovery and an established infrastructure to support high throughput screening, in vitro pharmacology, medicinal chemistry, and preclinical drug metabolism studies.

"We are excited to receive this award and wish to extend our gratitude to Team NTSAD for raising the funding to support the award." - Dr. Marlene Jacobson