
Mucopolysaccharidosis Type IV (ML4): Mucopolysaccharidosis Type IV is caused by a single-gene mutation in p19 which encodes for MCLON1. Most patients experience total loss of this transmembrane protein resulting in severe psycho-motor delays, neurodegeneration, and blindness. One \$53,634 grant is available. We offer this grant to investigators conducting research on all aspects of disease including disease pathogenesis and clinical studies. Preference will be given to those research projects focusing on gene therapy development, biomarkers, functional outcome measures to assess therapeutic impact, and natural history research. We are particularly focused on research that is collaborative and that can show impact for patients. This grant is made possible by TeamCureML4, Pedal4Paul, Dream4Danielle, Love4Rose, LovingJackHenry, Treatments4Tommy, and Bike4Austin and our Israeli partners.