

# **A Phase 3 Study of UX003 Recombinant Human Betaglucuronidase (rhGUS) Enzyme Replacement Therapy in Patients With Mucopolysaccharidosis Type 7 (MPS 7)**

Children's Hospital Oakland, Oakland, California, United States

Children's Hospital of Orange County, Orange, California, United States

Miami Children's Hospital, Miami, Florida, United States

University of Minnesota, Minneapolis, Minnesota, United States

The Phase 3 study will use a novel randomized, intra-subject placebo-controlled, single crossover design, referred to as Blind Start, to evaluate the safety and efficacy of UX003. The Blind Start is a novel design whereby participants will be randomized to 1 of 4 groups, each representing a different treatment sequence, and will cross over to UX003 at different pre-defined time points in a blinded manner. All groups will receive a minimum of 24 weeks treatment with 4 mg/kg UX003 every other week (QOW).