

RGX-111 Gene Therapy in Patients With MPS I

Children's Hospital of Philadelphia, Philadelphia, Pennsylvania, United States

University of Pennsylvania, Philadelphia, Pennsylvania, United States

RGX-111 is a gene therapy which is intended to deliver a functional copy of the α -L-iduronidase (IDUA) gene to the central nervous system. This is a safety and dose ranging study to determine whether RGX-111 is safe and tolerated by patients with MPS I.