

RGX-121 Gene Therapy in Patients With MPS II (Hunter Syndrome)

Children's Hospital of Pittsburgh - UPMC: Program for Neurodevelopment in Rare Disorders, Pittsburgh,
RGX-121 is a gene therapy which is intended to deliver a functional copy of the
iduronate-2-sulfatase (IDS) gene to the central nervous system. This study is a safety and
dose ranging study to determine whether RGX-121 is safe and tolerated by patients with MPS
II.