# A Study to Assess the Safety, Tolerability, and Efficacy of Long-term SOBI003 Treatment in Pediatric MPS IIIA Patients

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MPS IIIA, also known as Sanfilippo A, is an inherited lysosomal storage disease (LSD). MPS IIIA is caused by a deficiency in sulfamidase, one of the enzymes involved in the lysosomal degradation of the glycosaminoglycan (GAG) heparan sulfate (HS). The natural course of MPS IIIA is characterized by devastating neurodegeneration with initially mild somatic involvement. The aim of the present study is to assess the safety, tolerability and efficacy of long-term SOBI003 treatment. SOBI003 is a chemically modified recombinant human (rh) Sulfamidase developed as an enzyme replacement therapy (ERT).