A Study to Assess the Safety and Tolerability of SOBI003 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 aims of the present study is to assess the dose related safety, tolerability, PK and PD of SOBI003, a chemically modified recombinant human (rh) Sulfamidase developed as an enzyme replacement therapy (ERT).