

Randomized, Controlled, Open-label, Multicenter, Safety and Efficacy Study of rhHNS Administration Via an IDDD in Pediatric Patients With Early Stage MPS IIIA Disease

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Sanfilippo syndrome Type A, or Mucopolysaccharidosis (MPS) IIIA, is a rare lysosomal storage disease caused by deficiency of the enzyme heparan N-sulfatase (sulfamidase). In the absence of this enzyme, there is an accumulation of the glycosaminoglycan, heparan sulfate, resulting in progressive neurodegeneration. Symptoms are usually first noted in the 1st or 2nd year of life, although definitive diagnosis is often delayed, with an average age of diagnosis of 4.5 years. The disease is characterized by developmental delays initially, followed by neurological developmental arrest, then regression. These developmental deficits are typically associated with severe behavioral disturbances. Patients have a significantly reduced lifespan, with few surviving beyond the 2nd or 3rd decade.

The purpose of this study is to evaluate the safety and efficacy of recombinant human heparan-N-sulfatase (rhHNS) in pediatric patients with Early Stage Mucopolysaccharidosis Type III A Disease.