# Clinical Trial Case Study

## Pediatric Study in Mucopolysaccharidosis type III (MPSIIIA)

### Type of study

An Open, Non-Controlled, Parallel, Ascending Multiple-Dose, Multicenter Study To Assess The Safety, Tolerability, Pharmacokinetics And Pharmacodynamics Of a Chemical Compound in Pediatric Patients with Mucopolysaccharidosis type III (MPSIIIA)

**Phase:** I/II

### Key Achievements

- Multi-center study with a centralized single database
- Adherence to incredibly tight timelines and a short enrollment period
- Maximize testing with minimum blood volume due to patient demography

### Study Details

**Patients Screened:** 16

**Patients Enrolled:** 9 (between 1 and 6 years old)

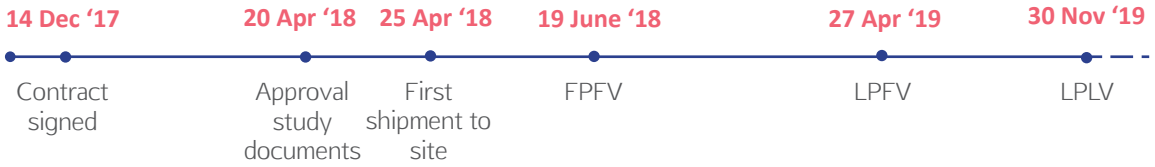
**Sites:** 5

**Regions-Countries:** North America, Western, Central and Southern Europe

**Services Provided:**

- Safety testing
- Acute infusion reaction or anaphylactic reaction testing (IL-1Ra, TNF-a, IgE, tryptase and CRP)
- Specialized testing histology lab (white blood cell isolation from whole blood for XXX activity, DBS card for SGSH genotyping, purification of fibroblasts from skin biopsy for CRIM status)
- Sample management (PK, ADA, and neutralizing antibodies, collection of other samples such as serum, urine, whole blood and CSF)

### Timeline




### Challenges

In pediatric studies, one of the biggest challenges is the permitted and available blood volume. Blood sampling from children has strict limits, and there are different guidelines from globally recognized bodies. In addition, sampling materials such as needles, IV-lines, and more need to be adjusted for children. Sample loss in these instances is near catastrophic for all of the above reasons and accentuated in orphan indications.

### Successes

Our teams were able to maximize testing with a minimum amount of blood volume. Guidelines with specific recommendations concerning sampling materials suitable for pediatric patients were implemented and closely monitored during the study's set-up and duration. Additionally, we took extra measures to track samples in transit to avoid the loss of already scarce samples.

Cerba Research

CERBA RESEARCH HQ
Industriepark Zwijnaarde 3
9052 Gent, Belgium

Tel: +32 9 329 23 29
info@cerbaresearch.com
www.cerbaresearch.com