Case study

SIMBEC-ORION

Surpassing timelines & rapid enrolment in an ultra-rare paediatric study

Niemann-pick type c

A prospective non-therapeutic study in patients diagnosed with Niemann-Pick disease type C (NP-C), characterising the individual patient disease profile and historic signo-symptomatology progression pattern.

Background

The sponsor needed to produce a new batch of their product with concentrations adapted based on the weight of paediatric patients. An observational study was needed to identify and profile patients before progressing to an interventional study. Patients enrolled were between 2 to 18 years of age with a diagnosis of NP-C.

NP-C is an ultra-rare indication in paediatric patients, requiring an agile CRO who could work with the sponsor as the study evolved.

Objectives

- To characterise the individual patient disease progression profile (disease burden and progression) through the clinical, imaging, biological status, and quality of life prospectively recorded, together with the historic disease information collected from patient medical records.
- The secondary objective was to evaluate the safety data of the disease-related therapy and to record every adverse event (AE) linked to the disease.

Patient-centric approach critical to success of the study

Ultra-rare paediatric indication

Observational study smoothly moved in to interventional study within 10 months

Regulatory team proactively worked with the clinical authorities and ethics committees

Key opinion leader (KOL) review and input of the protocol and study design

Central laboratory provided time-critical delivery of samples

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Challenges

Niemann-Pick type C (NP-C) is defined as an ultra-rare disease affecting an estimated 1:150,000 people, with around 500 patients identified globally.

To meet the scientific objectives of their study the sponsor needed us to identify centres which would excel in a patient-centric study. It was important that selected centres not only diagnosed and treated patients with Niemann-Pick Type C, but that they also understood the unique medical needs of their patients.

With a paediatric patient group ranging from 2 to 18 years, the study would need to consider patient's individual requirements while adhering to additional paediatric regulations.

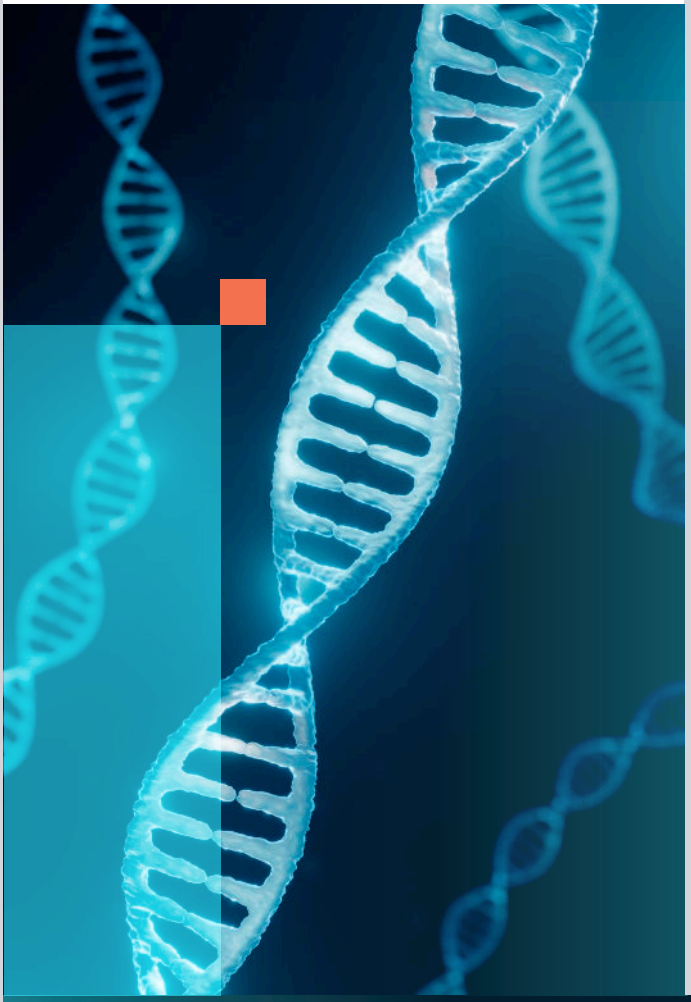
Solutions

- Simbec-Orion deployed CRAs to evaluate centres of excellence with capabilities to perform this complex clinical trial in more than 18 European countries, resulting in 16 sites across 11 countries.
- Our regulatory team prepared the necessary documentation and directly interacted with each of the clinical authorities and ethics committees to promptly address any potential concerns.
- We took an active role in the writing of the protocol, and were responsible for designing the eCRF, collaborating with the sponsor to prepare the innovative statistical plan.
- Our regulatory team prepared the necessary documentation and directly interacted with each of the clinical authorities and ethics committees to promptly address any potential concerns.
- Designed the PICF (Patient Informed Consent Form) in all the required languages, adapting them to be suitable for the different paediatric age groups concerned.
- Mobilised the patients and families to be included in the study and provided the appropriate legal support to adapt the sites contracts upon the individual requirements.
- To accelerate timelines, we were able to organise the sample preparation under special conditions, precisely coordinating the time-critical delivery to our central laboratory.

Outcome

The observational study took just 10 months to complete and was swiftly integrated into the interventional study once the IMP was ready and the drug supply organised.

The sponsor was able expand the Phase II/ III interventional study in the US and Europe, with enrolment for the interventional study completed in record time.



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