

MEETING THE CHALLENGES OF A FRAGILE, PEDIATRIC RARE DISEASE POPULATION

OVERVIEW

The study was designed to evaluate the safety for a therapeutic administered once weekly by intravenous (IV) infusion for male Hunter syndrome patients under five years of age.

The study would also evaluate and explore:

- Pharmacodynamic effects
- Single- and repeat-dose pharmacokinetic parameters in patients
- Liver and spleen volumes by abdominal ultrasound
- Routine development milestones using the Denver II Developmental Screening Test
- Growth using height and weight measurements
- Significant clinical events that reflect disease progression, e.g., the occurrence of respiratory-related events, hearing-related events, and surgical procedures

CHALLENGES

The most significant challenge involved the fragile, pediatric population in this rare disease. The recruitment and retention of study participants spanned country borders and travel logistics required special attention. Caregivers needed to be engaged and supported. An enhanced level of investigator management was required as the sites had not previously conducted clinical research.

SOLUTION

To accommodate what were relatively long travels distances for a sickly child, inVentiv Health Clinical collaborated with the sites on logistics and managed reimbursement for the patient's and parent s' travel arrangements on behalf of the sponsor.

It was also necessary to handle several deviations to the protocol due to patient conditions that disallowed some procedures to be performed. inVentiv Health Clinical facilitated communication between the sites and sponsor and ensured that justification and approval for deviations were properly documented.

Clinical research naïve sites were provided extensive ICH-GCP training and periodic communications throughout the study. Monitors established excellent relationships with the study sites which facilitated rapid information transfer to accommodate frequent data-cut-offs for regulatory safety reporting purposes.

The drug supply was closely monitored within a cold supply chain and the monitor was notified of any drug storage conditions outside the required temperature range.

RESULTS

In collaboration with the sponsor, inVentiv Health Clinical successfully completed enrollment within the sponsor's timelines in a challenging population of study participants.

ABOUT INVENTIV HEALTH CLINICAL

inVentiv Health Clinical, formerly PharmaNet/i3, is a leading provider of global drug development services to pharmaceutical, biotechnology, generic drug, and medical device companies. With 7,000 employees in more than 36 countries, inVentiv Health Clinical offers therapeutically specialized capabilities for all phases of clinical development, bioanalytical services, and strategic resourcing from a single clinical professional to an entire functional team.

➤➤ TRANSFORMING PROMISING IDEAS INTO COMMERCIAL REALITY

CASE STUDY

Therapeutic Area
Neuroscience

Indication
Hunter's Syndrome

Clinical Phase
Phase IV

Participating Countries
3 – Taiwan, Poland, Brazil

Study Details

- Number of activities: 3
- Patient randomization: 30

Services

- Clinical Monitoring
- Clinical Supplies Management
- Data Management
- Medical Monitoring
- Medical Writing
- Pharmacovigilance
- Project Management
- Regulatory Review and Submission in US and CA
- Site Management, Contracts and Administration
- Vendor Management