



# Global Pediatric Full-Service Phase I/II Clinical Trial Begins on Heels of Successful Natural History Study

## Veristat Enables Proof-of-Concept Study to Drive Novel Gene Therapy Program

### Background

A mid-size biotechnology company sought Veristat early in clinical development for help with a natural history trial targeting an ultra-rare neurological disease in a pediatric population with no available therapies. After the successful implementation of the natural history study, the sponsor moved forward with the novel gene therapy treatment program, which has already treated its first patients.

Veristat continues to manage the ongoing natural history study and has started the Phase I/II clinical trial, providing full-service support including strategic consulting, statistical and programming support, site, data and program management, and medical affairs services.

## Study Demographics



### Indication

Ultra-rare, genetic CNS disease



### Biologics Therapy Type

Gene therapy



### Full-Service Support

- Regulatory Consulting
- Biostatistics
- Programming
- Data Management
- Project Management
- Site Management
- Medical Affairs



### Phase I/II Treatment Trial



### Demographics

2-4 sites in North America & Europe, goal of nearly 50 patients



*We really appreciate the extra effort and hours you and your team must have put in to get this done. Thanks for going above and beyond yet again to address a time-sensitive need for our program.”*

— Project Lead, Sponsor

## SOLUTION

A global rare disease clinical trial demands an exceptional level of coordination. With the added complexity of patients needing to relocate for this study, there are more responsibilities required of the sponsor than compared to a typical clinical program. Throughout the trials, Veristat team worked diligently to:

- Successfully streamline the site start-up process.
- Design a series of check-ins and communication tools to foster understanding, communication, and visibility into the trials among sites, patients, and families.
- Provide comprehensive support to the sponsor throughout the entire process, particularly for international subjects and families who faced great challenges compared to US subjects due to the need for relocation. This encompassed a wide range of services, including arranging long-term living accommodations near the treatment center, deploying social workers, providing transportation and translation services, supplying furniture and household items, and various other forms of aid.
- Due to a lack of a strong sponsor statistics team, Veristat's biostatistics experts supported the sponsor in moving the study forward by producing outputs needed for data sharing and successful endpoint analyses.
- Provide FDA insights to assist in regulatory decision-making.

## IMPACT

### Stepping Up to Serve as an Implementation Arm of the Sponsor

To ensure a seamless trial and optimize the patient experience, Veristat's multidisciplinary team demonstrated remarkable flexibility in accommodating the sponsor's unique requirements and solving unexpected challenges across numerous areas. Our bold-thinking experts are committed to providing tailored solutions to both sponsors and patients, even if unconventional or outside the standard framework, and acting as an advocate for the clinical sites to address all sponsor and subject needs.

The Phase I/II trial is ongoing and will continue over the next two years. Currently, more than five patients have received the gene therapy with the goal of treating nearly 50 patients.

## ABOUT VERISTAT

### CRO of Choice for Biologic Therapies

We understand how high the stakes are with your cell or gene therapy program. Nothing is standard about study design, study conduct, or regulatory process in this specialized area. In the past three years, Veristat has successfully conducted more than 40 full-service biologics trials for more than 20 sponsors, with a global team of scientific experts who are adept at both strategy and execution across the clinical development journey. Whatever the study's unique considerations – patients, products, process, follow-up, regulatory – Veristat can help you successfully get through it.

# Contact Veristat Today

To learn more about Veristat or how we can assist you, reach out to us today.

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