VERISTAT

Pursuing Breakthrough Therapies for Rare Metabolic Diseases

How Veristat's Full-Service Clinical Trial Team Helped Tightly Coordinate a Gene Therapy's Dosing Administration and Data Review

Background

A biotech organization focusing on gene therapies for metabolic diseases partnered with Veristat to support their Phase I/II clinical study for the treatment of a rare human genetic lysosomal storage disorder.

Study Demographics

Study Phase
Multinational Phase I/II parent study and long-term follow-up study

Indication
Rare genetic lysosomal storage disorder

Biologics Therapy Type
Gene therapy

Full-Service Support
- Clinical Operations
- Project Management
- Site Management & Monitoring
- Data Management

SPONSOR CHALLENGE 1: Time-sensitive Therapy Administration Process

For this study, the investigational product (IP) was manufactured from each subject's own stem cells. Manufacturing slots were limited and needed to be reserved at least six months in advance. If the slot was not able to be used and was not cancelled within six weeks of the manufacturing date, the sponsor was responsible for paying a substantial late cancellation fee. This requirement placed a constraint on subject scheduling for stem cell collection: subjects needed to undergo stem cell collection 1-2 days prior to the reserved manufacturing slot.

Prior to enrollment and stem cell collection, subjects underwent a multi-phased screening process. Labs for eligibility assessments needed to be collected within the screening window and results were needed at least six weeks prior to the manufacturing slot to avoid a cancellation fee. These tight timelines required meticulous control and coordination.

Veristat Solution

Manufacturing slots were determined by the sponsor and manufacturer in six-month blocks and the dates were shared with Veristat approximately six months prior to the first slot in the block. As Principal Investigators (PIs) identified and pre-screened potential subjects, they were tentatively assigned a slot, with back-ups assigned when available.

Veristat provided a detailed timeline of screening assessments based on the manufacturing dates and shared these timelines with the sites. Veristat closely coordinated with the labs processing screening samples to ensure testing was fast-tracked to stay on schedule. Veristat's Site Management Team worked closely with the sites to ensure sites' adherence to the screening timeline.

SPONSOR CHALLENGE 2: Expedited Data Reviews Between Cohorts

This study included a cascading enrollment scheme requiring Data Monitoring Committee (DMC) reviews of 30-day data for each cohort prior to opening the next cohort for enrollment. As previously shared, the screening and stem cell collection timeline was tightly controlled and difficult to reschedule; therefore, DMC meetings between cohorts needed to be scheduled for the window between the availability of 30-day treatment data and the manufacturing date for the next subject. There was one instance that was further complicated by a treatment delay for the patient in the preceding cohort, which pushed back the date of the availability of 30-day data. largely based on the manufacturing requirements of the therapy whose manufacturing dates could not be changed.

Veristat Solution

Veristat worked closely with the sponsor and the DMC members to accommodate this tight timeline. Veristat organized a DMC meeting to facilitate an early review of the data through two weeks post-treatment. This initial review by the DMC allowed the subject in the

next cohort to move forward in preparation for stem cell extraction. Veristat then provided weekly updates to the DMC of any new safety events that occurred. Updated data were provided to the DMC once 30-day data were available and the DMC met virtually to determine whether the next subject could move forward with treatment. They confirmed and the subject moved forward with stem cell extraction within two days of the DMC's decision.

IMPACT

- Flexibility to work with the sponsor as a true partner and fulfill their study needs.
- Collaborative partnership between Veristat and the sponsor addressed critical patient, lab, and manufacturing logistics.
- High sponsor satisfaction with Veristat's communication and coordination efforts.
- Flexibility served to accommodate a study with challenging patient enrollment and study logistic requirements.

Veristat continues to work with the sponsor in managing post-transplantation patient requirements through a long-term follow-up study.

MEET VERISTAT

Are you getting ready to run a clinical trial or is your current trial experiencing setbacks? Our Clinical Operations, Medical Affairs, Site Management, Project Management, and Data Management teams work together as an integrated team. We strive to be responsive, flexible, and mindful throughout the trial and site management process, looking for innovative ways to improve clinical operations and site productivity. Significant emphasis is placed on flexible and creative approaches that highlight efficiency and streamlining of activities, thereby providing optimal solutions for our clients.

Contact Veristat Today

To learn more about Veristat's tailored clinical trial deployment solutions or how we can assist you in determining if our expertise meets your needs, reach out to us today.

www.veristat.com