VERISTAT

Flexibility Drives Progress During a Rare Disease Gene Therapy Clinical Trial

How Veristat Helped a Biotech Company Address Clinical Operations Challenges to Meet Changing Requirements

Background

Veristat was engaged by a biotech organization focusing on gene therapies for metabolic diseases to support their clinical study for the treatment of a rare human genetic lysosomal storage disorder.

Study Demographics

Study Phase
Multinational Phase 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
- Medical Affairs
- Project Management
- Site Management & Monitoring
- Data Management*

*Long-term follow-up study service

SPONSOR CHALLENGE 1: Site Enrollment

The sponsor initially provided Veristat with a list of sites they believed to be suitable to assess the safety/tolerability/efficacy of the gene therapy through several clinical, molecular, and biochemical assessments. Unfortunately, none of the sites provided by the sponsor proved viable.

Veristat Solution

Veristat's Medical Affairs team conducted peer-to-peer conversations with numerous clinicians involved in the treatment of the disease and used its existing network of principal investigators (PIs) and sites to support a successful study start. An extensive tracker of physicians and their site capabilities was created for contact control and sponsor reporting. Nearly 10 sites were recruited globally by Veristat and the sponsor.

As a standard process in assisting sponsors with the evaluation and site identification process, Veristat authored a comprehensive site feasibility questionnaire to confirm:

- Investigator and site knowledge and experience administering a gene therapy.
- Site availability on a morning-to-evening basis to accommodate patient scheduling requirements.
- Site and staff availability to conduct blood draws at specific times and intervals to inform patient dosing.
- Eligible patient pool and network for patient referrals.
- Site experience with collecting/processing and shipping specimen samples to specialty labs.
- Site experience with shipping harvested cells to manufacturing facility as well as receiving and infusing the manufactured product.
- Site experience in administering conditioning therapies prior to gene therapy.
- Availability of PIs and sub-investigators during conditioning and gene-therapy administration.
- Therapeutic dose monitoring (TDM) capabilities at the site and site ability to work with outside TDM facilities.
- Availability of apheresis unit and hematology/BMT unit.

Veristat provided information back to the sponsor regarding key logistical and subject recruitment challenges communicated by the sites. With the information provided, the sponsor amended the protocol where possible to address some of the challenges.

SPONSOR CHALLENGE 2: Time-sensitive Therapy Administration Process

Eligible patients needed to undergo a multi-phased screening and carefully timed cell collection and gene therapy administration process. The process was largely based on the manufacturing requirements of the therapy whose manufacturing dates could not be changed.

Veristat Solution

Subject screening/baseline assessments were planned, working backward, from the manufacturing date that had been reserved. A detailed schedule for screening/baseline assessments was provided to the sites with the Veristat team following up to ensure that all activities were completed as planned.

SPONSOR CHALLENGE 3: Expansion of Metrics Reporting Requirements

The sponsor management team had several new members join during the duration of the study who came from organizations that tracked several incremental data points not on the original metrics summary report. Additionally, ADI and risk log reporting were also requested by the new team members.

Veristat Solution

Veristat was able to quickly respond with:

> A sample specimen tracker that tracked samples required for each subject at each visit (per protocol), the samples actually drawn, and if results were available for each sample that was drawn (if not, the reason was provided). This effort ensured the timely tracking of all subject samples.

> A customized ADI and risk log to meet the sponsor's requirements.

> Updated monthly metric reports that tracked several robust measures, including:

- Time from final protocol to first site activation
- % MVs performed per monitoring plan (MP)
- % MVRs finalized beyond scope per MP
- Timeliness of action item resolution
- % important PDs
- % SDV vs. available CRF pages
- Timeliness of data entry
- Timeliness of query resolution
- % data frozen
- % outstanding external data query
- % samples analyzed vs expected samples
- AE/SAE rates per site
- Subject enrollment tracking
- % scheduled QC checks conducted per TMF Plan
- % errors found in TMF QC checks
- % study-specific team training completed by the due date
- Delayed escalation of critical issues
- # major quality issues
- % milestones achieved

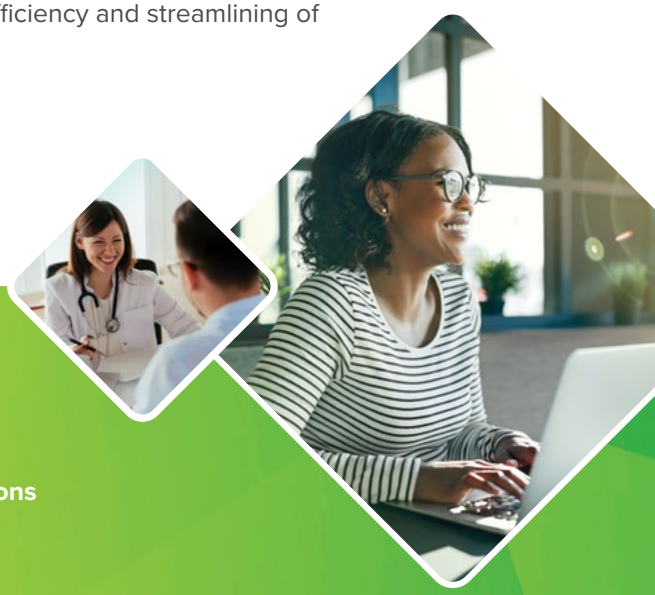
IMPACT

- Flexibility to work with our sponsor as a true partner fulfilled their changing study needs.
- Collaborative partnership between Veristat and our sponsor addressed critical patient, lab, product manufacturing, logistics, and tracking/ reporting requirements.
- High sponsor satisfaction with Veristat's thorough site evaluation led to successful site recruitment and enrollment.
- Flexibility served to accommodate a study with challenging patient enrollment and study logistic requirements.
- Development of comprehensive metrics reporting, specimen sample tracker, and ADI/Risk log supported ongoing study assessments.

Although the study was halted due to disappointing efficacy results, 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.

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