› CASE STUDY

VERISTAT

Full Service Support for a Rare Pediatric Gene Therapy Program

Veristat named “Gold Standard CRO” for work on critical program

Background: Guiding a Biotech's Gene Therapy Trials

A clinical-stage biotechnology start-up asked Veristat to run complex US and EU trials of their unique gene therapy. Veristat brought invaluable global capabilities and expertise to a small client team tackling their first clinical stage program without the large infrastructure or resources to conduct studies in this space themselves. Our end-to-end support started at Phase I and will continue into post-marketing and regulatory follow-up.

Study Demographics

Rare Pediatric Global Trial:

Patients from Europe and US, each with central sites and local hospitals

Primary Services Provided:

- Project management
- Clinical monitoring
- Medical monitoring
- Pharmacovigilance
- Data management
- Regulatory affairs

SOLUTION

Managing a Multifaceted Trial from Start to Finish

Throughout the engagement, Veristat's global cell and gene therapy team utilized our extensive expertise across the entire clinical program, leveraging our relationships with key global regulatory agencies and superior knowledge in gene therapy logistics, including enhanced requirements for handling of genetic material.

PHASE I

- Successfully **rescued early studies** from a former CRO partner
- Activities included **project management, clinical monitoring, medical monitoring, pharmacovigilance, data management, and regulatory submissions and approvals**

PHASE II

- Drawing on our strong relationships with global regulators and high-quality processes, were able to **anticipate agency questions and have proper answers prepared**
- Handled **all core clinical development activities**, including clinical monitoring, project management, data management, regulatory and medical affairs
- **Used game-changing new central site model:** patients treated with gene therapy at central sites in the US and EU, then treated by local physicians to avoid unnecessary travel burden on patients and caregivers

PHASE III

- Created process for **cross-continent handling and transporting sensitive cell material** – *for US West Coast patients, harvested cells had to be transported to EU GMP facility in 48 hours*
- Handled **all core clinical development activities**, including clinical monitoring, project management, data management, regulatory and medical affairs
- Drew upon our qualified partners and extensive **experience managing the complicated logistics of gene therapy trials**

BLA SUBMISSION

- Successfully responded to a **Full Safety Review** across all clinical programs for Investigator re-assessment of SAEs following global safety review data
- Drawing on our inspection experience and high-quality process, **managed inspection readiness activities at clinical sites and with the sponsor – including mock inspection, training and process development – to ensure a smooth inspection process on pre-market approval inspections**
- Utilized our flexible, client-focused approach to **provide *ad hoc* data outputs and documentation to support preparation of the BLA submission package**

Unique Adverse Events Reporting Requirements

Cell Harvest Procedures & Conditioning regimen: Autologous gene therapies require harvest of the patient's own stem cells either via bone marrow harvest or apheresis. These procedures have added risks because of the additional medications required to facilitate the harvest or the procedures themselves e.g. surgical related Adverse Events (AEs) through bone marrow harvest or potential Central/PICC line insertion/ infection risks.

In addition, to allow for successful engraftment, gene therapy administration regularly requires administration of a chemotherapy conditioning agent, leading to chemotherapy-related toxicity.

We developed a unique AEs reporting system to allow sub-classification of AEs to separate out those AEs related to the harvest procedures or conditioning regimen, vs those actually related to the investigation product.

Expanded Access / Compassionate Use Programs

Patients with life-threatening, long-lasting or seriously debilitating illnesses that cannot be treated satisfactorily with any currently authorized medicine are eligible for Expanded Access and Compassionate Use programs that allow them to use investigational drugs undergoing clinical trials. Veristat was able to secure FDA authorization and EMA approvals to provide gene therapy to pediatric patients who were too sick to participate in the client's trials, enabling critical treatment for those who need it most.

IMPACT

Confronted with various hurdles, an evolving regulatory environment and logistical complexities, Veristat successfully created new agile processes to help our client reach the application stage for its gene therapy.

Veristat Sets Gold Standard

The client named Veristat their “Gold Standard CRO” for our work on these studies due to the quality of our end-to-end support. Going forward, we will use the same project teams for new work to maintain the gene therapy technology experience dedicated to this client, and to take advantage of best practices and lessons learned.

About Veristat

The cell and gene therapy partner of choice

We understand how high the stakes are with your cell and gene therapy program. We know that nothing is standard about study design, study conduct or regulatory process in this specialized area. Veristat has successfully supported more than 100 cell and gene therapy projects, with a seasoned global team of scientific experts who are adept at strategy and execution across the clinical development pathway. Learn more about Veristat and how we can assist you with your cell or gene therapy trial development, execution, and regulatory submission preparation.

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