VERISTAT

Supporting Global Approval of a First-in-Class Gene Therapy for a Rare Genetic Disorder

Background

A biopharmaceutical company developing a first-in-class gene therapy for a rare, life-threatening genetic disorder engaged Veristat to support its clinical development and regulatory strategy. The therapy represented a significant advancement for patients with limited treatment options and was being evaluated through a complex global development program involving multiple studies, interim analyses, and evolving regulatory expectations.

Throughout the engagement, Veristat supported our client during critical stages of development, including preparation for regulatory inspections, ongoing clinical oversight, and coordination across multiple functional teams. The program ultimately progressed toward regulatory submissions in both the United States and Europe.

Challenges

Complex, Multi-Year Clinical Development
The program involved multiple studies, interim analyses, and long-term follow-up, requiring sustained operational consistency and documentation rigor over several years.

High Regulatory Scrutiny and Inspection Readiness
The client underwent CRO transitions, Sponsor transitions and regulatory authority inspections, all requiring continuous inspection readiness.

Dynamic Study Requirements
Evolving requirements, such as changes in monitoring intensity and source data verification (SDV) expectations, required rapid adaptation without disrupting study timelines.

Global Coordination and Transition Management
The program included cross-functional coordination across regions, introducing complexity in communication, oversight, and execution.

STUDY DEMOGRAPHICS

STUDY PHASE
- Multi-year, multi-study global clinical development program

DISEASE
- Rare, life-threatening genetic disorder

PROGRAM MILESTONES
- IND submission and maintenance
- Multiple interim analyses
- BLA and CHMP submissions
- FDA and EMA inspections
- Regulatory approval in US and EU

SERVICES PROVIDED
- Clinical Operations and Monitoring
- Regulatory Inspection Support
- Project Management
- Data Review Coordination

Solutions

Adaptive Clinical Monitoring and Oversight
Experienced Veristat clinical professionals, including dedicated monitors, responded quickly to our client's evolving needs. This included providing increased SDV coverage on short notice and accommodating changes to monitoring strategy throughout the trial lifecycle.

Inspection and Audit Readiness Support
The Veristat team played a central role in preparing the sites and TMF for regulatory inspections (EMA and FDA), ensuring documentation completeness, formatting compliance, and inspection readiness.

Cross-Functional Collaboration
Our team collaborated closely with the client's regulatory, biostatistics, data management, and medical writing teams to ensure unified timelines and consistent data presentation, especially during multiple interim analyses and submission preparation phases.

Sustained Program Engagement
Veristat remained actively engaged throughout the multi-year program, rapidly onboarding new team members, and maintaining alignment across all workstreams.

Impact

The program culminated in regulatory approvals in both the United States and Europe, marking the first approved gene therapy for this rare genetic disorder. The full approval represented a major milestone for patients with limited treatment options and demonstrated the effectiveness of a coordinated, cross-functional approach to complex clinical development.

The collaboration enabled our client to maintain momentum through a demanding development timeline and deliver a transformative therapy to patients in need.

Meet Veristat

Rare Disease and Cell & Gene Therapy Expertise That Accelerates Success
If you've struggled with missed deadlines or regulatory uncertainty, talk to Veristat. We have deep experience supporting rare disease and gene therapy development programs from first-in-human trials through global approvals. With specialized expertise and a proven track record, our team accelerates complex clinical development without compromising quality. It's not just business for Veristat—it's personal.

Contact Veristat Today

Learn how our strategic and regulatory expertise can guide your study to success.

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