› CASE STUDY

VERISTAT

Cell and Gene Therapy Team Guides Troubled Rare Disease Programs to Success

One Study Leads to an Ongoing Partnership

Background: Complex Trial for a Rare Pediatric Gene Therapy

A clinical-stage biotechnology start-up asked Veristat to run a new European trial of their complex gene therapy. After a successful bid defense, the sponsor asked us to rescue three ongoing studies because of quality concerns. Veristat experts complemented the client's small team taking on their first clinical stage program. Our global cell and gene therapy team brought knowledge in genetic material handling, relationships with key global regulatory agencies, and expertise in accelerated pathways to approval.

Study Demographics

Rare Pediatric Global Trial:

Patients from all over the world with central sites and local primary care centers

Primary Services Provided:

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

SOLUTION

Roadmap to BLA Submission for Three Rescue Studies

Veristat began by putting together a letter of intent with the client so we could hit the ground running to be prepared for the BLA submission. We performed a gap analysis of the three rescue studies to assess the other CRO's work to date. As this was a rare disease program with a small patient base, every data point counted.

Of the three studies to rescue, one was in Europe and two in the United States (US). The two studies in the US had not been adequately managed or monitored and the databases were poor, having major issues with data integrity, GCP and 21 CFR Part 11 compliance. These studies were pivotal and may have led to significant inspection findings with the potential to delay marketing authorization.

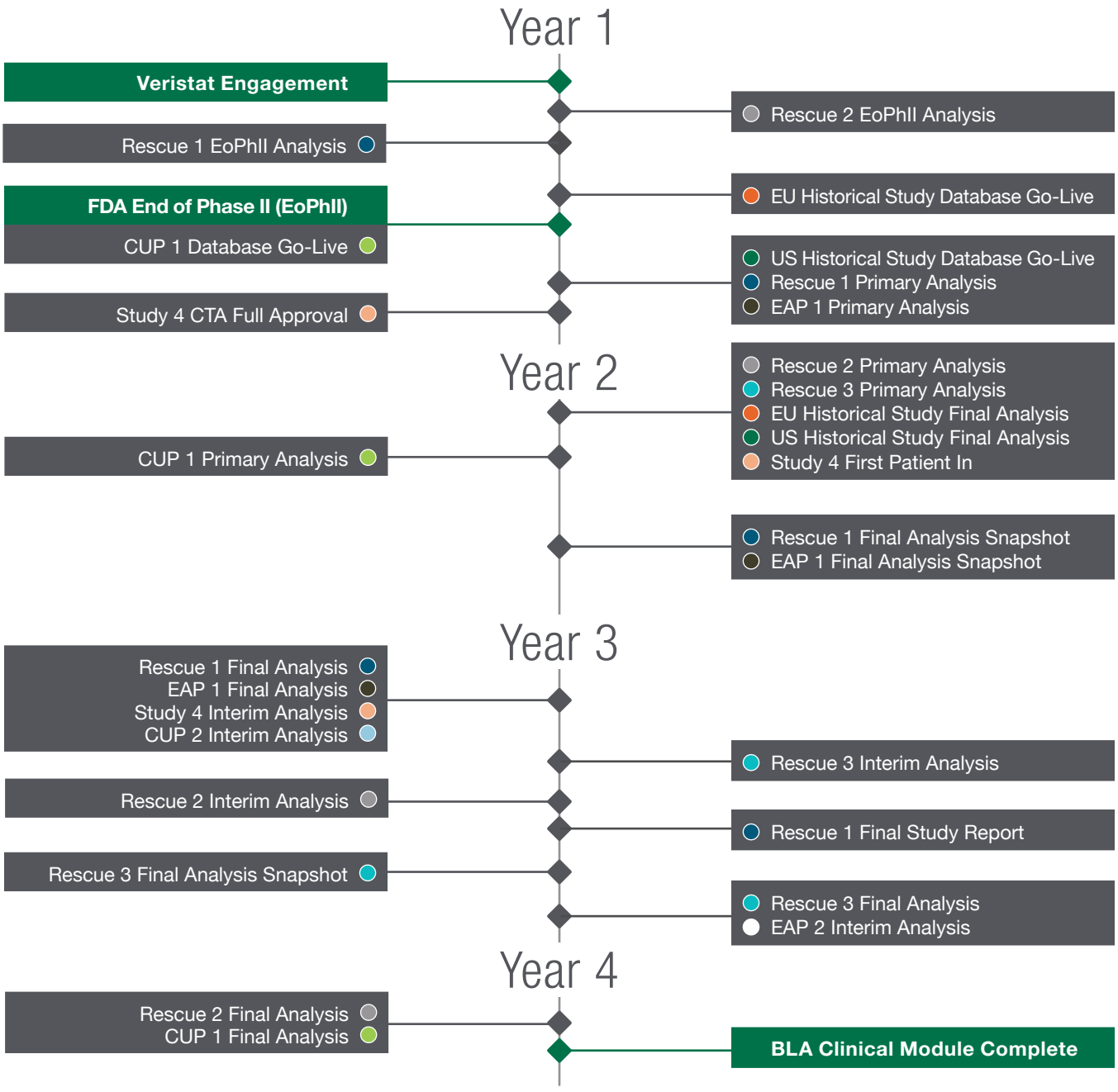
We assessed all available elements and took steps necessary in the interest of the study's success. The new European cryopreserved formulation study was set up in parallel to gap analyses and back-to-back database locks on the rescue studies. There were conflicting priorities and tight timelines to navigate.

Game-Changing Central Site Model

As this was now a global program, Veristat set up a new model allowing patients to receive the gene therapy treatment at the central sites in the US and Europe. The patients then went to their local primary care doctor for efficient follow-up and data gathering.

Ongoing Collaboration

Timelines were aggressive, requiring multiple database locks across 15 months. Our team worked on the gap analysis and on the database locks simultaneously to keep the BLA on track. We made sure the studies were brought up to standard and had no impact on timeline to be BLA ready.



IMPACT

Veristat Sets Gold Standard

The client is now prepared to submit for BLA and has asked us to handle European regulatory affairs on this program, set up a new study, rescue two additional programs, and perform a gap analysis on another program because of the quality of services provided.

The client named Veristat their gold standard CRO for our work on these studies. We are using the same project teams for the new studies. Doing so keeps the gene therapy technology experience dedicated to each study and affords efficiencies of applying lessons learned to improve processes and to run all studies with consistent forms, templates and standard operating procedures (SOPs).

> *"I first used Veristat to bail out a study we had placed with a well-known global CRO which had been unable to deliver to the agreed timelines and budget. We went on to use them for all our development programs worldwide. They have always been responsive, efficient and prepared 'to go the extra mile'."*
>
> *Director of Clinical and Regulatory Affairs, start-up biotech*

ABOUT VERISTAT

Every cell and gene therapy is unique, involving a complicated regulatory approval process full of unknowns. Veristat has assembled an extraordinary global team of experts in this field to help your study avoid errors in design, data collection or analysis. We're here to help you get your compound to market faster and positively impact patients' lives.

Contact Veristat Today

Learn more about our patient recruitment, consulting, data analysis, or regulatory expertise.

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