Less Than 1 Month to Unlock Database and Capture 2,700 Lab Records

Introduction

When an emerging biotech company was conducting clinical trials for a breakthrough cell therapy treatment, a novel cord blood therapy. The treatment aimed to minimize the outcomes associated with hematopoietic stem cell transplant. Emmes has been partnering with this research institution since 2010 in providing data management, biostatistics, monitoring and project management services. Advantage EDC was used to support data entry and capture.

The phase 3 study, which began in 2017, was a randomized study to assess the effect of the novel cord blood treatment in participants with hematological malignancies. The study included 55 clinical sites across 9 countries including the United States, Spain, the Netherlands, and Singapore. Ultimately there were 119 patients treated with the novel cord blood therapy. The primary endpoint was neutrophil counts measured at 1-week intervals over 6 weeks. Having 3 measures above a pre-determined level indicated successful treatment. The database was successfully locked on time in April 2021 and the BLA was submitted to the FDA.

Challenge

With COVID treatments prioritized for review at the FDA during 2021 and 2022, it wasn't until the second half of 2022 that the FDA began its review. In September 2022, the FDA requested that more laboratory data be included in the BLA submission. Instead of weekly neutrophil counts, the FDA requested that daily neutrophil counts be included to more accurately determine when neutrophil recovery occurred.

To accommodate the request, the database was unlocked in September 2022 so that the additional lab data could be entered. The Emmes team worked to prepare Advantage EDC to accept supplemental data, trained the clinical site staff to enter this data, and conducted extensive data cleaning to ensure the data was ready for submission to the FDA. Additional data was entered, and the database relocked over 29 days, an unprecedented timeline to respond to the FDA request.

Data Collected

119	Patients Treated
55	Trial Sites
9	Countries
Day	7, 14, 21, 28, 35, 42 Neutrophil Counts

Supplemental Data Collected

2,700	Lab Records

Outcome

Ultimately the study achieved its primary objective, showing that the breakthrough therapy led to neutrophil recovery in 11 days, compared to 22 days in the control group. The FCSR was used as the basis for a BLA to the FDA which for market approval of the breakthrough cell therapy.

The flexibility and ease-of-use within Advantage EDC meant the database could be modified quickly with simple training plans for site staff. Additionally, with the close collaboration between Emmes Data Management and the Advantage EDC development team, the EDC was able to undergo a rapid 1-day release to ensure that 18 supplemental visits could be captured (beyond the standard 8 supplemental visits).

Without Advantage EDC and the Emmes support team, it's unlikely the sponsor could have met its aggressive timeline for responding to the FDA request. Emmes continues to work closely with them throughout the ongoing FDA review of study data, waiting for the day when this treatment will be available to patients around the world.