Cell Therapy Case Study

Emmes Supports Cell Therapy Development Program across Phases: Dynamic Processes and Staff Stability Enhance Execution and Advance Study

When an emerging biotech company was ready to begin its pivotal trial for a breakthrough cell therapy, it turned to Emmes for expert support. The Sponsor had begun the collaboration with Emmes 13 years earlier with its first-in-human (FIH) study for the treatment. The full scope hematopoietic cell transplantation (HSCT) clinical trial had the potential to be lifesaving in this rare disease area, and, therefore, it was critical that every aspect from project management and site management to data management and medical monitoring be carried out reliably to meet the study timelines and speed the treatment to patients.

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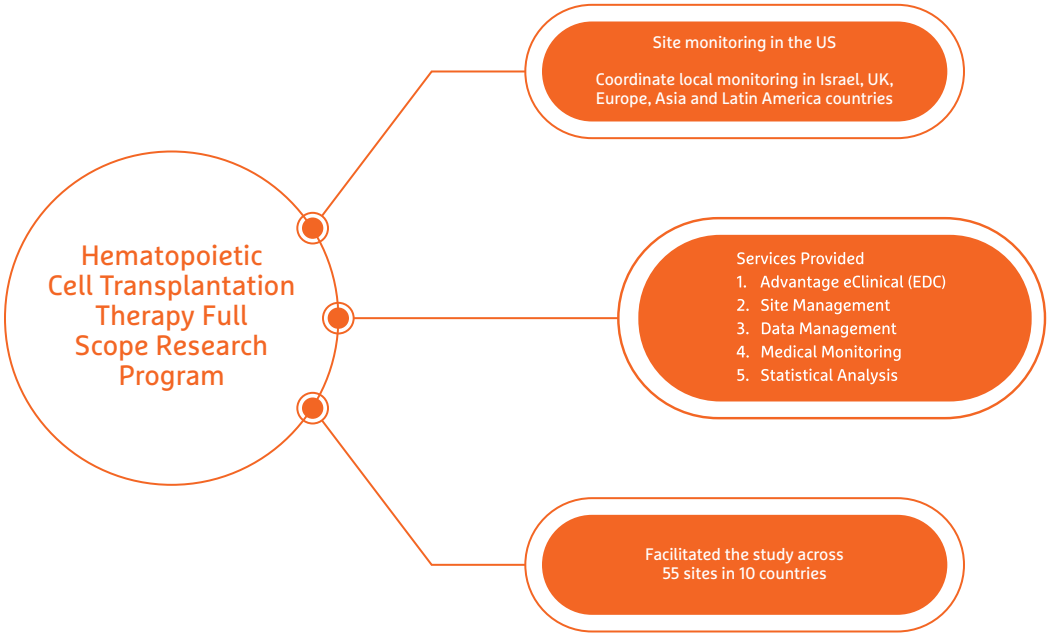
The Challenge: Accelerated Timelines and a Small Patient Population

Having worked with Emmes on the earlier phases of HSCT research, the biotech company knew it could rely on the same skilled staff to oversee the implementation of the phase III study which was to evaluate the therapy's safety and efficacy compared to the standard of care.



The open-label, randomized trial posed three significant, interrelated challenges, two of which were foreseen at the start.

1. The timelines for every interim step were ambitious, calling for efficiency and experience in how tasks were executed.

2. Given the rarity of the disease being treated, the available patient population was very small, posing statistical challenges in ensuring balance across the treatment arms.

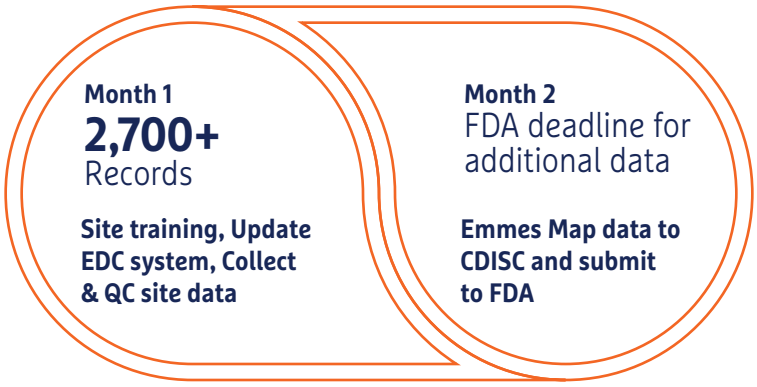
3. A year after the database was locked, the regulatory expectations had changed, and the US Food and Drug Administration (FDA) requested that additional data be collected and that it be mapped to CDISC and resubmitted. The response was required in less than 2 months – a tight timeline that nonetheless needed to be met to keep the agency's review on schedule.

The Emmes Solution: Consistent Advice, Oversight, and Execution

Throughout phases I, II and III of the research, the Emmes' Cell & Gene Therapy (CGT) Center collaborated with the Sponsor on what data needed to be collected, how it could be collected, and how it should be analyzed. To jump-start the process and with an eye to meeting the study's timelines with quality data, we:

- Were able to offer a set of data collection forms specific to the requirement of cell therapy as a starting point, owing to our decades of experience in HSCT
- Addressed the statistical challenge of the small sample size by implementing a minimization randomization scheme to ensure the validity of the study results
- Analyzed the trial data using statistical methodologies specifically developed for the HSCT research program
- Saved time and resources in training site staff as we had a relationship with the US-based sites, and they were familiar with the our Advantage eClinical EDC software
- Developed and maintained collaborative working relationships with our international research partners to ensure seamless cooperation and delivery consistency across countries

Such efficiency was possible not only because Emmes' CGT Center supported the Sponsor throughout the development program, but also because the continuity of knowledge was retained through all phases of this study for over a decade.



FDA requested additional data a year after the database lock to meet new regulations. This required unlocking the database, site training and coordinating with clinical centers to collect, QC and enter over 2,700 records, and relocking the database. Emmes accomplished this in under a month – a remarkable feat and one that allowed the agency's review to proceed without delay.

The Results: License Application Submitted on Schedule

The Emmes CGT team met all of the interim deadlines throughout phases I, II and III of the study - from site activation and patient enrollment through site monitoring and data review. The database was locked and the top line data report delivered within 10 days of the last patient's last visit (LPLV).

The Sponsor was able to file its Biologics License Application with the FDA as scheduled upon our delivery of the raw study data, the analyzed data with accompany tables and figures, the clinical study report, and integrated summaries of safety and efficacy from the Emmes study team.

Key:

- Years
- Protocols

> "We've received numerous information requests from the FDA regarding clinical datasets. For each, Emmes has worked with us to develop an approach and executed each analysis with high quality and within aggressive timelines. Emmes' work has been key to addressing the FDA's questions."
>
> **The Sponsor**

The results of the phase III trial were remarkably similar to those of earlier phases, suggesting that the studies were well-designed and executed across the board, owing in part to the consistency of staff and quality implementation and oversight by Emmes. Taken together, Emmes' therapeutic expertise, site knowledge, flexible solutions, and collaborative approach were instrumental in the Sponsor's ability to complete the trial successfully according to its timeline.

For additional information on Clinical Trial Management, please visit:
www.emmes.com/cell-and-gene-therapy