Premier Insight 272

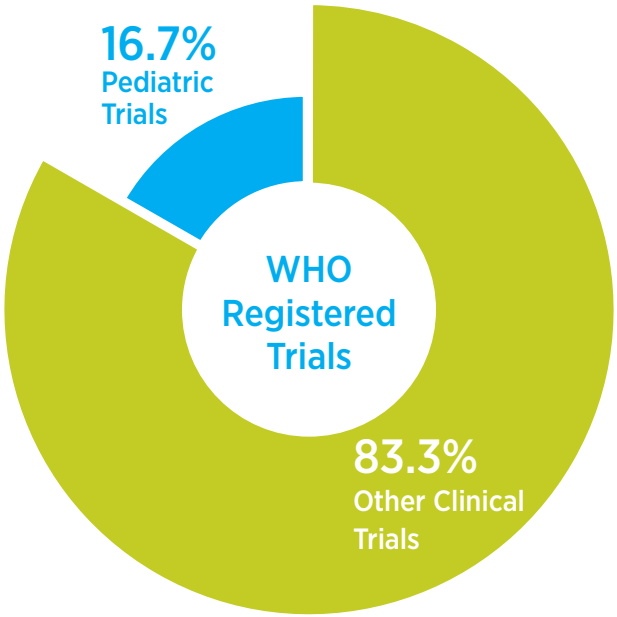
Delivering the Global Expertise Required to Coordinate a Pediatric Gene Therapy Trial

Introduction/Background

With their potential for long-term or even curative efficacy, gene therapies are of great interest to researchers, clinicians, patients and caregivers alike. But coordinating and conducting a global, multi-center gene therapy trial is a complex, high-risk undertaking.

Beyond the usual protocols and procedures required to ensure patient safety and data quality, gene therapy studies must also meet specific requirements surrounding the use of a genetically modified organism (GMO) as an investigational medicinal product (IMP).

A European biotech company approached Premier Research with a pediatric trial involving a rare, incurable genetic disorder. With only **16.7 percent of World Health Organization-registered trials classified as pediatric**, children are significantly underrepresented in clinical studies. Recent regulations such as the Research to Accelerate Cures and Equity (RACE) Act are designed to promote research and development of therapies for children.



Objective

In addition to new regulatory considerations, this trial also called for an extremely challenging intracerebral administration. To succeed, Premier needed to develop a clear, compliant strategy for obtaining informed consent and implementing an innovative data management plan to track patients between surgical and clinical sites.

PEDIATRIC RARE DISEASE

Meeting the Challenge: Executing a pediatric gene therapy trial

Challenge #1: Enhancing the Participant Experience

Along with great interest in the study came questions from families about the enrollment process and the scope of the study requirements (both during surgery and the extensive follow-up process).

Our Strategy

Knowing that a positive experience for patients and their families is critical to the success of a study of this complexity, we developed materials to support family discussions, including an informed consent flip chart. We also managed high-touch travel support to facilitate participation and initiated a bi-annual newsletter to foster long-term engagement.

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Challenge #2: Tracking patient data across sites

Patients were set up in the database at their respective clinical sites, and the gene therapy was administered by a surgeon under general anesthesia at one of multiple different surgical sites. This allowed long-term follow-up to take place at separate clinical sites, but created a challenge when it came to tracking patient data across sites.

Our Strategy

We developed a database access scheme that allowed for each patient to be associated with a consistent patient number throughout the study, minimizing potential issues associated with vendor database set-up. This also eliminated the need to transfer the patients' data from one site to another in the database, creating greater data management efficiencies.

Challenge #3: Navigating use of a GMO as IMP

Specific requirements around the use of a GMO as an investigational product had to be met, both for the start-up process and for the IMP and patient management at the site in case of viral shedding.

Our Strategy

Premier engaged early with the surgical sites to ensure their GMO SOPs were in compliance with study procedures. Where SOPs were not yet adequately developed, we leveraged our specialized regulatory expertise to provide support to sites.

Takeaway

When planning a gene therapy trial – especially one involving children – the details matter. Partnering with a team that has the expertise to guide your study, from planning to patient retention, can make the difference.

Successful Site Initiation and Gene Therapy Administration

Study Description
Phase 2/3 study of gene therapy in the treatment of children with a rare and currently incurable genetic disorder

Therapeutic Area
Pediatric rare disease

Geographic Scope
U.S. and Europe

Patient Population
20 patients at eight sites

Enrollment Period
February 2019 – March 2020

Outcome
10 patients efficiently enrolled in US and European sites

10 successful surgeries were performed

10 pediatric patients are currently doing well

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