Rare Disease Experience: Inherited Metabolic Diseases

Cmed is currently working on a Phase II trial with a US pharmaceutical company involving pediatric patients with selected inherited metabolic disorders undergoing stem cell transplantation. The study aims to magnify the necessary cell population within a UCB to quicken the time to reach transplant effectiveness and ultimately aims to positively impact the transplant success and survival rate.

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Inherited Metabolic Diseases

Inherited metabolic diseases (IMD), including Hurler syndrome, cerebral adrenoleukodystrophy (cALD), globoid cell leukodystrophy (GLD or Krabbe) and metachromatic leukodystrophy (MLD), are a group of rare, inherited disorders that result in a progressive loss of neuromotor and cognitive abilities. Untreated, these disorders are progressive and lethal.

Enzyme replacement of lysosomal disorders cannot correct the central nervous system defects due to inability to pass through the blood-brain-barrier.

Current Therapeutic Solutions

The only accepted therapy for Hurler syndrome, MLD, GLD, and cALD is allogeneic hematopoietic stem cell transplantation (HSCT), after which immature cells of hematopoietic origin migrate to tissues, including the central nervous system.

Challenges for Patients

While HSCT is considered the standard of care for these diseases, it also has a high risk of complications and death. Deaths from transplant-related complications occur in 20% to 30% patients. Most of the deaths occur within the first 6 months of transplant.

Unrelated Umbilical Cord Blood (UCB) donor programs are most often utilized because most of these children do not have a genetically identical bone marrow donor. The amount of necessary cells (stem and progenitor) within a UCB unit is infinitely small and therefore takes a very long time to be effective for the patient, a primary factor of the deaths observed.

Cmed Solutions

Enrollment

Changes have been made to the protocol to extend the inclusion criteria; motivational visits to site for support have been conducted; additional sites have been activated; the Sponsor website has been updated to support parents researching the study

IMP Supply

Manufacturing complexities have been managed plus transparent and timely communication with the site has enabled the process to run as smoothly as possible.

Recurring IDMC Meetings

Scheduled and ad hoc IDMCs have been supported by Cmed's encapsia EDC, enabling committee members to assess real-time data

Medical Monitoring

Medical monitors are medical science experts who answer any questions the clinical team and sites may have, review the data in encapsia in real-time, and respond to any safety findings that require the sponsor to convene an ad hoc IDMC meeting

Cmed has worked on over 100 clinical trials for rare diseases. Let's talk about how our experts can help with your next trial!

info@cmedresearch.com Europe: +44 (0) 1403 755 050 USA:+1 919 595 6900