New Clinical Trial Grants for Rare Diseases

A painful, potentially life-threatening skin disease, an inherited disease that can cause long-term brain development issues, and pediatric brain cancer are among the rare diseases for which the US Food and Drug Administration has awarded 11 new clinical trial research grants.

The grants, totaling more than $25 million over the next 4 years, support clinical trials of products that address unmet needs in rare diseases or significantly improve their treatment or diagnosis. The funds also support a demonstration project to evaluate a tool that could improve data accuracy for multisite clinical trials.

Many of the studies involve children, some as young as newborns. One trial is evaluating the treatment of recessive dystrophic epidermolysis bullosa, the inherited skin disorder that can lead to painful, life-threatening blisters and wounds. Another study is evaluating early treatment of tuberous sclerosis complex before the onset of seizures in infants. And a third will test a novel peptide vaccine to treat pediatric brain cancers. Other grant recipients will focus on treatments for such conditions as Becker muscular dystrophy, short bowel syndrome, and multiple system atrophy.

First Thymus Tissue Product Approved for Rare Disease

The FDA recently approved the first thymus tissue product as a 1-time treatment of congenital athymia, an ultrarare immune disorder in which children are born without a thymus.

Children with congenital athymia lack adequate working T cells and typically die before their second birthday after experiencing repeated infections.

The disorder is initially detected by T-cell deficiency observed in newborn screening for severe combined immune deficiency (SCID), which is now required in all 50 US states and the District of Columbia. Congenital athymia’s US incidence is estimated to be 17 to 24 live births per year, according to Enzyvant, the company that developed the new treatment.

Allogeneic processed thymus tissue-agdc (marketed as Rethymic) comprises donor-derived human thymus tissue that is processed and cultured and then surgically implanted into patients with congenital athymia to help improve their immune function. It is not indicated for treating SCID.

The treatment’s safety and efficacy were established in clinical trials involving 105 patients, aged 1 month to 16 years, from 1993 to 2020. Of these patients, 29 died after treatment, including 23 within a year, according to Enzyvant and the FDA. But most treated children survived at least 2 years, and those who survived past the first year generally survived long-term. What’s more, their frequency and severity of infections also declined over time.

Adverse reactions included high blood pressure, cytokine release syndrome, rash, low platelets, and graft-vs-host disease. The FDA noted that because the product is derived from human tissue, it could potentially transmit infectious disease. Effective donor screening and manufacturing processes greatly reduce this risk but don’t eliminate it.

Reconstituting the immune system takes at least 6 months, during which time treated patients must continue strict precautions to prevent infections, and clinicians should treat accordingly, the FDA said. — Rita Rubin, MA

Note: Source references are available through embedded hyperlinks in the article text online.