# Why life-saving gene therapy isn’t available yet to children who need it most

[The first patient to permanently edit his DNA,] Brian Madeux, 44, of Arizona, is part of a clinical trial testing a gene-editing approach for Hunter syndrome, a type of metabolic disorder that slowly destroys the body’s cells. The life expectancy of people born with the disease is 10 to 20 years, so most patients are children. For now, the trial Madeux is enrolled in is only open to adults. So young patients who desperately need the therapy to survive will need to wait to get it.
The U.S. Food and Drug Administration often wants companies to show that a treatment is safe in adults before it can be used in children. Sangamo Therapeutics, the company conducting the trial, is aiming to treat nine adult patients before it can admit children into the current trial.

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The Sangamo therapy uses a gene-editing technology called zinc finger nucleases rather than the newer CRISPR. It’s designed to insert a correct copy of the IDS gene into liver cells. Sangamo thinks this should enable the liver to produce a lifelong and stable supply of the enzyme the patient lacks.

[Hunter Syndrome Foundation founder Jeanette] Henriquez says that despite her optimism, she’d be reluctant to enroll her son in the trial if it became open to children, because gene editing comes with risks.
**The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion, and analysis. Read full, original post:** A New Gene-Editing Therapy Would Benefit Kids Most – Here’s Why They Won’t Get It Yet