Hannah's year: Gene transfer treatment gives hope to curing rare neurodegenerative disease

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion and analysis.

Eleven-year-old Hannah Sames can still curl her toes, just barely. But time is running out.

If Hannah can move her toes for a few more weeks, until she becomes the fourth child in a clinical trial for gene transfer to treat giant axonal neuropathy (GAN), the disease might halt – and she may even regain function, as mice did.

It's been an eight-year wait. So *Facebook* friends call 2016 "Hannah's year."

The first sign that something was amiss in the motor neurons that extend from Hannah's spinal cord down towards her toes was an odd, hesitant gait as a toddler. A "diagnostic odyssey" finally told Lori and Matt Sames in 2008 that their little girl had an untreatable, ultra-rare condition that's fatal within two decades. The devastated parents immediately formed Hannah's Hope Fund, which has nearly entirely funded the research behind the trial at the NIH Clinical Center. Steven Gray, PhD, from the University of North Carolina and his colleagues, developed the protocol.

DNA Science has chronicled Hannah's journey and the start of the clinical trial nearly a year ago. CBS National News covered the story in October.

The GAN gene transfer is the first into the spinal cord, and so it could pave the way for treatment for other conditions, such as amyotrophic lateral sclerosis. The gene behind GAN encodes a protein, gigaxonin, that aligns the girder-like intermediate filaments that fill certain cells.

Read full, original post: Hannah's 2016: From Curling Toes to Gene Therapy