After 8 years and $8 million, gene therapy begins for 12-year-old with rare neurological disorder

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

[July 21, 2016], 8 years and $8 million fund-raised dollars after the Sames family of Rexford, NY, began their battle against giant axonal neuropathy (GAN), their daughter Hannah is finally receiving gene therapy.
About 120 trillion viruses are being injected into the fluid surrounding 12-year-old Hannah's spinal cord, at the NIH Clinical Center. Each virus carries a working copy of the gene that encodes a protein called gigaxonin.

Although Hannah is the fifth child in the clinical trial, she's the first whose body doesn't make the protein at all, thanks to two deletion mutations. She required a separate protocol to suppress her immune system so that it would accept the treatment, which uses the harmless adeno-associated virus as the vector to deliver the genes. Her parents Lori and Matt had found themselves in the unimaginable situation of having funded a clinical trial that might have to exclude their own child.

DNA Science has chronicled the astounding efforts of Hannah's family to treat this ultrarare disease, which is like ALS in children.

Read full, original post: Hannah Has Her Gene Therapy for GAN: When Science Becomes Medicine