Gene therapy improves vision in Canavan disease patient even after 15 years

The first thing that Max Randell's parents noticed after gene therapy for Canavan disease in 1998, when he was nearing his first birthday, was better vision.

…

Max had an appointment with his ophthalmologist [recently] and his vision continues to improve. His doctor is very interested in gene therapy and she said that the gene is still active in his brain because his optic nerve shows absolutely no signs of degeneration and looks the same each year.

…

Canavan gene therapy circa 2002 targeted less than 1% of brain cells with fewer viral vectors than is attempted in clinical trials today. But it looks like some of the vectors made their way to Max's optic nerves, and clearly elsewhere judging by his interest in math and critical thinking.

…

Fewer than 1,000 people in the US are known to have Canavan disease, which strips the insulating myelin from brain neurons, due to deficiency of an enzyme (aspartoacylase, or ASPA). The white matter becomes a spongy mass of fluid-filled bubbles. Gene therapy provides the ASPA gene.

The GLP aggregated and excerpted this blog/article to reflect the diversity of news, opinion and analysis. Read full, original post: Vision Improving 15 Years After Gene Therapy for Canavan Disease