

The Washington Post article "Scientists are inventing treatments for devastating diseases. There's just one problem," written by Carolyn Y. Johnson and published on January 11, 2026, covers the recent push to bring personalized gene therapies to patients with rare genetic diseases. The article highlights a few major cases from 2025, most notably KJ Muldoon, a baby who became the first person in the world to receive personalized CRISPR gene therapy for a rare metabolic disorder called CPS1 deficiency. The article also describes a biotech company's use of a newer gene-editing tool to treat a severe immune disorder. While the science has clearly made impressive progress, Johnson points out that access and cost remain major hurdles; these treatments are expensive to develop and difficult to scale, especially for diseases that affect only a handful of patients worldwide.

This article relates to genetics because the diseases being discussed are caused by mutations in single genes. CPS1 deficiency, for example, results from a mutation in the CPS1 gene, which encodes an enzyme involved in the urea cycle. When this gene doesn't work, toxic ammonia builds up in the blood. CRISPR technology works by using a guide RNA to direct the Cas9 protein to a specific location in the genome, where it makes a cut and allows for a correction to be made. The idea is to fix the broken gene directly rather than just treating symptoms, which is a fundamentally genetic approach to medicine.

A 2025 review article by Cetin et al. published in *Expert Reviews in Molecular Medicine* supports the general claims made in the Washington Post piece. The review confirms that CRISPR-based therapies have moved well beyond the lab and into clinical trials, with the FDA approval of Casgevy for sickle cell disease serving as the clearest proof of concept. The review also validates the article's point about ongoing challenges: off-target edits, delivery of the gene-editing machinery into the right cells, and long-term safety are all still active problems. So while the Washington Post article is mostly accurate and does a reasonable job explaining the promise of gene therapy, it leans a little optimistic and doesn't spend much time on the real scientific obstacles that still exist. Overall the article is credible and the science checks out.

## References

Johnson, C. Y. (2026, January 11). Scientists are inventing treatments for devastating diseases. There's just one problem. *The Washington Post*.

<https://www.washingtonpost.com/health/2026/01/11/gene-therapy-rare-diseases/>

Cetin, B., Erendor, F., Eksi, Y. E., Sanlioglu, A. D., & Sanlioglu, S. (2025). Advancing CRISPR genome editing into gene therapy clinical trials: progress and future prospects. *Expert Reviews in Molecular Medicine*, 27, e16. <https://doi.org/10.1017/erm.2025.10>