Viewpoint: GlaxoSmithKline abandonment of rare disease gene-therapy drug program because it's not a money generator hurts children

When GlaxoSmithKline, long a global leader in the effort to pioneer gene replacement therapies, announced it would halt its drug development program for rare diseases, I understood that the decision made practical sense for the company. But as the mother of a child with a rare disease — one for which GSK was developing a highly effective treatment — I was devastated. My daughter, Cal, was diagnosed with metachromatic leukodystrophy (MLD) at age 2.

…

Experts in the field called the results from GSK's clinical trials of gene therapy for MLD "stunning." Children who should have been unable to talk or walk, who needed feeding tubes and hospice care, were instead attending school and riding bicycles and living remarkably normal lives.

…

[CEO Emma] Walmsley wants the company to focus on "real winners" — medicines that generate substantial returns. She's correct in her assessment that GSK would have had trouble making money on gene therapy for MLD.

…

I wish that Walmsley had the chance to meet children with MLD whose lives were saved and forever changed by the gene therapy her company developed. Had that happened, I suspect she would have felt differently about research she did not believe was a "winner." She would have realized that GSK had something that was far better: a miracle.

Read full, original post: Rare disease treatment may not be a 'real winner.' But it's definitely a miracle maker