Teenager’s experimental gene therapy treatment could change the lives of millions of sickle cell patients worldwide

Meet Helen Obando, a shy 16-year-old who likes to dance when her body isn’t ravaged by the debilitating symptoms of sickle cell disease. The genetic blood disorder can cause strokes, organ damage and intense pain.

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After a lifetime of pain and potential permanent damage to her body, Helen had the opportunity to receive a breakthrough experimental treatment at Boston Children’s Hospital that would make her the youngest person in the U.S. to have her DNA reset in an attempt to cure her sickle cell disease.

The outcome of her gene therapy could help determine how an estimated 100,000 people in the U.S. and millions more around the world are treated. Sickle cell disease most commonly affects people from sub-Saharan Africa, and about 1 in 500 African-Americans have the blood disorder, the most commonly inherited blood disorder in the U.S. But some people with the disease have southern European, Middle Eastern or Asian backgrounds or, like Helen, are Hispanic. For decades, attention and money for research have not matched the scale of the problem.

Why has it taken so long for the scientific research community to push ahead with promising therapies for sickle cell?

Read full, original post: A Teenager’s Breakthrough Gene Therapy for Sickle Cell Disease