Podcast: Has sickle cell disease met its match in CRISPR gene editing?

Sickle cell disease (SCD) is an inherited condition that predominantly affects people of African descent. The disease results in chronic pain and early death and is caused by a misfolding of oxygen-toting hemoglobin, a central protein in red blood cells. Declining mortality rates in young children show that progress has been made against SCD, but the condition still afflicts roughly 100,000 Americans and can cost upwards of $15,000 to treat, according to the Centers for Disease Control (CDC).

While a cure has so far eluded researchers, advances in human gene-editing technology are poised to put SCD in check. Vertex Pharmaceuticals and the CRISPR Therapeutics companies have developed a therapy that allows doctors to edit stem cells to produce fetal instead of adult mutant hemoglobin. The engineered stem cells are returned to the patient, who then manufactures fetal hemoglobin in their own blood cells, potentially curing the disease. Clinical trials to evaluate the safety and efficacy of the treatment have just begun.

On this episode of Talking Biotech, Dr. Brenda Eustace, director of discovery research at Vertex, joins University of Florida plant geneticist Kevin Folta to discuss this promising sickle cell treatment that could bring needed relief to millions of people worldwide.

https://geneticliteracyproject.org/wp-content/uploads/2019/08/199-Vertex.mp3

Brenda Eustace is the Director and Head of Discovery Biology at Vertex Pharmaceuticals. Follow Vertex on Twitter @VertexPharma

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