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Exploring equitable treatment options for pediatric patients with sickle cell disease

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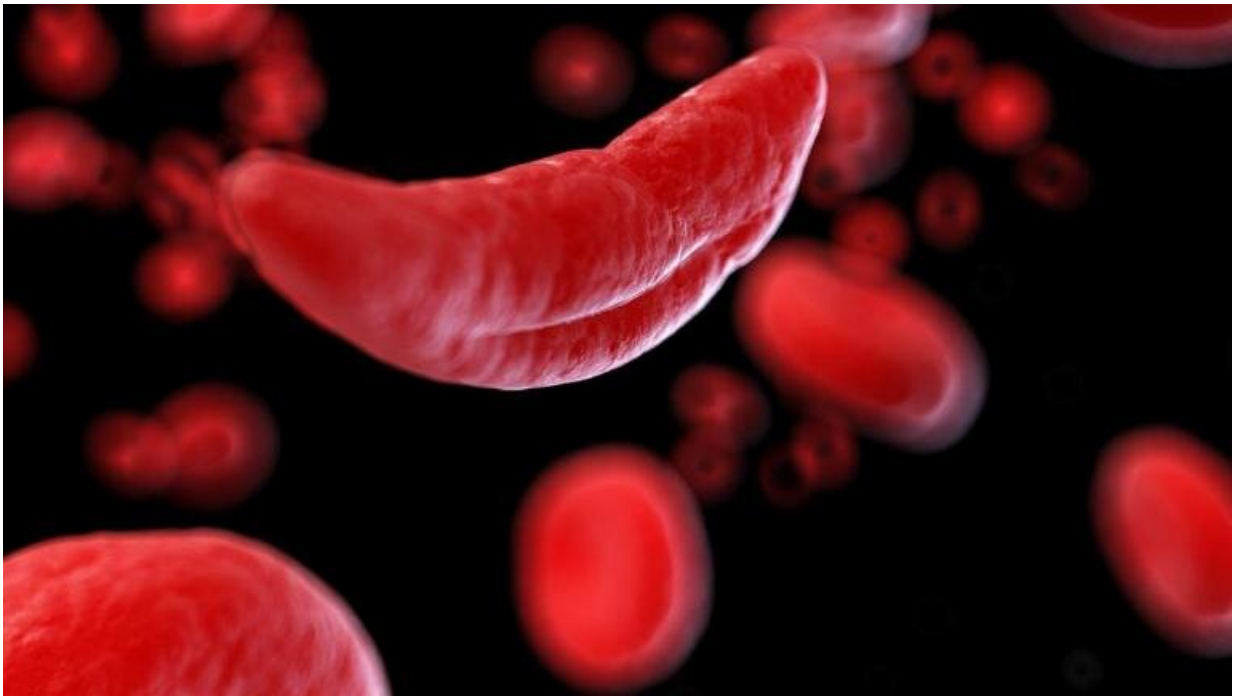

Credit: National Institutes of Health

Patients with sickle cell disease (SCD) often face a reduced quality of life and a lower life expectancy. Allotransplantation, the first treatment for SCD with curative potential, comes with risks, including transplant-related mortality. Gene therapy, once approved for SCD, could also offer a lifelong cure without the risk associated with allotransplantation.

A new study led by Yale Cancer Center researchers at Yale School of Medicine for the first time analyzed the cost-effectiveness of allotransplantation therapy and gene therapy to help determine equitable treatment options for this historically marginalized pediatric patient population in the United States. First author George Goshua, MD, a member of Yale Cancer Center, will present the findings at the American of Society for Hematology (ASH) Annual Meeting on December 10.

The study used an incremental cost-effectiveness ratio (ICER) to evaluate cost-effectiveness of each therapy. Allotransplantation had an ICER of $138,000 per quality-adjusted life-year (QALY), while gene therapy had an ICER of $253,000.

"While these therapeutic strategies appear to be expensive, and they are, pursuing allotransplantation or gene therapy both meet distributional equity standards in the United States," said Goshua, who is an assistant professor of medicine (Hematology) at Yale School of Medicine.

Researchers emphasize the importance of considering equity in decision-making for pediatric SCD treatment. "We hope to continue to provide more equity and value-informed transparent benchmarks for stakeholders making crucial funding decisions, and also that other groups join us in doing so," said Goshua.

More information: George Goshua et al, Allotransplantation and Gene Therapy Equity for Children with Sickle Cell Disease: Distributional Cost-Effectiveness of Allotransplantation Vs Gene Therapy Vs Standard-of-Care in Pediatric Patients with Sickle Cell Disease in the United States. ash.confex.com/ash/2023/webpro … ram/Paper191072.html