Docket #: S21-175

Potential Curative Treatment for Alpha-Thalassemia Using CRISPR-Mediated Genome Editing

Researchers at Stanford have developed a potentially curative treatment strategy for alpha-thalassemia, one of the most common autosomal recessive disorders in the world involving the genes *HBA1* and/or *HBA2*. The team used CRISPR-mediated genome editing to integrate a full-length alpha globin transgene at the start site of the beta globin locus in hematopoietic stem and progenitor cells (HSPCs). This work represents a **novel, safe and effective** approach for introducing *HBA1* or *HBA2* transgenes into autologous HSPCs and red blood cells in vivo or ex vivo, **increasing the amount** of alpha globin in red blood cells and **improving the balance** between alpha and beta globin. Currently, the only curative strategy for alpha-thalassemia is donor-derived hematopoietic stem cell transplantation. However, in the majority of cases no matched donor is available and even if one is identified, transplantation carries a risk of immune rejection and graft-versus-host disease. The Stanford strategy may provide a definitive cure for alpha-thalassemia by integrating into existing *ex vivo* HSPC editing/transduction workflows followed by autologous transplantation.

Stage of Development
Screening of clinical vectors in alpha-thalassemia patient-derived cells.

Applications

- Treatment strategy for alpha-thalassemia

Advantages

- New, safe and potentially transformative approach
- Integrates into existing HSPC editing/transplantation workflows

Publications

- Cromer, M.K., Camarena, J., Martin, R.M. et al. (2021). Gene replacement of α-globin with B-globin restores hemoglobin balance in B-thalassemia-derived hematopoietic stem and progenitor cells. Nat Med 27, 677–687.

Patents

- Published Application: WO2023028469
- Published Application: 20250127927

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