



## FDA Approval to Gene Therapies for Sickle Cell Disease

**Food and Drug Administration (FDA)**, an agency under U.S. Department of Health and Human Services, approved two gene therapies for [sickle cell disease](#): **Lyfgenia from bluebird bio and Casgevy by Vertex Pharmaceuticals and CRISPR Therapeutics.**

- Sickle cell disease is a genetic blood disorder characterized by **an abnormality in hemoglobin**, the protein responsible for carrying oxygen in red blood cells (RBC).
  - It causes RBC to adopt a sickle or crescent shape, hindering their movement through vessels, leading to potential complications like severe pain, infections, anaemia, and strokes.
- These therapies aim to **transform treatment by leveraging CRISPR gene editing technology** to either insert modified genes or edit stem cells, potentially offering one-time treatments.
- Concerns exist about the therapies' long-term effectiveness and risks, including the need for **high-dose chemotherapy, potential infertility**, and concerns about unintended genomic alterations.

**Read more:** [CRISPR-Cas9 for Sickle-Cell Anaemia](#)

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