

## 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 <u>sickle cell disease</u>: **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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