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