Breakthrough CRISPR treatment for sickle cell anemia up for FDA approval

On 31 October, external advisers to the US Food and Drug Administration (FDA) will meet to discuss a DNA-altering therapy for sickle-cell disease, a genetic condition that causes red blood cells to become misshapen, which can lead to debilitating pain. The advisers' discussions are likely to be laser-focused on safety data submitted by the treatment's developers, Vertex Pharmaceuticals in Boston, Massachusetts, and CRISPR Therapeutics in Zug, Switzerland.

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In particular, the FDA flagged two potential shortcomings in assays that Vertex and CRISPR Therapeutics used to determine exa-cel's risk of causing off-target changes. In one assay, researchers searched a database of genomes to find regions that are similar to exa-cel’s CRISPR–Cas9 target site and that therefore might be mistakenly cleaved by the Cas9 enzyme. The scientists then gauged the risk of changes at these sites.

Most of the sites did not raise concerns, says [pediatrician Mark] Walters. But sickle-cell disease predominantly affects people of African descent, and FDA examiners are concerned that the genetic diversity in this population was not captured in the genomes that the companies searched.

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