Viewpoint: Skeptical take on newly-approved CRISPR sickle cell treatment tool

Changing the course of debilitating genetic diseases like sickle cell is the promise held out by CRISPR, and a main reason why the technology has spawned a growing pipeline of experimental drugs. Among them, exa-cel is the furthest along.

Yet exa-cel’s benefits may not be accessible by all, or even most, of the tens of thousands of people estimated to have severe sickle cell in the U.S., never mind the millions more in other areas of the world where the disease is more prevalent.

The therapy is bespoke, created from an individual’s own stem cells via a laborious and expensive process. There are side effects, particularly during a preparatory chemotherapy regimen before exa-cel is infused. That regimen is so arduous that older people and those with organs damaged by sickle cell may not be healthy enough to receive it. The chemotherapy drugs can cause infertility, forcing a difficult decision on people who hope to have children.

Exa-cel also comes with the theoretical risk that wayward gene edits in the transplanted cells may one day lead to other problems, like cancer. Last week, a group of advisers convened by the FDA debated this possibility, but judged the risk to be small and exa-cel to be safe.

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