

FDA advisors say new gene therapy for sickle cell disease is safe

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A new gene therapy for sickle cell disease has been deemed safe by a

U.S. Food and Drug Administration advisory panel, paving the way for full approval by early December 2023.

The FDA had already decided that the therapy, known as exagamglogene autotemcel (exa-cel), was effective. Developed by Vertex Pharmaceuticals of Boston and CRISPR Therapeutics of Switzerland, exa-cel frees patients from the excruciating symptoms of [sickle cell disease](#). If approved by Dec. 8, as expected, it would become the first medicine to treat a genetic disease with the CRISPR gene-editing technique, CRISPR Therapeutics said in a [news release](#).

But it will not be the only new treatment for the inherited condition coming down the pike. By Dec. 20, the FDA will also decide on a second potential cure for the disease that typically strikes Black people, a [gene therapy](#) crafted by Bluebird Bio, of Somerville, Massachusetts.

"We are finally at a spot where we can envision broadly available cures for sickle cell disease," said John Tisdale, M.D., director of the cellular and [molecular therapeutics](#) branch at the National Heart, Lung and Blood Institute and a member of the advisory committee, *The New York Times* reported.

In [briefing documents](#) filed with the advisory committee before the meeting, Vertex said that 46 people got the treatment in its study. Among the 30 who had 18 months of follow-up, 29 were free of pain crises for at least a year and all 30 avoided being hospitalized for pain crises.

Vertex has said it plans to follow clinical trial patients for 15 years, and the advisory committee members said they saw no reason to delay approval of the treatment.

There can always be additional studies, noted committee member Alexis

Komor, Ph.D., a professor of chemistry and biochemistry at the University of California in San Diego, *The Times* reported. But that would be "expecting perfection at the expense of progress," she said.

More information: [The New York Times](#) article.

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