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DIVE BRIEF

FDA won't hold advisory meeting for Bluebird's sickle cell gene therapy

The decision stands in contrast to the agency's plans for a would-be rival gene treatment from Vertex Pharmaceuticals and CRISPR Therapeutics.

Published Aug. 16, 2023

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Dive Brief:

- The Food and Drug Administration opted not to ask for the advice of outside experts on Bluebird Bio's latest gene therapy, a treatment called lovo-cel designed for sickle cell disease patients.
- The decision puts Bluebird on a different regulatory path than rival Vertex Pharmaceuticals, which developed a competing candidate called exa-cel with partner CRISPR Therapeutics. Vertex told investors earlier this month that the FDA plans to hold an advisory committee meeting to review its option.
- Bluebird's announcement Wednesday doesn't change the overall timeline for the experimental treatment. The company won a priority review, with the FDA promising an answer on the application for lovo-cel by Dec. 20. Vertex and CRISPR also got a priority review and expect their answer by Dec. 8.

Dive Insight:

The FDA’s decision to skip an advisory panel may indicate that agency reviewers are more unified in their views of the Bluebird therapy. Or it could simply indicate a higher comfort level with the type of treatment Bluebird is developing; FDA committees unanimously backed two Bluebird gene therapies that are delivered in a similar way in June 2022, and both went on to win approval.

By contrast, Vertex and its partner are vying to market the first therapy based on CRISPR gene editing technology. Vertex CEO Reshma Kewalramani told investors on an Aug. 1 call that the decision to hold an advisory panel wasn’t surprising, “given the new mechanism of action.” The meeting will offer an opportunity to hear from patients and highlight the “transformative potential” of exa-cel, she said.

In addition to the comfort level the FDA has after reviewing previous Bluebird applications, the agency may also be more familiar with the latest data because of a protracted filing process for lovo-cel, Stifel analyst Benjamin Burnett said in a note to clients. Even though “regulatory situations are inherently opaque,” Burnett said he still expects Vertex and CRISPR’s option to ultimately win FDA approval.

About 100,000 people in the U.S. have sickle cell disease, a progressive and debilitating disease that significantly shortens the lifespan of its victims. There are currently no therapies that address the underlying genetic cause of the condition.