BIOPHARMADIVE

DIVE BRIEF

# BioMarin delays planned FDA filing for hemophilia gene therapy

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## Dive Brief:

- BioMarin Pharmaceutical has delayed plans to resubmit an approval application to the Food and Drug Administration for its experimental hemophilia gene therapy, revealing Tuesday that the agency requested additional information be included in its filing.
- BioMarin now expects to refile its application by the end of September, rather than by June as it had previously indicated. The biotech company did not specify what information the FDA is seeking, but noted that the regulator did not ask for further preclinical or clinical testing.
- BioMarin's therapy, known as Roctavian or valoctocogene roxaparvovec, was unexpectedly rejected by the FDA in August 2020. At the time, the company said the FDA sought two-year follow-up data on clinical trial participants, data it has since obtained successfully.

## Dive Insight:

BioMarin's road back to the FDA keeps getting longer, with the agency's request adding new uncertainty to a resubmission that's

been in the works for nearly two years.

The California biotech disclosed the FDA's feedback alongside updated five- and six-year results from an early study of Roctavian, which, if approved, would be the first gene therapy for hemophilia A, the most common form of the inherited bleeding disorder.

Trial participants remained off standard hemophilia treatments and, on average, were experiencing less than one bleeding episode per year — a reduction of more than 90% from prior to treatment.

Two-year results from a much larger Phase 3 trial showed a similarly powerful benefit, although the data from that study indicated that levels of the clotting protein produced by Roctavian declined over time. The waning of protein levels has led to questions over how long the benefits of gene therapy might actually last.

BioMarin executives have said they believe treatment will deliver at least five years of bleeding control, and potentially more.

The Phase 3 trial results will be included in BioMarin's resubmission, but the FDA also asked for "additional information and analyses of data to be included," BioMarin said. Additionally, the company noted that three-year results from the study would likely become available during the FDA's review if the agency accepts the refiled application as expected. It's not clear whether the FDA would eventually request to see those data as well.

"In our view, it's hard to really know if this changes the probability-of-success much either way, as it appears that after recent discussions with FDA, [BioMarin] has garnered more clarity on specific data analyses that the agency is looking for, and these will take a few additional months to compile," wrote Paul Matteis, an analyst at Stifel, in a note to clients.

Shares in BioMarin declined by as much as 5.7% on Tuesday morning on news of the delay.

Yet while Roctavian's possible arrival to the U.S. market has been delayed, in Europe the drug is currently under regulatory review, with an opinion from the European Medicines Agency expected by "mid-year." The EMA has moved more quickly than the FDA to approve several gene therapies, including two developed by Bluebird bio that are currently being evaluated in the U.S.