BIOPHARMADIVE

BioMarin finally secures FDA approval of hemophilia gene therapy

After a prolonged journey, the medicine, known as Roctavian, is now cleared for certain patients with hemophilia A, the more common form of the rare bleeding disorder.

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An illustration of red blood cells. *Brillianata via Getty Images*

The Food and Drug Administration approved on Thursday the first gene therapy for the most common form of hemophilia, clearing the way for what patients, doctors and the medicine's developer hope could be a one-time treatment for the rare bleeding disorder.

BioMarin Pharmaceutical, the California-based company behind the therapy, plans to sell it under the brand name Roctavian. It's specifically meant to treat hemophilia A, which is caused by genetic mutations that inhibit the production of a key blood-clotting protein known as Factor VIII.

The company has set a list price of $2.9 million in the U.S., higher than the therapy's cost in Europe, where it's priced at about €1.5 million and has been approved since last August.

The FDA's decision concludes a prolonged journey to the U.S. market for Roctavian. BioMarin originally filed an approval application at the end of 2019, supported by evidence from a large

clinical trial that showed its therapy sharply reduced bleeding rates and the need for Factor VIII infusions in patients with severe hemophilia A.

In spite of these results, the agency unexpectedly rejected BioMarin's application and asked the company to collect at least two years' worth of data on each participant in that trial. Notably, the safety and long-term effectiveness of gene therapies have been a focal point for the FDA and other drug regulators. There's also been signs that the effects of BioMarin's therapy may wane with time.

BioMarin met this request, but late last year said the agency now also wanted to see three-year data. The company submitted those results shortly after, but, in order to allow enough time for a proper review, the FDA pushed back its review deadline to June 30.

With approval now in hand, BioMarin's focus will turn to ensuring a successful launch of its therapy. Though the company has seven other marketed drugs, it hasn't been profitable for most of its 26 years in operation. But analysts believe Roctavian could be the tipping point. Joseph Schwartz of SVB Securities has forecasted around $2.2 billion in peak annual sales.

BioMarin and other gene therapy developers have argued that, dosed just once, these treatments could be more cost-effective than regular Factor VIII replacements or newer, longer-acting medicines. Roche's hemophilia A drug Hemlibra, for example, carries a list price of about half a million dollars, and has gained popularity because it can be administered as infrequently as once every four weeks.

BioMarin's therapy won't be available to all hemophilia A patients in the U.S., however. The FDA approved it only for patients with severe disease who test negative for a type of antibody that attacks

the virus Roctavian uses to deliver its helpful genetic material into cells.

Previously, BioMarin estimated that about 20% of patients in the U.S and 30% globally would be ineligible for Roctavian because of these antibodies.

The FDA's nod for Roctavian comes less than a year after the agency approved Hemgenix, the first gene therapy for the less common "B" form of hemophilia. It was developed by the Dutch biotech UniQure and is sold in the U.S. by CSL Behring, at a list price of $3.5 million.

Editor's note: This story has been updated with Roctavian's list price, which was disclosed on a conference call Thursday afternoon.