BRIEF

FDA approval sets stage for a showdown between Alexion and Apellis

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Dive Brief:

- The Food and Drug Administration on Friday granted Apellis Pharmaceuticals approval to start selling a medicine that, for the first time, will create direct competition for Alexion Pharmaceuticals' biggest products.
- The Apellis medicine, Empaveli, is meant to treat a rare, life-threatening blood disease known as paroxysmal nocturnal hemoglobinuria, or PNH. Empaveli is now approved for adult patients who have never received treatment, as well as those switching off of Alexion's two marketed PNH therapies, Soliris and Ultomiris.
- Apellis set Empaveli's initial price at $458,000, which the company says is on par with Ultomiris and lower than the price tag on Soliris. Alexion recorded just over $5.1 billion in net sales from those two drugs last year, with Soliris accounting for about 80% of the total.

Dive Insight:

Alexion stands as one of the largest developers of rare disease drugs, and owes much of its success to Soliris.

First approved for PNH in 2007, Soliris has since been cleared to treat three other uncommon illnesses. The drug, which for years ranked as one of the world's most expensive medicines, also continues to be Alexion's main source of income. In 2020, it was responsible for two-thirds of the company's $6.1 billion in net product sales.

Though Soliris has remained a top seller, Alexion knows the patents protecting its prized franchise won't last indefinitely. In Europe, for example, biosimilar competition is expected to arrive next year. Alexion has therefore been working to switch PNH patients off of Soliris and onto Ultomiris, which was approved in late 2018. Already, the company's made progress, as net sales of Ultomiris were just under $1.1 billion in 2020, up from $339 million in 2019.

Now, though, Alexion also must contend with Empaveli.

The FDA's approval decision was based on a study named PEGASUS, which tested Empaveli head to head against Soliris. Results from the study recently published in The New England Journal of Medicine show that Apellis' drug was both superior on a measure of blood health, and non-inferior to Soliris when looking at blood transfusion avoidance. According to Apellis, 85% of the patients given Empaveli were transfusion free over 16 weeks, compared to 15% of Soliris-treated patients.

"This is excellent data and positions [Apellis' drug] as standard of care in Soliris non-responders," Umer Raffat, an analyst at Evercore ISI, wrote in early 2020, when data from the study were first disclosed.

With approval in hand, Apellis is turning its attention to launching Empaveli. The company said in a Monday presentation that it's

targeting 1,000 to 2,000 healthcare professionals at more than 90 key treatment centers for PNH.

Pricing Empaveli similar to or lower than Alexion's drugs may also help Apellis get a foothold in the PNH treatment market, though some were expecting a steeper discount. Derek Archila, an analyst at Stifel, wrote in a Sunday note to clients that his team had forecasted a price tag in the $300,000 range.

Still, the FDA's decision to clear Empaveli for first-line use, meaning in patients who've never received treatment, comes as an "upside surprise" that should prop up sales, according to Archila.

"This is better than what we think the Street was expecting as most thought it would gain approval only as a second-line treatment," the analyst wrote.

Apellis shares were up 20% Monday morning, to trade a little over $53 apiece.