BIOPHARMADIVE

DIVE BRIEF

Orchard nears FDA decision on rare disease gene therapy

Three years after gaining European approval, Libmeldy is now under U.S. review with a deadline set for March.

Published Sept. 18, 2023

Jonathan Gardner
Senior Reporter

An Orchard Therapeutics sign hangs at a medical meeting. Jonathan Gardner / BioPharma Dive

Dive Brief:

- The Food and Drug Administration is reviewing Orchard Therapeutics' approval application for a gene therapy to treat the rare disease metachromatic leukodystrophy, setting a decision deadline of March 18, 2024, the company said Monday.
- Approved in Europe in 2020 as Libmeldy, the gene therapy has been used in patients from six different countries since its commercial launch there. U.S. approval could help significantly expand use and sales revenue, which amounted to $7.2 million through the first six months of 2023.
- The London-headquartered company last year laid off workers and discontinued development of some of its pipeline projects to help sustain operations through a U.S. launch of Libmeldy as investor enthusiasm for gene therapies cooled. Orchard closed a fundraising worth up to $188 million with private equity and venture capital firms to help fund operations through at least 2025.

Dive Insight:

Metachromatic leukodystrophy affects about 1 in every 100,000 newborns, or fewer than 50 new cases per year in the U.S. It's caused by a mutation that results in an accumulation of fat in the brain and other organs, in turn causing neurological problems and death. It is one in a family of diseases called "lysosomal storage disorders" that have been targeted by gene therapy developers.

Like other conditions treated by gene therapies, metachromatic leukodystrophy's rarity has been accompanied by sky-high prices for treatment, with Orchard seeking 2.9 million euros per patient. A significant discount helped Orchard secure coverage in England and Wales, and investors are likely looking toward U.S. approval for it to achieve sales expectations, which Stifel analyst Dae Gon Ha pegged at $200 million in 2035.

Unlike the first gene therapies approved in the U.S., which relied on adeno-associated viruses infused into patients to deliver a genetic payload, Libmeldy uses a lentivirus to correct faulty genes in stem cells that have been removed from the patient and are later reintroduced to treat the disorder.

Two lentivirus-based gene therapies have now been approved — Bluebird bio's Zyntelgo and Skysona — so the FDA has experience in reviewing treatments using this approach. As a result, regulators may not feel a need to seek input from an advisory committee, Ha wrote in a note to clients.

Ha added that FDA approval may be "symbolic" for investors, who are looking for Orchard to achieve a successful Libmeldy U.S. launch before they put more value on the company's earlier-stage assets.