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Sarepta stands behind Elevidys after FDA requests gene therapy be pulled from market

By **Fraiser Kansteiner**
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Sarepta | Sarepta Therapeutics | Elevidys | gene therapy

The company staked its position shortly after the FDA asked Sarepta to voluntarily stop all shipments of Elevidys, which is being investigated by the U.S. drug regulator after two patients died following treatment with the therapy earlier this year. (Sarepta)

As the controversy over the safety of Sarepta Therapeutics' gene therapies comes to a head, the biotech is standing firm behind its approved Duchenne muscular dystrophy (DMD) treatment.

The FDA, for its part, is asking the company to pull the drug from the market.

Sarepta will continue to ship (https://www.businesswire.com/news/home/20250718534546/en/Sarepta-Therapeutics-Provides-Statement-on-ELEVIDYS) its gene therapy Elevidys to DMD patients who can still walk independently, the company said in a statement late Friday, citing its own "comprehensive scientific interpretation of the data, which shows no new or changed safety signals in the ambulant patient population."

"We look forward to continued discussions and sharing of information with FDA in order to advance our shared purpose of protecting patient safety and informed access to care," Sarepta added.

The company staked its position shortly after the FDA asked (https://www.fda.gov/news-events/press-announcements/fda-requests-sarepta-therapeutics-suspend-distribution-elevidys-and-places-clinical-trials-hold) Sarepta to voluntarily stop all shipments of Elevidys, which is being investigated by the U.S. drug regulator after two patients died following treatment with the therapy earlier this year.

At the same time, the FDA revoked Sarepta's platform technology designation—which can help hasten the FDA review process for new products stemming from the same platform—and placed Sarepta's investigational gene therapy trials in limb-girdle muscular dystrophy on clinical hold.

Sarepta's limb-girdle program was already greatly diminished after the company on Wednesday announced (https://www.fiercebiotech.com/biotech/sarepta-pivots-sirna-and-lays-500-staffers-elevidys-gets-box-warning) a pipeline shakeup and hundreds of layoffs in a bid to save $400 million annually.

The new clinical hold affects four Sarepta gene therapy assets, three of which the company had already discontinued last week, Sarepta noted in an SEC filing (https://www.sec.gov/Archives/edgar/data/873303/000119312525161516/d882671d8k.htm) Monday. Sarepta said it plans to discuss a potential Biologics License Application for the fourth asset, SRP-9003, with the FDA once the clinical hold is lifted.

The FDA action—and Sarepta's refusal to step down on Elevidys—capped off a whirlwind week for the company, with the restructuring announcement Wednesday quickly followed by the revelation of third patient death late Thursday and a tense call (https://www.fiercebiotech.com/biotech/3rd-patient-dies-following-treatment-sarepta-gene-therapy-reports) with analysts Friday morning.

The third death of a patient on a Sarepta therapy occurred in a 51-year-old man with limb-girdle muscular dystrophy type 2D/R3 who participated in a phase 1 trial. He had not received Sarepta's approved treatment Elevidys, but rather an investigational gene therapy coded SRP-9004. As with the prior deaths of patients who'd received Elevidys, the SRP-9004 patient died from acute liver failure.

Sarepta said it was pausing development of SRP-9004 and many other gene therapy programs on Wednesday, prior to news of the latest fatality breaking, and the company made no mention of the issue during a conference call that same day.

"We recognize that the death of any patient is heartbreaking, including the recent death of a 51-year-old non-ambulant Limb-Girdle Muscular Dystrophy (LGMD) patient," Sarepta said in its Friday press release. The company stressed that the investigational gene therapy was intended to treat a different disease, administered using a different dose and manufactured under a different process than Elevidys.

Still, Sarepta's platform is now steeped in scrutiny, with the FDA noting in its announcement that the three deaths of Sarepta patients that have been reported "appear to have been a result of acute liver failure in individuals treated with Elevidys or investigational gene therapy using the same AAVrh74 serotype that is used in Elevidys."

The FDA said it revoked Sarepta's platform technology designation "because, among other things, given the new safety information, the preliminary evidence is insufficient to demonstrate that AAVrh74 Platform Technology has the potential to be incorporated in, or utilized by, more than one drug without an adverse effect on safety."

While Sarepta executives repeatedly stressed last week that the latest patient death was unrelated to Elevidys, analysts at Jefferies wrote in a Sunday note that doctors, patients and their families may "rethink" treatment with the approved gene therapy in light of the latest developments.

"[W]e wouldn't be surprised if the patient community grew more divided" around Elevidys, the analysts added.

With Sarepta refusing to pull Elevidys in ambulatory patients, it remains unclear if the FDA has the power to force a market withdrawal, the Jefferies team noted. The drug boasts full approval in walking DMD patients, as opposed to an accelerated nod, which the FDA can revoke if a drug fails to impress in subsequent confirmatory studies.

Word that the FDA was contemplating an Elevidys market pull surfaced (https://www.fiercepharma.com/pharma/fda-mulls-elevidys-market-withdrawal-following-3rd-death-after-sarepta-gene-therapy) shortly after Sarepta hosted a conference call Friday to discuss the third patient death. A federal official was said to be "taking a hard look at pulling [Elevidys] from the market," according to a statement Fierce Pharma received from the agency late last week.

"We first heard of this potential request earlier in the day at the same time the public and our patient communities did, through media reports," Sarepta noted in its release on the status of Elevidys shipments.

Elevidys was initially approved in June 2023 in ambulatory DMD patients before winning a label expansion to treat those who are non-ambulatory a year later. Sarepta reported the first death of an Elevidys patient in March and a second in June of this year, which prompted the company to suspend use of the gene therapy in the commercial setting for non-walking DMD patients.

After the second death, Sarepta said it'd seek FDA approval of an enhanced mitigation measure leveraging the immunosuppressant sirolimus to help patients on the approved treatment manage liver toxicity.

Sarepta | Sarepta Therapeutics | Elevidys | gene therapy | U.S. FDA | drug safety | patient death | clinical hold | drug sales | Pharma