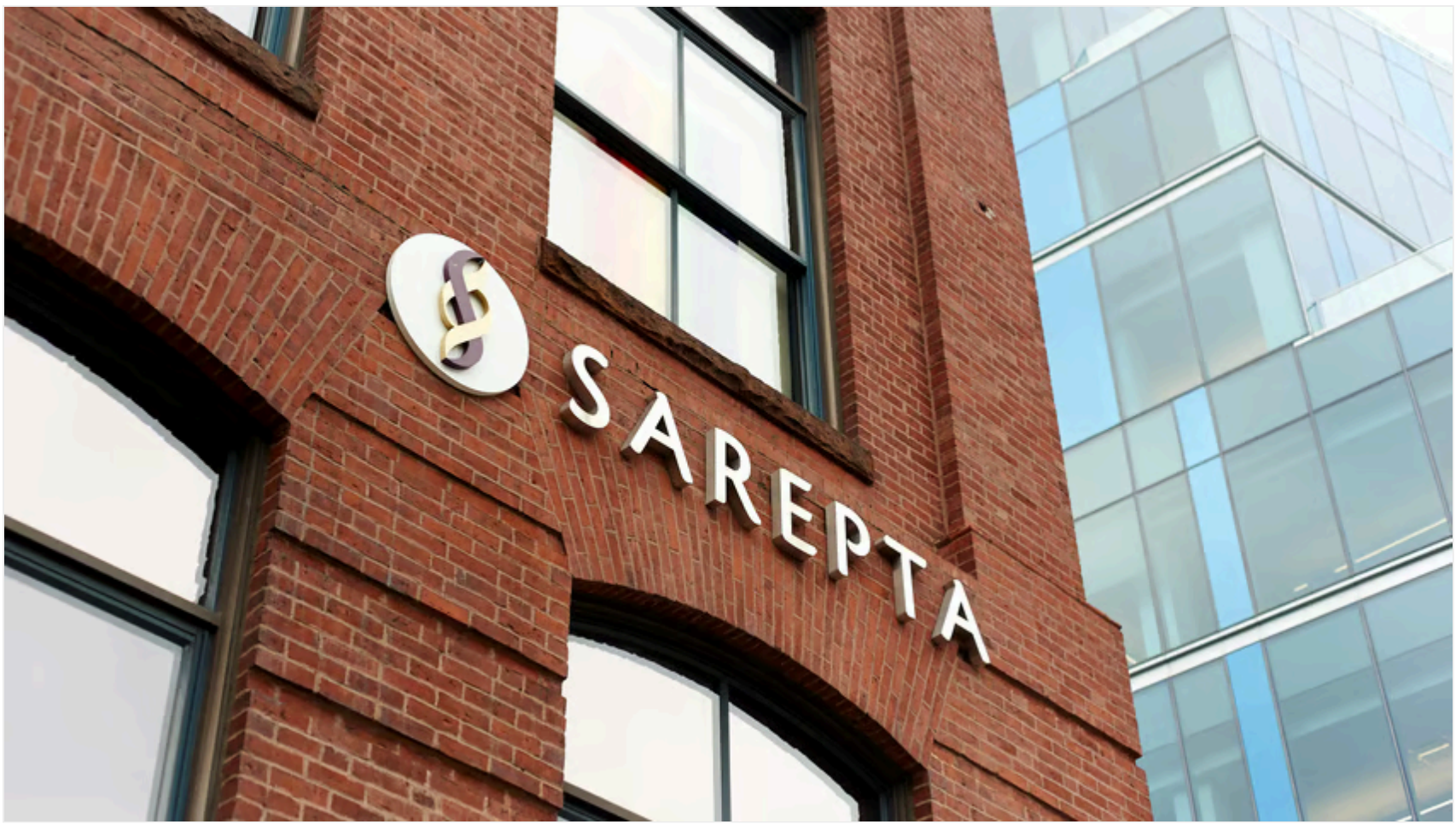
PHARMA

Sarepta, bowing to FDA pressure, pauses shipments of Duchenne gene therapy Elevidys

By **Angus Liu**
Jul 22, 2025 9:25am

Sarepta Therapeutics | Cell & Gene Therapy | Elevidys | Duchenne muscular dystrophy (DMD)



Sarepta Therapeutics said the pause will allow the company "the necessary time to respond to any requests for information" and to complete updating Elevidys' safety labeling per an agreement with the FDA. (Sarepta Therapeutics)

A brief skirmish between Sarepta Therapeutics and the FDA has ended before escalating into a full-on regulatory clash, as the company has bowed to the agency's demand.

In a surprising reversal, Sarepta on Monday said it will pause (https://www.businesswire.com/news/home/20250721946260/en/Sarepta-Therapeutics-Announces-Voluntary-Pause-of-ELEVIDYS-Shipments-in-the-U.S.)all shipments of its Duchenne muscular dystrophy gene therapy Elevidys in the U.S., effective at the end of business on July 22.

The FDA made the distribution suspension request (https://www.fiercepharma.com/pharma/fda-mulls-elevidys-market-withdrawal-following-3rd-death-after-sarepta-gene-therapy)Friday after multiple patient deaths, including two following treatment with Elevidys. More recently, Sarepta disclosed the death of a third patient who was given one of its gene therapies. That patient had received an investigational therapy for a different disease—limb-girdle muscular dystrophy (LGMD)—in a clinical trial.

Sarepta initially refused (https://www.fiercepharma.com/pharma/sarepta-stands-ground-elevidys-sales-after-fda-requests-gene-therapy-be-pulled-market) to play ball with the FDA's request, citing its own "comprehensive scientific interpretation of the data," but it's now changing course.

In its Monday release, Sarepta said the pause will allow the company "the necessary time to respond to any requests for information" and to complete updating Elevidys' safety labeling per an agreement with the FDA. The company is working with the FDA to include a black box warning for acute liver injury and an updated risk management scheme in the drug's label.

The Massachusetts biotech "looks forward to a collaborative, science-driven review process and dialogue with the FDA," Sarepta said in its release.

Going against the FDA may have been a fight Sarepta was destined to lose.

"It is important for the patients we serve that Sarepta maintains a productive and positive working relationship with FDA, and it became obvious that maintaining that productive working relationship required this temporary suspension while we address any questions that FDA may have and complete the Elevidys label supplement process," Sarepta CEO Doug Ingram said in a statement.

Further, the pressure might not have come from the FDA alone, even though Sarepta said the move was "voluntary" and "proactive."

Children's Hospital Los Angeles had paused infusion (https://www.bloomberg.com/news/articles/2025-07-21/sarepta-s-elevidys-halted-at-children-s-hospital-los-angeles?sref=XGjS8839) of Elevidys on Friday, citing patient safety, Bloomberg reports. An article in November noted that the hospital had treated 10 boys with Elevidys at the time, making it one of the largest Elevidys treatment facilities by patient numbers, Leerink Partners analysts pointed out in a Monday note.

The marketing suspension means Duchenne patients won't be able to get Elevidys for an indefinite period. Sarepta had argued that because Duchenne is an irreversible progressive disease, patients need timely treatment.

"While this pause will be a disappointment for the many families of boys with DMD in need of treatment options, we believe it is the right move and may help [Sarepta] rebuild goodwill with the FDA, which has likely eroded following recent events," Leerink's analysts said.

Sarepta reported the two Elevidys patient deaths in March and June. In both cases, the teenage boys were no longer able to walk independently because of the progression of their disease. Both passed away after developing acute liver failure.

Following the second death, Sarepta voluntarily paused (https://www.fiercepharma.com/pharma/sarepta-records-second-death-after-dmd-gene-therapy-elevidys-stops-dosing-half-patients) shipments for non-ambulatory patients but insisted that Elevidys remain available for ambulatory patients, who are typically younger.

The FDA's demand followed a third death (https://www.fiercebiotech.com/biotech/3rd-patient-dies-following-treatment-sarepta-gene-therapy-reports) from a Sarepta product. A 51-year-old non-ambulant LGMD patient died after receiving Sarepta's experimental SRP-9004 in a phase 1 clinical trial. Elevidys and SRP-9004 use the same adeno-associated virus vector, which is known to carry liver toxicity risks.

Alongside the request for pausing Elevidys' distribution, the FDA also put Sarepta's LGMD trials on clinical holds and revoked (https://www.fiercebiotech.com/biotech/sarepta-ldmd-trials-all-hit-fda-hold-amid-newly-surfaced-safety-concerns) Sarepta's platform technology designation, which is meant to support the development of novel therapies based on the same tech platforms.

Sarepta Therapeutics | Cell & Gene Therapy | Elevidys | Duchenne muscular dystrophy (DMD) | drug safety | gene therapy | U.S. FDA | FDA | Marketing | Pharma

PHARMA

AbbVie wins $56M royalty award from Revance in Botox patent infringement case

By **Kevin Dunleavy**
Jul 22, 2025 11:22am

Allergan | AbbVie | Botox | patent infringement