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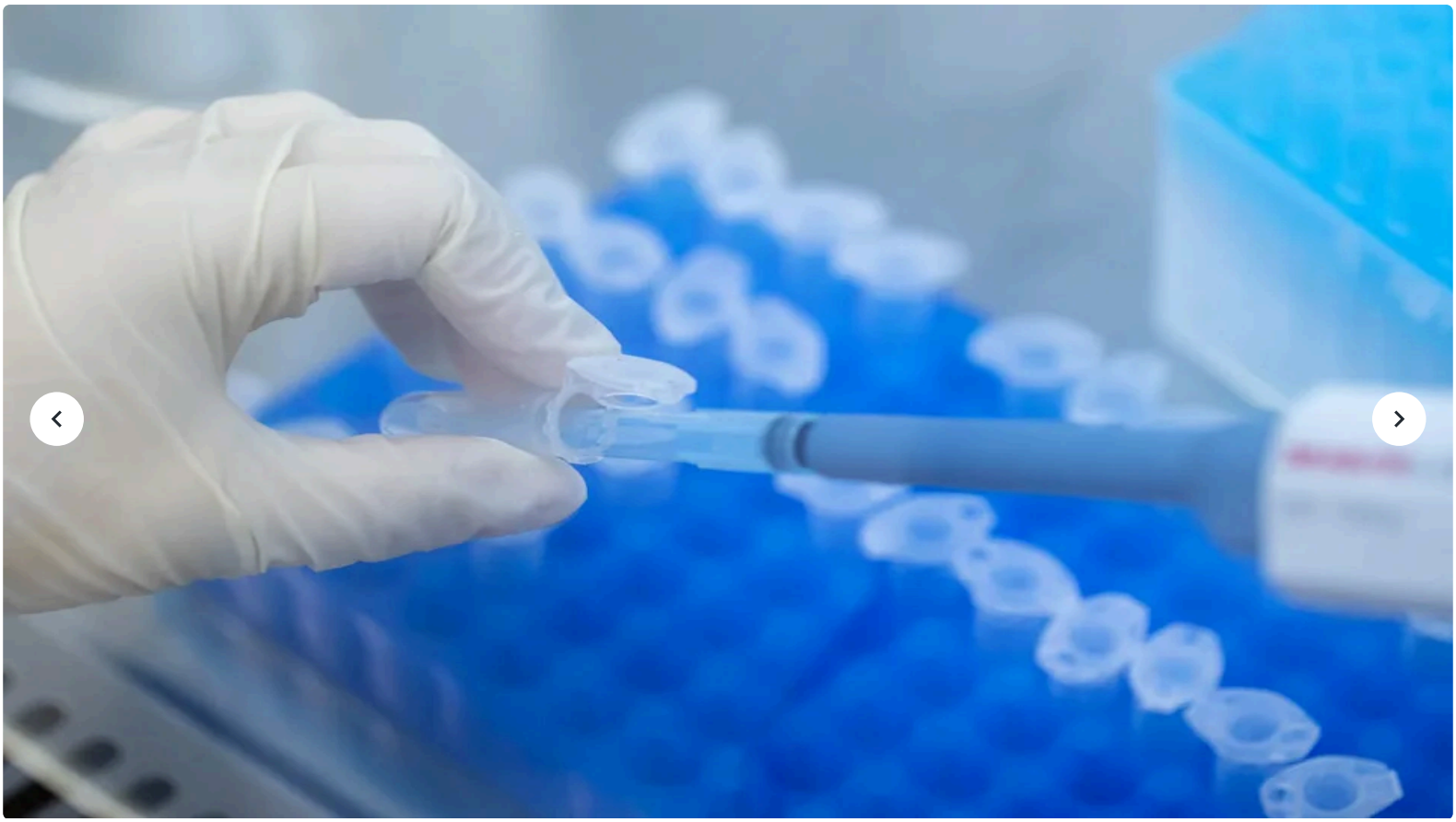

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Sarepta Refused FDA's Request to Halt Elevidys Shipments

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SRPT -5.36% ☆

(Bloomberg) -- Sarepta Therapeutics Inc. has refused to pause all shipments of its Elevidys treatment after three deaths were linked to the company's gene therapies, the Food and Drug Administration said Friday.

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Two teenage boys died of acute liver failure in recent months after taking Elevidys. They were being treated for Duchenne muscular dystrophy and weren't able to walk because of the muscle-wasting disease. Separately, the company said Friday that a 51-year-old patient died of acute liver failure last month in an early-stage trial of a gene therapy to treat limb-girdle muscular dystrophy.

FDA leaders met with Sarepta, the agency said in a statement, and requested it voluntarily stop all shipments of the drug, which is its biggest product. "The company refused to do so," the agency said.

In its own statement Friday, Sarepta said it decided to continue shipping Elevidys "based on our comprehensive scientific interpretation of the data, which shows no new or changed safety signals" in patients who can walk.

Shares of Sarepta tumbled 36% on Friday, to their lowest since 2016. The company has lost about $8.5 billion in value since March 18, when the first patient death was reported.

Elevidys is a key drug for Sarepta, making up more than half of the company's net product revenue in the second quarter of this year. In June, Sarepta suspended shipments of the drug for patients who can no longer walk.

Given the new safety information, the FDA told Sarepta the gene therapy should only be given to boys who can still walk. They make up about 85% of the patients who have been treated with Elevidys since its launch, the company has said.

The developments have also raised doubts about the drug's future.

"Following reports that the FDA will ask Sarepta to voluntarily stop all shipments of Elevidys, we think the risks of the FDA removing the drug fully from the market are now greatly amplified," Baird analyst Brian Skorney said in a note Friday.

The newest patient death occurred in a trial of a gene therapy that uses a similar viral delivery method as Elevidys, meaning it could have safety implications for patients getting the drug that's on the market.

In an interview earlier Friday, FDA Commissioner Marty Makary said the regulator was examining whether Elevidys should remain available for sale. The FDA was already investigating the two previous patient deaths. Makary made the comment in response to a question from Bloomberg News and didn't provide any further details.

The regulator is also under pressure for its stance toward Sarepta's gene therapy, which it approved even though early trials didn't clearly show it slowed the disease. The deaths pose one of the first big tests for Vinay Prasad, the new head of the regulator's gene therapy division. As an academic at the University of California San Francisco, prior to his current role, he was highly critical of the FDA's expedited approval process for Sarepta's treatment.

The latest death also sparked an unusually tense exchange between analysts and Sarepta executives on a Friday call. Two days before, the company had held another lengthy call with the same group of analysts, but failed to disclose a third patient had recently died on a similar treatment as Elevidys. On Friday, two analysts asked whether there were any other fatalities the company hadn't revealed.

Sarepta Chief Executive Officer Douglas Ingram said there weren't and defended the company's record, adding: "We are historically a very transparent organization."

Ingram said the company didn't disclose the third patient death earlier in the week because "it was neither material nor central" to what was being discussed. In a note before the Friday call, Leerink analyst Joseph P. Schwartz said the fact Sarepta didn't talk about the latest fatality on Wednesday was "deeply troubling and further undermines credibility." William Blair analyst Sami Corwin said it could increase "investor distrust."

Liver Failure

On Wednesday, Sarepta said it had agreed to warn doctors and patients about the risk of liver failure from Elevidys at the request of the FDA. Sarepta executives said the warning label appeared to resolve the FDA's concerns with using the gene therapy to treat children with Duchenne who can walk.

“On the general question about whether Elevidys as a therapy will remain on the market, the answer is I think pretty clearly yes,” Ingram said Wednesday on the call.

Duchenne muscular dystrophy is a genetic disorder mainly affecting boys that interferes with the production of the protein dystrophin used by muscle cells. It causes severe muscle weakening and atrophy, with patients needing to use wheelchairs as they get older. Limb-girdle muscular dystrophy is a different form of the condition that often weakens muscles around the hips and shoulders.

Elevidys has been one of the most popular gene therapies on the market. More than 800 patients have been treated with Elevidys in commercial settings and clinical studies, the company said in May. Many families are desperate for anything that might help their children with the fatal muscle disease, despite a lack of data showing Sarepta’s treatment actually slows overall progression of the condition.

Debra Miller, founder of the patient advocacy group CureDuchenne, said families “deserve clear and transparent information” about the safety and efficacy of a treatment, and some have few or no other options.

“Families facing Duchenne do not have time to waste,” she added.

But the series of deaths could make patients more reluctant to use Elevidys. On Wednesday, Ingram said the company needed to “assess the impact that’s occurred from hesitancy associated with these events.”

That same day, the company said it was cutting more than a third of its workforce and pausing several drugs in its pipeline. The moves would contribute to an estimated $400 million in annual cost savings, the company said.

--With assistance from Subrat Patnaik.

(Updates with comments from Sarepta statement in the fourth paragraph.)

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