BIOTECH

UPDATED: Analysts demand transparency after Sarepta's roundabout disclosure of 3rd patient death

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Sarepta | Sarepta Therapeutics | gene therapy | muscular dystrophy

While the previous two deaths occurred in patients treated with Elevidys, the most recent patient had received one of Sarepta Therapeutics' investigational treatments. (Sarepta)

Following a tumultuous week (https://www.fiercebiotech.com/biotech/sarepta-pivots-sirna-and-lays-500-staffers-elevidys-gets-box-warning) for Sarepta Therapeutics, the biotech has confirmed that a third patient has died after receiving one of the company's gene therapies. While the previous two deaths occurred in patients treated with Elevidys, the most recent patient was receiving one of Sarepta's investigational treatments.

As with the two previously reported deaths of Sarepta patients, the outcome was linked to liver toxicity, the company has confirmed with Fierce Biotech.

The newest death reported was a 51-year-old man with limb-girdle muscular dystrophy (LGMD) type 2D/R3 who'd received Sarepta's gene therapy candidate SRP-9004 in a phase 1 trial, Sarepta told BioCentury (https://www.biocentury.com/article/656520/third-death-from-a-sarepta-gene-therapy), the first outlet to report the news late Thursday.

The death occurred last month and was caused by liver failure.

The company has informed regulators and clinical investigators of the death, a Sarepta spokesperson told Fierce.

"Advancing the science of genetic medicine is incredibly challenging—especially in ultra-rare diseases like the limb-girdle subtypes," the spokesperson said. "While we do everything possible to ensure patient safety, there is inherent risk in clinical trial participation and we are grateful to the courageous patients and families who participate."

Sarepta's stock price was down more than 33% by midday Friday, marking a reversal from the gains the biopharma experienced after announcing its corporate overhaul late Wednesday.

Notably, as part of a major restructure announced this week, Sarepta deprioritized development of most of its gene therapy assets in LGMD, including SRP-9004. However, the patient death was not disclosed on an investor call or release shared Wednesday about the changes.

"[W]e did not discuss this matter in our call on Wednesday because it was neither material nor central to the topics at hand," Sarepta's CEO Douglas Ingram said during a Friday investor call that was announced after several news outlets reported the death.

The fatality may now further compound safety questions around the company's approved gene therapy Elevidys and another candidate under development in a different subtype of LGMD called SRP-9003, multiple analysts have warned.

"We view the patient death as unfortunate and concerning, particularly since it occurred with one of the company's other gene therapies," analysts at William Blair wrote in a Friday note to clients, alluding to the prior two deaths (https://www.fiercepharma.com/pharma/sarepta-records-second-death-after-dmd-gene-therapy-elevidys-stops-dosing-half-patients)that occurred after treatment with Duchenne muscular dystrophy (DMD) treatment Elevidys.

In its corporate update Wednesday, Sarepta said it would deprioritize its LGMD franchise save for SRP-9003, with the William Blair team now speculating that "the patient death could have played a role in the company's pipeline reprioritization."

Ingram disagreed with that notion on Sarepta's call, stating that, "[t]his event occurred in a trial that was otherwise completed with all dosing and for a therapy for which we determined independent of this not to proceed."

Sarepta still plans on submitting an FDA approval application for the other LGMD therapy SRP-9003 in the back half of 2025, the analysts noted.

The team over at Leerink Partners was more scathing in its assessment, writing that news of the death will "likely diminish any remaining goodwill management had." The Leerink note was released before Sarepta publicly confirmed it was aware of the death prior to its restructuring announcement.

The sentiment was echoed by analysts who participated in Sarepta's Friday call.

"You just hosted a conference call on Wednesday outlining the strategic initiatives and restructuring at the company. You talked about how the limb-girdle patient death has been known for about a month or so," J.P. Morgan's Anupam Rama said at the top of the question-and-answer portion. "Can you outline the thinking and considerations around the disclosure strategy here?"

"The normal approach to discussing clinical trial results would be in the context of all of the data," Ingram responded. "We don't have that data yet."

That did little to alleviate the skepticism in the virtual room, with Baird analyst Brian Skorney deeming the patient death information "highly material" and calling on Sarepta to be transparent immediately.

Meanwhile, both William Blair and Leerink agreed in their notes that the turn of events will likely have a knock-on effect for the separate gene therapies Elevidys and SRP-9003.

The death "could lead to greater scrutiny of SRP-9003's safety profile" ahead of Sarepta's submission and "could affect commercial interest, if approved," the William Blair team said.

"In addition, we think the LGMD patient death could amplify patient hesitancy to use commercial Elevidys and increase investor distrust since the company did not disclose the event on its call on July 16," the analysts cautioned.

The Leerink team concurred that "investors will likely begin to further question the risk/benefit of SRP-9003 … as well as the Elevidys program."

While the risk-benefit calculus in Elevidys' approved indication is "very different" than in LGMD given the relative severity of DMD, the two assets are engineered using the same viral vector, the Leerink team pointed out.

Ingram, for his part, said he doesn't believe the patient death from the limb-girdle trial will influence the perception of Elevidys if the situation is "properly contextualized."

"I don't believe this readthrough to Elevidys for a number of reasons," the CEO explained, stressing that the death occurred with a "different therapy … different dosing [and] different manufacturing process."

"Also, even if it did have readthrough, it would be a readthrough to a signal that we've already fully appreciated and disclosed and know about," he added.

Nevertheless, FDA Commissioner Martin Makary, M.D., told Bloomberg News on Friday that he's now "taking a hard look" (https://www.fiercepharma.com/pharma/fda-mulls-elevidys-market-withdrawal-following-3rd-death-after-sarepta-gene-therapy) at whether Elevidys should remain on the market. The commissioner's comment appeared to worsen Sarepta's stock losses (https://finance.yahoo.com/quote/SRPT/) Friday.

Given the mounting uncertainty around Sarepta's transparency on safety, analysts asked at multiple points whether there were any other deaths or serious safety signals they should know about that the company has yet to divulge. Sarepta's management repeatedly said there were not.

"We are, I think, historically, a very transparent organization," Ingram said near the call's close.

News of the patient death caps off a tough week for Sarepta in which layoff rumors swirled online until the company ultimately confirmed (https://www.fiercebiotech.com/biotech/sarepta-pivots-sirna-and-lays-500-staffers-elevidys-gets-box-warning) a major pipeline pivot, 500 job cuts and a black box warning for acute liver injury on the label for Elevidys.

The restructuring followed the deaths earlier this year of two non-ambulatory DMD patients—both teenage boys—who had received Elevidys, prompting safety scrutiny that has loomed over Sarepta for the better part of 2025.

Under the reorganization, Sarepta has paused development of the bulk of its gene therapy candidates, opting instead to focus on siRNA programs hailing from its internal platform. Those include investigational therapies for myotonic dystrophy type 1, idiopathic pulmonary fibrosis and Huntington's disease.

The company has also said it will seek strategic alternatives, like partnering, for its deprioritized programs.

Editor's note: *This story was updated at 12:49 p.m. ET on July 18 with executive comments from a Sarepta investor call.*

Sarepta | Sarepta Therapeutics | gene therapy | muscular dystrophy | patient death | drug safety | Adenovirus | clinical trial | Elevidys | Cell & Gene Therapy | Biotech