

Novartis says two children treated with gene therapy died

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Two children who received Zolgensma, a gene therapy produced by Swiss drugmaker Novartis to treat spinal muscular atrophy, have died from liver failure, the company said Friday.

Health authorities in Russia and Kazakhstan, where the deaths occurred, have been notified along with those in all countries where the treatment is used, Novartis said in an e-mailed statement.

The children died five to six weeks after receiving infusions of Zolgensma and one to 10 days after receiving a steroid aimed at reducing side effects, the company said.

"While acute [liver failure](#) is a known adverse event, these are the first fatal cases of [acute liver failure](#)," Novartis said, adding that it would update the labelling of the drug to specify that fatal liver failure has been reported.

Spinal muscular atrophy affects about 1 in 10,000 births, and results in death or the need for permanent ventilation by the age of two in 90 percent of cases.

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