

Girl aged 5 dies in US during French gene therapy trial

October 15 2020

A five-year-old girl with a rare neurodegenerative disease died in the US while taking part in a gene therapy trial run by French biotechnology company Lysogene, the firm said Thursday.

The little girl was suffering from Sanfilippo syndrome or mucopolysaccharidosis type III, a rare genetic disease that alters [brain development](#) after birth and leads to [premature death](#).

In a statement Lysogene, a company developing [gene therapy](#) for central nervous system diseases in children, said "the immediate cause of death is currently unknown" and that there was as yet "no evidence that the event is linked to the study [drug administration](#)".

It said it was "profoundly saddened by the passing of this child" and was collecting "additional information" about the circumstances.

The share price of the company dropped 19 percent to 2.05 euros in morning trading in Paris.

The girl was one of 19 people being treated in the trial conducted at eight hospitals in Europe and the United States.

She died at home several months after receiving the therapy, consisting of a single injection, at one of four treatment sites in the US, Lysogene told AFP.

In its statement the company said it was following the remaining 18 patients closely and remained "committed to the LYS-SAF302 development program".

On June 5, the US Food and Drug Administration (FDA) ordered a clinical hold on the trial after observing "localized findings on MRI images at the intracerebral injection sites" suggesting "a potential connection to delivery".

In a statement at the time the company said "no clinical symptoms have been observed that could be directly attributed to the observed MRI findings".

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Citation: Girl aged 5 dies in US during French gene therapy trial (2020, October 15) retrieved 3 February 2023 from <https://medicalxpress.com/news/2020-10-girl-aged-dies-french-gene.html>

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