FDA: approval of zolgensma was based on manipulated data

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The maker of Zolgensma (onasemnogene abeparvovec-xioi) gave

manipulated data to the U.S. Food and Drug Administration when it approved the drug, the agency said Tuesday.

In late May, the FDA approved the gene therapy Zolgensma to treat children with a severe form of spinal muscular atrophy, a leading genetic cause of infant death. The drug costs $2.125 million for a one-time treatment, *CBS News* reported. A month after the approval, the FDA discovered a "data manipulation issue that impacts the accuracy of certain data from product testing performed in animals," according to the agency.

The FDA said the drug's maker AveXis, a unit of Novartis, knew about the data problem before the drug was approved but did not inform the FDA until after the drug was given the green light, *CBS News* reported. "The agency will use its full authorities to take action, if appropriate, which may include civil or criminal penalties," according to Peter Marks, an FDA official. Despite the inaccurate data, the FDA said it "remains confident that Zolgensma should remain on the market."

"We maintain that the totality of the evidence demonstrating the product's effectiveness and its safety profile continue to provide compelling evidence supporting an overall favorable benefit-risk profile," Novartis said in an emailed statement, *CBS News* reported. "At no time during the investigation did the findings indicate issues with product safety, efficacy, or quality."

More information: *CBS News* Article
More Information: FDA

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