Animal safety concerns prompt FDA to halt study testing Zolgensma gene therapy for spinal muscular atrophy

The Food and Drug Administration has halted a clinical trial of Novartis' Zolgensma gene therapy due to a safety concern found in an animal study, the company said [October 30].

The hold affects the Novartis (NVS) clinical trial known as STRONG, which was testing a higher dose of Zolgensma administered by spinal injection to older children with spinal muscular atrophy (SMA). It does not affect the already approved treatment of infants and children.

Novartis said its subsidiary AveXis informed regulators about findings from an animal study that showed "dorsal root ganglia (DRG) mononuclear cell inflammation, sometimes accompanied by neuronal cell body degeneration or loss." The clinical significance of this adverse safety signal is not known, but it can be associated with "sensory effects," the company added.

Halting the STRONG clinical trial is a setback for Novartis' effort to expand the use of Zolgensma to older patients with SMA.

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Novartis said it has seen no reports of "sensory effects" in patients and is working with the FDA to resolve safety concerns and resume dosing of Zolgensma in the clinical trial.

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