# Novartis gene therapy helps children with rare muscle disorder in study

Novartis was testing the therapy, onasemnogene abeparvovec or OAV101 IT, in a late-stage trial of patients between the ages of 2 and less than 18 with spinal muscular atrophy (SMA).


Bengaluru: Swiss drugmaker Novartis said on Monday its gene therapy helped improve motor function in children with a rare muscle disorder that leaves patients too weak to walk, talk and swallow.

Novartis was testing the therapy, onasemnogene abeparvovec or OAV101 IT, in a late-stage trial of patients between the ages of 2 and less than 18 with spinal muscular atrophy (SMA).

The condition prevents the body from producing a protein necessary for neuromuscular development.

The Swiss drugmaker's one-time therapy, branded as Zolgensma, is already approved in the U.S. to treat children less than 2 years of age with the condition.

Crystal Proud, principal investigator at Children's Hospital of the King's Daughters said that "maintaining motor function is a key goal for many older patients with SMA", as it allows them to perform daily activities as independently as possible.

In the study, patients who received the therapy showed improvement in measures such as sitting, rolling, crawling, and standing as measured on a commonly used scale, compared to those who received a sham procedure.

Other approved treatments include Biogen's spinal injection Spinraza, and Roche-PTC Therapeutics' oral drug Evrysdi.

SMA is the leading genetic cause of infant deaths and affects about 1 in 10,000 people, according to U.S. government data. The disorder is categorized into five types - based on the onset of symptoms.

## News Source:

https://pharma.economictimes.indiatimes.com/news/research-and-development/novartis-gene-therapy-helps-children-with-rare-muscle-disorder-in-study/116831111?utm_source=whatsapp_web&utm_medium=social&utm_campaign=socialsharebuttons