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Novartis adds gene therapy for spinal muscular atrophy with \$8.7B acquisition of AveXis

By STEPHANIE BAUM

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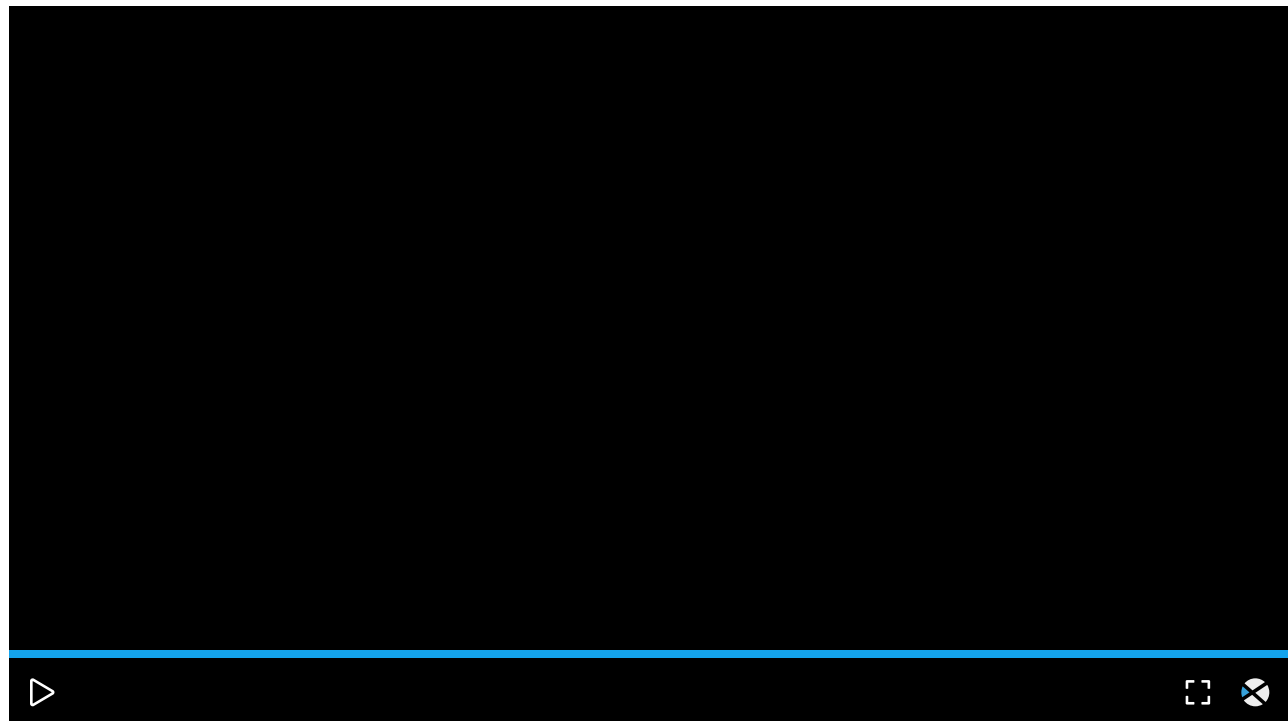
Novartis, which became the first pharma company to secure FDA approval for a gene therapy last year, [has acquired AveXis](#) for \$8.7 billion, which has developed a

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AveXis has breakthrough therapy designation in the U.S. market for the treatment of spinal muscular atrophy Type 1. AVXS-101 also has Orphan Drug designation for the treatment of SMA. What makes the treatment a source of excitement in the gene therapy community is that SMA Type 1 is the leading genetic cause of death in infants.

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In addition to spinal muscular atrophy, AveXis is also developing treatments for Rett Syndrome (RTT) and a genetic form of amyotrophic lateral sclerosis (ALS) caused by mutations in the superoxide dismutase 1 (SOD1) gene.

It marks the second gene therapy deal Novartis has made this year. It also secured a [licensing deal with Spark Therapeutics](#) for its ophthalmologic gene therapy voretigene neparvovec in markets outside the U.S.

One lingering question will be how much Novartis will charge for the one and done treatment. On a conference call with the media Novartis CEO Vas Narasimhan, who took over on February 1, said the company estimates the

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lymphoblastic leukemia for which other treatments had no effect. The price tag of the drug was a whopping \$475,000.

Photo: crazydiva, Getty Images



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