



challenges

matching patients  
with providerswhich employer  
wellness tools

BIOTECH, PHARMA

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

By STEPHANIE BAUM

Post a comment / Apr 9, 2018 at 7:03 AM



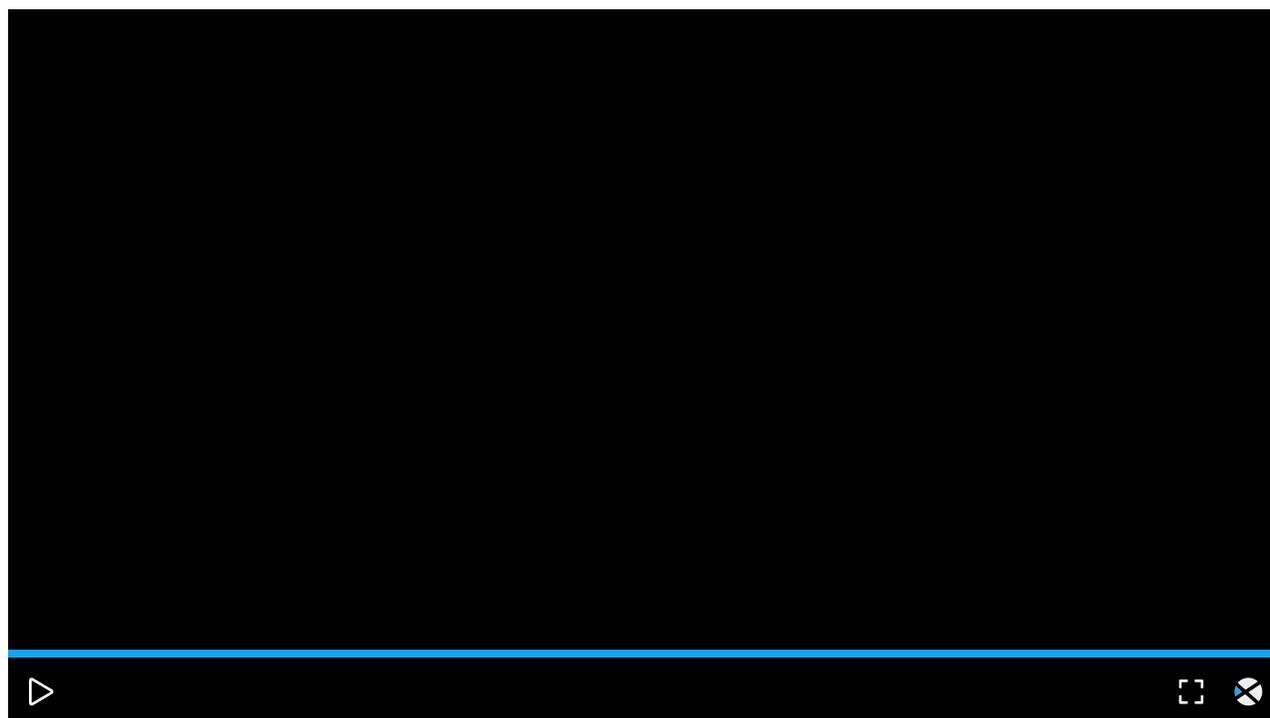
Novartis, which became the first pharma company to secure FDA approval for a gene therapy last year, [has acquired AveXis](#) for \$8.7 billion, which has developed a

## MedCityNews

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.

—ADVERTISEMENT—

SPONSORED BY CONNATIX



MEDCITY NEWS - DAILY HIGHLIGHTS

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

## MedCityNews

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

*Photo: crazydiva, Getty Images*



Join us for [MedCity INVEST](#) May 1-2, 2018, the premier national healthcare investing conference. INVEST unites over 300 active investors with corporate business development executives to facilitate investment opportunities with the most promising healthcare startups. [Register now »](#)



### TOPICS

AveXis, Basel, gene therapy, Illinois, M&A, Novartis, orphan drug, pharma, spinal muscular atrophy, Switzerland



**Hear the latest industry news first.  
Sign up for our daily newsletter.**



### WE RECOMMEND

Gene therapy for spinal muscular atrophy secures funding to begin clinical trials

[MedCity News](#)

Deerfield Management's Jim Flynn on CRISPR, and that \$550M new fund

Novartis to buy US gene therapy group AveXis for \$8.7 bn [↗](#)

[MedicalXpress](#)

Experiments in mice may help boost newly FDA-approved therapy for spinal muscular