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

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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

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.

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

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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