New Drug Approved for DMD

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On Dec. 12, 2019, the US Food and Drug Administration (FDA) granted accelerated approval to Sarepta Therapeutics' golodirsen (Vyondys 53) for the treatment of Duchenne muscular dystrophy (DMD) in patients amenable to skipping exon 53. It is the second exon-skipping, disease-modifying drug to treat DMD, the most common childhood form of muscular dystrophy, and is given intravenously (into the vein).

The approval was based on an observed statistically significant increase in dystrophin production in skeletal muscle of 25 boys with DMD treated with Vyondys 53 in a pivotal (phase 1/2) clinical trial, which is reasonably likely to predict clinical benefit for those patients. The continued approval of Vyondys 53 may be contingent on confirmation of a clinical benefit in a post-marketing confirmatory trial (ESSENCE), which is currently enrolling and expected to conclude by 2024.

"The Duchenne community is clamoring for new therapeutic options," says John Brandsema, MD, a pediatric neurologist at Children's Hospital of Philadelphia. "Each new approved therapy increases options for provider teams and families to strive together in optimizing quality of life for those living with DMD."

DMD is caused by mutations in the dystrophin gene on the X chromosome that result in little or no production of dystrophin, a protein essential to keeping muscle cells intact. Vyondys 53 is called an exon-skipping drug in that it is designed to target and promote skipping over a section of genetic code in order to avoid the gene mutation and produce more of the dystrophin protein. It is estimated that up to 8% of patients with DMD have mutations amenable to treatment with Vyondys 53.

Although treatment with Vyondys 53 will not cure DMD, it could slow progression of the disease, which in turn could extend the length of time individuals with DMD could walk, eat independently, and breathe without assistance.

In September 2016, the approval of Sarepta's eteplirsen (Exondys 51) marked a watershed moment for treating neuromuscular diseases with gene-targeting therapies such as exon skipping. Approval of Vyondys 53, another exon skipping drug designed to treat a different subset of DMD individuals than those who qualify for Exondys 51, is another significant step forward in the development of therapies for DMD — and all neuromuscular diseases — that target the root cause of the disease.