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FDA Grants Orphan Drug Designation to Ifetroban for Treatment of Duchenne Muscular Dystrophy-Associated Cardiomyopathy

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Key Takeaways

- Ifetroban received FDA orphan drug and rare pediatric disease designations for cardiomyopathy in Duchenne muscular dystrophy.
- The FIGHT DMD trial evaluates ifetroban's effectiveness, safety, and pharmacokinetics in DMD patients.

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The drug is being investigated for its potential to reduce fibrosis associated with muscular dystrophy that can cause scarring of the heart tissue.

The FDA granted orphan drug designation (ODD) and rare pediatric disease (RPD) designation to ifetroban, a thromboxane-prostanoid receptor (TPr) antagonist, for the treatment of cardiomyopathy associated with Duchenne muscular dystrophy (DMD), according to a news release from Cumberland Pharmaceuticals.[1]

Duchenne muscular dystrophy can cause skeletal and muscle weakness. | Image Credit: © Premium Graphics | stock.adobe.com

"For Duchenne muscular dystrophy, a devastating genetic disorder affecting young boys, securing both orphan drug and rare pediatric disease designations for ifetroban from the FDA is a critical step forward," AJ Kazimi, CEO of Cumberland Pharmaceuticals, said in the news release.[1]

ODD is granted by the FDA to encourage the development of new therapies for rare diseases or conditions through clinical

trials. Additionally, RPD is granted to medications that are rare in children, requiring a special focus for therapeutic options. Ifetroban is currently being investigated in the FIGHT DMD trial, a 12-month, double-blind, placebo-controlled study designed to evaluate oral ifetroban's effectiveness, safety, and pharmacokinetics in patients with DMD.[1,2]

"These designations not only recognize the urgent need for effective treatments, but also provide vital support to accelerate research and development," Kazimi said. "These important regulatory milestones represent hope for families and a pathway to bring transformative medicines to a vulnerable patient population more quickly and efficiently."[1]

Particularly, the trial seeks to address the fibrosis that occurs in patients with DMD. In fibrosis, normal tissues around the heart are replaced with scarred tissue. Until now, ifetroban had not been studied as a potential anti-fibrotic medication in patients with DMD. However, studies in animal models have shown that oral ifetroban can prevent scarring in the heart and improve survival outcomes compared to placebo.[2,3]

DMD affects about 1 in every 3300 male births worldwide. As rare and fatal genetic disorder, it is characterized by mutations in the gene that produces dystrophin and causes damage to the skeletal and cardiac muscle of patients. Cardiomyopathy, for which the FDA granted ODD and RPD to further study ifetroban, is the primary cause of death in patients with DMD.[1]

Ifetroban's mechanism of action involves exhibiting high affinity for TPr on multiple cell types, including platelets, cardiomyocytes, vascular and airway smooth muscle, and fibroblasts. Notably, it lacks agonistic activity. Pre-clinical models of muscular dystrophy—including DMD—have indicated that ifetroban could prevent cardiac dysfunction and fibrosis while improving mortality.[1]

The drug is also being evaluated in a clinical trial for systemic sclerosis and pulmonary fibrosis. FIGHTING FIBROSIS, a multicenter, randomized, placebo-controlled phase 2 study, has enrolled patients with idiopathic pulmonary fibrosis (IPF), a progressive lung disease marked by inflammation and fibrosis of the lungs. Currently, there is no cure, and fibrosis can lead to rapidly declining lung function and reduced survival within 5 years of a diagnosis.[1,4]

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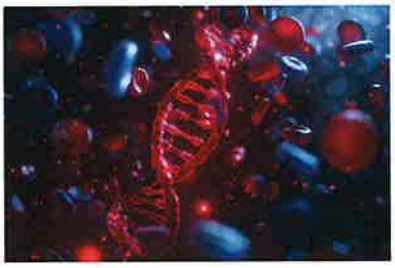

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