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Ifetroban Improves Heart Function in DMD

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Key Takeaways

- Ifetroban significantly improved LVEF in DMD patients, showing a 5.4% improvement compared with propensity-matched natural history controls, with high-dose treatment yielding the most benefit.
- The FIGHT DMD trial is the first successful phase 2 study targeting cardiac complications in DMD, addressing a critical unmet need.

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There is an unmet need for therapies targeting Duchenne muscular dystrophy (DMD)–related heart disease, and phase 2 results suggest ifetroban may improve left ventricular ejection fraction in patients with DMD.

Ifetroban, a novel oral thromboxane receptor antagonist, significantly improved left ventricular ejection fraction (LVEF) in patients with Duchenne muscular dystrophy (DMD) in the phase 2 FIGHT DMD trial (NCT03340675), according to a press release from Cumberland Pharmaceuticals.[1] There is an unmet need for drugs targeting cardiac complications in this patient population, and FIGHT DMD is the first successful phase 2 study in this area.

DMD is an incurable inherited disease characterized by progressive muscle weakness, and cardiomyopathy is the leading cause of death in these patients.[2] Cardiac treatment options typically aim to delay heart disease progression, but there is no cure for cardiac dysfunction in DMD. Ifetroban showed promise in preclinical models of muscular dystrophy, leading to an FDA Office of Orphan Products Development clinical trial grant to develop the FIGHT DMD trial.[1]

"This trial represents hope for our Duchenne community," Pat Furlong, founding president and CEO of Parent Project Muscular Dystrophy, said in a statement. "Heart disease remains one of the most devastating aspects of Duchenne, and these results suggest we may finally have a therapeutic option that could make a meaningful difference in the lives of patients and families."

Ifetroban may improve LVEF in patients with DMD. - REDPIXEL - stock.adobe.com

In the 12-month, double-blind, randomized, placebo-controlled study, a

total of 41 patients were randomized to receive high-dose ifetroban at 300
mg per day, low-dose ifetroban at 150 mg per day, or a placebo. Improvement in LVEF was the primary end point in the trial.

Those treated with high-dose ifetroban showed a 3.3% improvement in LVEF overall, with the high-dose cohort experiencing a 1.8% increase in LVEF and the placebo group showing an expected 1.5% decline in LVEF.

There was an even more significant improvement in LVEF in the high-dose group compared with propensity-matched natural history controls. The control patients showed a 3.6% decline in LVEF, making the overall improvement in LVEF 5.4% with high-dose ifetroban treatment. In both treatment groups, the therapy was well-tolerated. There were no serious drug-related adverse events.

"These results represent a significant milestone in DMD cardiomyopathy," Larry W. Markham, MD, professor of Pediatrics and Medicine, Indiana University School of Medicine, Division Chief of Pediatric Cardiology at Riley Children's Hospital and Principal Investigator of the FIGHT DMD trial, said in a statement. "We are seeing evidence that there is an opportunity to potentially alter the course of heart disease in DMD patients. The improvement in cardiac function observed with ifetroban, particularly in the high-dose group, offers hope for these patients and their families."

"These results represent a significant milestone in DMD cardiomyopathy," Larry W. Markham, MD, professor of Pediatrics and Medicine, Indiana University School of Medicine, Division Chief of Pediatric Cardiology at Riley Children's Hospital and Principal Investigator of the FIGHT DMD trial, said in a statement. "We are seeing evidence that there is an opportunity to potentially alter the course of heart disease in DMD patients. The improvement in cardiac function observed with ifetroban, particularly in the high-dose group, offers hope for these patients and their families."

Ifetroban could potentially fill a substantial unmet need among patients with DMD, and the therapy received Orphan Drug Designation and Rare Pediatric Disease Designation from the FDA. If it is approved, it would be the first therapy specifically targeting heart disease related to DMD. Further analysis and a full report on the FIGHT DMD study will be shared in a meeting with the FDA, according to the press release.

References

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2. Schultz TI, Raucci FJ Jr, Salloum FN. Cardiovascular disease in Duchenne muscular dystrophy: overview and insight into novel therapeutic targets. *JACC Basic Transl Sci*. 2022;7(6):608-625. doi:10.1016/j.jacbts.2021.11.004