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Cumberland's Duchenne drug improves blood flow from heart in phase 2 trial

By **James Waldron** · Feb 4, 2025 10:20am

Patients receiving 300 mg ifetroban daily saw a 1.8% increase in LVEF, while the placebo group saw their LVEF fall by an average of 1.5%, Cumberland said on Tuesday. (iStock / Getty Images Plus)

Cumberland Pharmaceuticals' Duchenne muscular dystrophy (DMD) drug has improved the amount of blood pumped by the heart, hitting the main goal of a phase 2 trial.

The active thromboxane receptor antagonist, called ifetroban, was evaluated in a trial of 41 patients with DMD. In the study, patients received either a 150 mg or 300 mg daily dose of the drug or placebo over 12 months.

The high-dose cohort saw a 3.3% improvement in the heart's left ventricular ejection fractions (LVEF)—a measure of the amount of blood pumped out in a heartbeat—compared to placebo, Cumberland said in a Feb. 4 release. Specifically, the 300 mg ifetroban group saw a 1.8% increase in LVEF, while the placebo group saw their LVEF fall by an average of 1.5%.

The biotech also compared the data to "propensity-matched natural history controls." This made the difference "even more pronounced," the company said, as the data from these control patients showed a 3.6% decline in LVEF.

Both doses of ifetroban were well tolerated, with no serious drug-related events, Cumberland noted.

Ifetroban was developed by Bristol Myers Squibb as a cardiovascular drug. After conducting some phase 2 trials of its own, the pharma decided to donate the program to Vanderbilt University. Cumberland acquired the program from the university in 2011.

In a statement, Jonathan Soslow, M.D., a professor of pediatrics at Vanderbilt, said the cardiac imaging data from this morning's trial results were "compelling."

"The preservation and even improvement in cardiac function seen with ifetroban treatment stands in stark contrast to the expected decline we typically observe in untreated DMD patients," Soslow said in Cumberland's release.

Now, Cumberland said it is analyzing the data while it prepares for an end-of-phase 2 meeting with the FDA "to determine next steps associated with the product's development and commercialization."

"These results validate our commitment to developing innovative treatments for rare diseases and underscore the importance of collaborative partnerships between industry, academia, and regulatory agencies in addressing critical unmet medical needs," Cumberland CEO A.J. Kazimi said in a statement.