Managed Healthcare EXECUTIVE

Cumberland Pharmaceuticals Reports Promising Results Itetroban for DMD Patients

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News Article

The company is evaluating whether the drug, which targets cardiac complications, might be used treat patients with other diseases, such as systematic sclerosis.

Cumberland Pharmaceuticals, a specialty pharmaceutical company has reported encouraging top-line findings from its Phase 2 FIGHT DMD trial.

In the study, the Nashville-based company assessed ifetroban, an innovative oral treatment for heart disease associated with Duchenne muscular dystrophy (DMD), which is the primary cause of mortality among DMD patients. Ifetroban is a once-daily oral medication that works by blocking the thromboxane receptor, which plays a key role in inflammation and fibrosis.

“The FIGHT DMD trial results represent a breakthrough for DMD patients because it’s the first successful Phase 2 study specifically targeting cardiac complications —the leading cause of death in this population,” a spokesperson for Cumberland Pharmaceuticals told *MHE* in an email. “The study demonstrated that ifetroban not only prevented the typical decline in heart function but actually improved cardiac function, with high-dose treatment showing a 3.3% improvement in LVEF [left ventricular ejection fraction] compared to placebo’s 1.5% decline.”

In fact, when compared with age-matched, background-therapy-matched and LVEF-at-baseline-matched natural history controls, the benefit was even more pronounced, showing a 5.4% improvement versus a 3.6% decline. Even more importantly, the treatment was well-tolerated with no serious drug-related events.

As a once-daily oral medication, ifetroban offers a practical treatment option that could meaningfully alter the course of cardiac complications in DMD patients.

“This addresses a critical unmet medical need, as current treatments can help manage some DMD symptoms but don't specifically target the cardiac complications,” the spokesperson said.

A prior study at Vanderbilt University Medical Center indicated that ifetroban offers protection against cardiomyopathy in various preclinical models of muscular dystrophy. The findings from that research were published in the *Journal of the American Heart Association*.

Building on these encouraging results, Cumberland Pharmaceuticals became the first recipient of a clinical trial grant from the FDA Office of Orphan Products Development for Duchenne muscular dystrophy.

The company continues to collect long-term data through the Phase 2 study’s optional open-label extension phase.

“These additional results will be analyzed and shared when available, providing valuable insights into ifetroban's long-term safety and efficacy profile in DMD patients,” the spokesperson said.

Up ahead, Cumberland Pharmaceuticals plans to conduct further data analysis and complete a full study report in preparation for an end-of-Phase-2 meeting with the FDA.

“The company has secured a growing portfolio of patents for the DMD indication, positioning us well for future development,” the spokesperson said. “The end-of-Phase-2 meeting will be crucial for determining the next steps for product development and commercialization strategy.”

While the primary focus remains on DMD-related heart disease, Cumberland is already exploring ifetroban's potential in other conditions.

“We currently have Phase 2 clinical studies underway evaluating ifetroban in patients with systemic sclerosis and idiopathic pulmonary fibrosis, demonstrating the broader potential applications of this therapy,” the spokesperson said.