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Cumberland Pharmaceuticals granted Orphan Drug Designation and Rare Pediatric Disease Designation for Ifetroban

The drug could be used to treat Duchenne muscular dystrophy, a devastating genetic disorder affecting young boys.

Cumberland Pharmaceuticals Inc., a specialty pharmaceutical company based in Nashville, has announced that the U.S. Food and Drug Administration (FDA) granted Orphan Drug Designation and Rare Pediatric Disease Designation to Ifetroban for the treatment of cardiomyopathy associated with Duchenne muscular dystrophy (DMD).

Cumberland is completing the **FIGHT DMD™ trial**, a multicenter, double-blind, placebo-controlled Phase II study investigating the pharmacokinetics, safety, and efficacy of once-daily oral Ifetroban in patients with DMD, a rare and fatal genetic disorder which affects about 1 in every 3,300 male births worldwide. Characterized by mutations in the gene responsible for producing dystrophin, DMD causes damage to the skeletal and cardiac muscle of DMD patients, with cardiomyopathy recognized as the primary cause of death. Ifetroban is being evaluated to treat the heart disease associated with DMD, which has received limited attention with current therapies.

Results of the double-blind trial will be announced later this year.

"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," said **A.J. Kazimi**, Chief Executive Officer of Cumberland Pharmaceuticals. "These designations not only recognize the urgent need for effective treatments but also provide vital support to accelerate research and development. These important regulatory milestones represent hope for families and a pathway to bring transformative medicines to a vulnerable patient population more quickly and efficiently."

The FDA grants Rare Pediatric Disease Designation to incentivize drug development for life-threatening diseases affecting less than 200,000 children in the U.S. As these diseases pose unique challenges to drug development, special focus is needed to provide therapeutic options to these children. Companies that receive approval for a drug or biologic with this designation may be eligible for a voucher which may be redeemed for priority review of a different product. Additionally, this voucher may be transferred or sold to another person.