Cumberland Pharmaceuticals Shares Phase 2 FIGHT DMD Trial Results at the Parent Project Muscular Dystrophy Annual Conference

New pharmacokinetic and cardiac biomarker data further demonstrate ifetroban's potential to protect the heart and reduce cardiac damage in DMD patients

NASHVILLE, Tenn. (June 23, 2025) - Cumberland Pharmaceuticals Inc. (NASDAQ: CPIX), a specialty pharmaceutical company committed to developing new products for rare diseases, shared the latest positive results from its Phase 2 FIGHT DMD trial evaluating ifetroban, a novel oral therapy for Duchenne muscular dystrophy (DMD) heart disease, at the annual Parent Project Muscular Dystrophy (PPMD) conference in Las Vegas. These new results highlight multiple indicators of cardiac benefit with ifetroban treatment in DMD heart disease, the leading cause of death in DMD, including previously unreported pharmacokinetic findings and cardiac biomarker data that demonstrate the drug's potential to prevent ongoing heart damage.

The trial results were presented by FIGHT DMD Trial Principal Investigator Larry W. Markham, MD, Professor of Pediatrics and Medicine at Riley Children's Hospital and the Indiana University School of Medicine, as part of the "*Therapies that Slow Progression*" conference session. "PPMD has had a focus on heart disease and has been a part of this trial since its inception. We are proud to share the latest promising findings from the FIGHT DMD Trial with a group that has consistently supported us throughout our efforts to develop an effective treatment for DMD heart disease," said Dr. Markham. The complete slide presentation is now available on Cumberland's website.

"Seeing these promising results validates our belief that targeted heart therapies can make a meaningful difference for our children," said Terry Marlin, President and Founder of FIGHTDMD, a community-based organization with the mission of advancing DMD research. "This trial represents what's possible when families refuse to accept the status quo and invest in advancing medical research."

FIGHTDMD helped co-fund the preclinical studies at Vanderbilt's Monroe Carell Jr. Children's Hospital which formed the foundation for the FDA grant funding this trial. The trial was named The FIGHT DMD Trial in honor of their organization's early support. The Marlin family also provided input into the clinical trial design and contributed personal photos for the trial's patient brochure.

The 12-month Phase 2 FIGHT DMD trial (NCT03340675) demonstrated that high-dose ifetroban treatment resulted in a significant 5.4% improvement in left ventricular ejection fraction (LVEF) compared to a control group composed of placebo-treated patients combined with propensity score-matched natural history patients. This represents a clinically meaningful difference in a progressive disease where heart function typically declines over time.

Critically, high-dose ifetroban treatment was associated with reduced blood levels of cardiac damage markers (NT-proBNP and cardiac troponin I), while these markers of heart damage increased in placebo-treated patients, suggesting ifetroban could help prevent ongoing cardiac injury. This biochemical evidence of reduced cardiac damage, combined with functional heart improvement, provides compelling evidence of ifetroban's cardioprotective effects.

The study also revealed pharmacokinetic insights, showing that DMD patients receiving higher doses than typical adults achieved similar plasma levels, with no evidence of drug accumulation, supporting the 300 mg daily dosing used in the high-dose group. Despite the higher dosing requirements, ifetroban was well-tolerated with an acceptable pharmacokinetic profile in patients with DMD.

The observed improvement in cardiac function together with the reduction in cardiac damage biomarkers suggest a clinically significant impact for ifetroban in both heart disease and potential to impact overall survival in DMD. Demonstrating confidence in the treatment, all patients who completed the 12-month study opted to continue with the open-label extension.

"These new results reinforce our conviction that ifetroban has the potential to address the leading cause of death in DMD patients," said A.J. Kazimi, Cumberland CEO. "The improvement in heart function observed, combined with the reduction in cardiac damage markers, suggests we may be able to meaningfully impact the progressive heart disease that affects virtually all DMD patients. What's particularly encouraging is that there is both functional improvement and biochemical evidence of reduced cardiac injury - exactly what you'd want to see in a therapy designed to protect the heart. We're deeply grateful to both Terry Marlin and FIGHT DMD for their early vision and funding and to PPMD for their longstanding support and partnership throughout this journey – as both organizations have been instrumental in making this breakthrough possible. We're optimistic about taking the next steps toward bringing this much-needed treatment to DMD patients and their families."

Ifetroban is a once-daily oral medication that works by blocking the thromboxane receptor, which plays a key role in inflammation and fibrosis. The drug has received both Orphan Drug Designation Rare Pediatric Disease Designation, from the FDA for the indication of DMD heart disease. There is currently no approved treatment specifically targeting DMD heart disease, highlighting the critical unmet medical need in this patient population where cardiac complications are universal and represent the leading cause of death.

Cumberland has secured a growing portfolio of patents protecting the product for this DMD heart disease indication. Next steps include the analysis of long-term treatment results and discussions with the FDA to determine the regulatory pathway forward based on these encouraging results.

More information regarding The FIGHT DMD Trial can be found here: www.fightdmdtrial.com

About Duchenne Muscular Dystrophy (DMD)

DMD is a rare and incurable pediatric disease caused by mutations in the gene encoding dystrophin, a protein critical for muscle function, including the heart. Patients with DMD slowly lose muscle function, resulting in the inability to walk, difficulty breathing, and heart failure. While current treatments can help manage some DMD symptoms, there are no approved therapies specifically targeting DMD-related heart disease, highlighting a critical unmet medical need.

About Parent Project Muscular Dystrophy (PPMD)

Parent Project Muscular Dystrophy is a grassroots, parent-led advocacy group with the mission to end DMD. Since its founding in 1994, PPMD has helped to accelerate treatments through research funding, to provide access to optimal DMD care for families, and to affect legislation through advocacy to improve the lives of children with DMD and their families. PPMD also hosts an annual conference which is the largest, most comprehensive, annual international conference focused entirely on DMD. This conference connects families across the world to share their stories and serves as a forum to highlight the progress in ending DMD.

About FIGHT DMD

FIGHT DMD is a community-based organization founded by Terry and Sonya Marlin with the mission of advancing research into DMD heart disease. The organization has provided crucial early-stage funding for multiple research projects focused on understanding and treating cardiac complications in DMD patients. Their support has been instrumental in advancing preclinical research that led to FDA grant funding for clinical trials, demonstrating the power of family-driven advocacy in catalyzing medical innovation.

About Cumberland Pharmaceuticals

Cumberland Pharmaceuticals Inc. is the largest biopharmaceutical company founded and headquartered in Tennessee and is focused on providing unique products that improve the quality of patient care. The company develops, acquires, and commercializes products for the hospital acute care, gastroenterology and oncology market segments. The company's portfolio of FDA-approved brands includes:

- **Acetadote®** (*acetylcysteine*) injection, for the treatment of acetaminophen poisoning;
- **Caldolor®** (*ibuprofen*) injection, for the treatment of pain and fever;
- **Kristalose®** (*lactulose*) oral, a prescription laxative, for the treatment of constipation;
- **Sancuso®** (*granisetron*) transdermal, for the prevention of nausea and vomiting in patients receiving certain types of chemotherapy treatment;
- **Vaprisol®** (*conivaptan*) injection, to raise serum sodium levels in hospitalized patients with euvolemic and hypervolemic hyponatremia; and
- **Vibativ®** (*telavancin*) injection, for the treatment of certain serious bacterial infections including hospital-acquired and ventilator-associated bacterial pneumonia, as well as complicated skin and skin structure infections.

The company also has a series of Phase II clinical programs underway evaluating its ifetroban product candidate in patients with cardiomyopathy associated with Duchenne Muscular Dystrophy, Systemic Sclerosis and Idiopathic Pulmonary Fibrosis.

For more information on Cumberland's approved products, including full prescribing information, please visit the individual product websites, which can be found on the company's website www.cumberlandpharma.com.

Forward-Looking Statements

This press release contains forward-looking statements, which are subject to certain risks and reflect Cumberland's current views on future events based on what it believes are reasonable assumptions. No assurance can be given that these events will occur. Forward-looking statements include, among other things, statements regarding the company's intent, belief or expectations, and can be identified by the use of terminology such as "may," "will," "expect," "believe," "intend," "plan," "estimate," "should," "seek," "anticipate," "look forward" and other comparable terms or the negative thereof. As with any business, all phases of Cumberland's operations are subject to factors outside of its control, and any one or combination of these factors could materially affect Cumberland's operation results. These factors include macroeconomic conditions, including rising interest rates and inflation, competition, an inability of manufacturers to produce Cumberland's products on a timely basis, failure of manufacturers to comply with regulations applicable to pharmaceutical manufacturers, natural disasters, public health epidemics, maintaining an effective sales and marketing infrastructure, and other events beyond the company's control as more fully discussed in its most recent annual report on Form 10-K as filed with the U.S. Securities and Exchange Commission ("SEC"), as well as the company's other filings with the SEC from time to time. There can be no assurance that results anticipated by the company will be realized or that they will have the expected effects. Readers are cautioned not to place undue reliance on forward-looking statements, which speak only as of the date hereof. The company does not undertake any obligation to publicly revise these statements to reflect events after the date hereof.

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