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PRESS RELEASE

Cumberland Pharmaceuticals Announces Breakthrough Results from the Phase 2 FIGHT DMD Trial in Duchenne Muscular Dystrophy Heart Disease

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PR Newswire

NASHVILLE, Tenn., Feb. 4, 2025

NASHVILLE, Tenn., Feb. 4, 2025 /PRNewswire/ -- Cumberland Pharmaceuticals Inc. (Nasdaq: CPIX), a specialty pharmaceutical company with development efforts focused on new products for rare diseases, today announced positive top-line results from its Phase 2 FIGHT DMD trial. The study evaluated ifetroban, a novel oral therapy for Duchenne muscular dystrophy (DMD) heart disease -- the leading cause of death in DMD patients. It marks a breakthrough for these patients, as it's the first successful Phase 2 study specifically targeting the cardiac complications of their condition.

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.

A previous study conducted at Vanderbilt University Medical Center demonstrated that ifetroban is protective against cardiomyopathy in several preclinical models of muscular dystrophy. The results of that study were published in the Journal of the American Heart Association (West 2019). Based on those promising results, Cumberland Pharmaceuticals became the first recipient of an FDA Office of Orphan Products Development clinical trial grant for DMD, funding the development of this Phase 2 clinical trial.

"These results represent a significant milestone in DMD cardiomyopathy," said 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. "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."

The FIGHT DMD trial (NCT03340675), is a 12-month, double-blind, randomized, placebo-controlled study evaluating ifetroban, an oral thromboxane receptor antagonist. The trial enrolled 41 DMD patients who received either low dose ifetroban (150 mg per day), high dose ifetroban (300 mg per day), or placebo. The study's primary endpoint was an improvement in the heart's left ventricular ejection fractions (LVEF).

Key findings include:

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   -- High dose ifetroban treatment resulted in an overall
3.3% improvement in
      LVEF.

   -- The high dose ifetroban group showed an increase in
1.8% in LVEF, while
      the study placebo group showed an expected decline in
LVEF of 1.5%

   -- When compared with propensity matched natural history
controls, the
      difference was even more pronounced, with the high
dose treatment
      providing a significant 5.4% overall improvement in
LVEF, as the control
      patients experienced a 3.6% decline in LVEF

   -- Both doses of ifetroban were well-tolerated, with no
serious drug-related
      events.
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"The cardiac imaging data from this trial is compelling," noted Jonathan Soslow, MD, Professor of Pediatrics, Vanderbilt University, pediatric cardiologist and cardiac imaging expert. "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."

"This trial represents hope for our Duchenne community," said Pat Furlong, Founding President and CEO of Parent Project Muscular Dystrophy. "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."

"These impressive results represent a pivotal moment for Cumberland Pharmaceuticals and, more importantly, for the DMD community," said A.J. Kazimi, Chief Executive Officer of Cumberland Pharmaceuticals. "As the first company to receive FDA Orphan Products Development funding for a DMD clinical trial, we're honored to be advancing a potential breakthrough therapy for DMD-related heart disease. 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."

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 and Rare Pediatric Disease Designation from the FDA, highlighting its potential significance in treating this devastating condition. If approved, ifetroban would be the first therapy specifically indicated for DMD-related heart disease.

Cumberland has secured a growing portfolio of patents with claims associated with the product for this DMD indication. Next steps include further data analysis and completion of a full study report in preparation for an end of Phase 2 meeting with the FDA to determine next steps associated with the product's development and commercialization.

More information regarding the FIGHT DMD trial can be found here: www.fightdmdtrial.com

References:

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West JD, Galindo CL, Kim K, et al. Antagonism of the thromboxane-prostanoid receptor as a potential therapy for cardiomyopathy of muscular dystrophy. J Am Heart Assoc. 2019;8(21):e011902. doi: 10.1161/JAHA.118.011902.

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(R) (acetylcysteine) injection, for the treatment of acetaminophen poisoning;

-- Caldolor(R) (ibuprofen) injection, for the treatment of pain and fever;

-- Kristalose(R) (lactulose) oral, a prescription laxative, for the treatment of constipation;

-- Sancuso(R) (granisetron) transdermal, for the prevention of nausea and vomiting in patients receiving certain types of chemotherapy treatment;

-- Vaprisol(R) (conivaptan) injection, to raise serum sodium levels in hospitalized patients with euvolemic and hypervolemic hyponatremia; and

-- Vibativ(R) (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.

In addition to this Duchenne muscular dystrophy program, the company also has Phase 2 clinical studies underway evaluating its ifetroban product candidate in patients with 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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