Cumberland Pharmaceuticals Receives FDA Orphan Drug And Rare Pediatric Disease Designations For New Treatment Of Duchenne Muscular Dystrophy

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NASHVILLE, Tenn., Nov. 6, 2024 /PRNewswire/ -- **Cumberland Pharmaceuticals Inc. (NASDAQ: CPIX)**, a specialty pharmaceutical company, announced today that the United States (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. Results 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 U.S. 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 sponsor.

Orphan Drug Designation is granted by the U.S. FDA to encourage development of new therapies for rare diseases or conditions affecting fewer than 200,000 people in the U.S. This designation offers sponsors multiple incentives, which include exemption from user fees, tax credits for qualified clinical trials and potential market exclusivity for seven years following approval of the product.

About Duchenne Muscular Dystrophy

DMD is 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.

About Ifetroban

Ifetroban is a potent and selective thromboxane-prostanoid receptor (TPr) antagonist. Ifetroban exhibits high affinity for TPr on many cell types including cardiomyocytes, platelets, vascular and airway smooth muscle, and fibroblasts, and lacks agonistic activity. In several preclinical models of muscular dystrophy, including limb-girdle and Duchenne, ifetroban prevented cardiac fibrosis and cardiac dysfunction and improved mortality.

Cumberland previously announced the acquisition of the ifetroban program in collaboration with Vanderbilt University and Cumberland Emerging Technologies.

Cumberland is also evaluating ifetroban for systemic sclerosis and pulmonary fibrosis. The ongoing FIGHTING FIBROSIS Trial is a multicenter, randomized placebo-controlled Phase II study in patients with idiopathic pulmonary fibrosis (IPF), a progressive interstitial lung disease. IPF is marked by inflammation and fibrosis of the lungs, resulting in rapidly declining lung function and reduced survival within five years of diagnosis. The study is evaluating the safety and efficacy of once daily oral Ifetroban for 52 weeks.

About Cumberland Pharmaceuticals

Cumberland Pharmaceuticals Inc. is a specialty pharmaceutical company focused on the delivery of high-quality prescription brands to improve patient care. The Company develops, acquires, and commercializes brands for the hospital acute care, gastroenterology and rheumatology 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;
- **Vaprisol®** (*conivaptan*) Injection, to raise serum sodium levels in hospitalized patients with euvolemic and hypervolemic hyponatremia;

- **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;
- **Kristalose®** (*lactulose*) for Oral Solution, a prescription laxative, for the treatment of chronic and acute constipation; and
- **Sancuso®** (*granisetron*) Transdermal delivery system, for the treatment of nausea and vomiting associated with chemotherapy.

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

About Cumberland Emerging Technologies

Cumberland Emerging Technologies, Inc. (www.cet-fund.com) is a joint initiative between Cumberland Pharmaceuticals Inc., Vanderbilt University, Launch Tennessee and WinHealth Pharma. The mission of CET is to bring biomedical technologies and products conceived at Vanderbilt and other regional research centers to the marketplace. CET helps manage the development and commercialization process for select projects and provides expertise on intellectual property, regulatory, manufacturing and marketing issues that are critical to successful new biomedical products. CET's Life Sciences Center, located in Nashville, Tennessee, provides laboratory space, equipment and infrastructure to early-stage life sciences companies.

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. 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 results of operations. These factors include market conditions, competition, an inability of manufacturers to produce Cumberland's products on a timely basis or failure of manufacturers to comply with regulations applicable to pharmaceutical manufacturers, maintaining an effective sales and marketing infrastructure, natural disasters, public health epidemics, and other events beyond our control, as more fully discussed in the Company's most recent Form 10-K and subsequent 10-Qs as filed with the SEC. 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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