Marathon Pharmaceuticals' U.S. Expanded Access Program Providing Deflazacort for Duchenne Muscular Dystrophy (DMD) Reaches New Milestone

10 U.S. Centers now eligible for patient enrollment in ACCESS DMD™ to receive deflazacort, an investigational drug for DMD, at no cost through the program.

Northbrook, Ill. – February 24, 2016 – Marathon Pharmaceuticals announced today that 10 centers throughout the United States are now participating in an open-label, expanded access program (EAP) called ACCESS DMD™ to provide deflazacort to qualified patients with Duchenne muscular dystrophy (DMD) under U.S. Food and Drug (FDA) Administration authorization. An additional 10 centers are expected to be approved by the end of April, 2016.

The approved Centers are located in Little Rock, AR; Chicago, IL; Kansas City, KS; Boston, MA; Charlotte, NC; Hershey, PA; Memphis, TN; Houston, TX; Norfolk, VA and Seattle, WA.

Deflazacort is an investigational therapy in the U.S. and has not been approved by the FDA. Patients will be able to participate in the ACCESS DMD™ expanded access program at no cost until deflazacort is approved. Expanded access programs provide a mechanism for early access to an investigational drug to treat patients with a serious or immediately life-threatening disease or condition that has no comparable or satisfactory alternative treatment options.

DMD is a recessive X-linked form of muscular dystrophy, which results in muscle degeneration, difficulty walking and loss of independent ambulation, impaired pulmonary and cardiac function, and ultimately death.[1] There are currently no therapies approved in the U.S. or other countries to treat DMD.

Marathon expects to submit a New Drug Application for deflazacort in May of 2016, which will be subject to review by the FDA. If approved, deflazacort is expected to be made commercially available in the U.S. in January of 2017.

Patients, families and physicians can learn more about ACCESS DMD™, including a list of clinical sites participating in the program, by visiting http://www.AccessDMD.com or calling 1-844-800-4DMD (4363). Marathon is working with patient advocacy groups including Charley's Fund, Coalition Duchenne, CureDuchenne, Foundation to Eradicate Duchenne, the Jett Foundation, Michael's Cause, Muscular Dystrophy Association, Parent Project Muscular Dystrophy and Ryan's Quest to raise awareness of ACCESS DMD. Marathon will continue its outreach in an effort to connect with all the patient groups who are part of the DMD community.

"We have received great feedback on this program from the physicians and clinics who specialize in treating DMD and we are proud to help patients in need of the medication, through this EAP," said Jeff Aronin, CEO of Marathon Pharmaceuticals. "The ACCESS DMD Expanded Access Program is an important part of Marathon's commitment to serving patients."

About Marathon Pharmaceuticals

Marathon Pharmaceuticals, LLC, is a biopharmaceutical company that develops new treatments for rare diseases with a focus on providing medicine to patients who currently have no treatment options. The company is developing a pipeline of treatments for rare neurological and movement disorders. Marathon is headquartered in Northbrook, Illinois, with offices in Chicago, New Jersey and Washington D.C. For more information, visit www.marathonpharma.com.

About Deflazacort

Deflazacort is a glucocorticoid with anti-inflammatory and immunosuppressant properties.[3] Based on published clinical studies, it appears that deflazacort may be an important new treatment option for patients with DMD.[4,5] Side effects reported to date include cushingoid appearance, hirsutism, weight gain, erythema, nasopharyngitis, irritability and cataract formation.

Deflazacort is currently not approved in the U.S. but is available outside the U.S. for many approved uses not including DMD. The FDA previously granted Fast Track status, Orphan Drug designation and Rare Pediatric Disease designation for deflazacort for the treatment of patients with DMD.

About Duchenne Muscular Dystrophy

DMD occurs as a result of mutations (mainly deletions) in the dystrophin gene.[1] These mutations lead to an absence of or defect in the protein dystrophin, which results in progressive muscle degeneration. The incidence is approximately 1 in 3,500 live male births.[6] There is currently no cure for DMD.[1,2,7] Treatment is generally aimed at controlling symptoms to maximize the quality of life.

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