COLLABORATIVE
TRAJECTORY
ANALYSIS
PROJECT

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Marathon Pharmaceuticals and Italfarmaco Join cTAP: Learning from Patient Data to Enhance Clinical Trial Design for Duchenne Muscular Dystrophy

Both companies have investigational treatments for Duchenne in late-stage clinical development

CAMBRIDGE, Mass., Feb. 7, 2017 (Business Wire) – Marathon Pharmaceuticals LLC and Italfarmaco SpA have joined the Collaborative Trajectory Analysis Project (cTAP), bringing their extensive knowledge and clinical expertise in Duchenne muscular dystrophy to the rapidly growing coalition.

Reflecting increased excitement about and participation in cTAP's unique collaborative structure, the new members bring the number of drug developers who have chosen to join the coalition to 10.

"We are thrilled to welcome these two major drug developers to cTAP," said Susan J. Ward, Ph.D., co-founder and executive director. "Our mission is to empower drug developers to design clinical trials in Duchenne that will generate a clear and unambiguous assessment of an investigational therapy. The cTAP team is excited to help Marathon and Italfarmaco benefit from cTAP analytic tools and research, which are based on the largest collection of harmonized patient data in the world, to advance their programs."

Duchenne muscular dystrophy is a progressive, fatal disease of boys characterized by gradual weakening of muscles. It is the most common fatal genetic disorder diagnosed in childhood. Most Duchenne patients die in their twenties.

Marathon, a private company based in Northbrook, Ill., is developing the investigational drug deflazacort in a variety of different forms for the treatment of Duchenne muscular dystrophy. In August 2016, the FDA accepted the New Drug Application (NDA) for deflazacort for both filing and for Priority Review.

“We are excited to join cTAP and contribute to this collaborative effort to advance research for new treatments and an ultimate cure for Duchenne muscular dystrophy.,” said Dr. Rick Munschauer, chief medical officer at Marathon. “Collaboration is a core value for Marathon. By working together with other researchers and the patient community, cTAP members are helping to accelerate the development of treatments.”

Paolo Bettica, Italfarmaco’s vice president of research and development, said, “Membership in cTAP will help us optimize the design of our clinical trials and improve our data analysis as we move Givinostat into pivotal trials for the treatment of Duchenne patients. We are proud to join Professor Eugenio Mercuri and other leaders in Duchenne research from Italy as we together seek to solve the common problems that has largely thwarted development of treatments for all Duchenne patients.”

Givinostat, discovered at Italfarmaco, has been shown in clinical trials to prevent muscle fibers being replaced with scar tissue in Duchenne patients. The privately held company was founded in 1938 and is a multinational leader in pharmaceuticals and manufacturing of active ingredients.

Dr. Brenda Wong, director of the Comprehensive Neuromuscular Center at the University of Cincinnati Medical Center and one of cTAP’s clinical experts in Duchenne, said, “The value of the cTAP collaboration grows as more members join from different parts of the Duchenne community. I look forward to working with new colleagues from Marathon Pharmaceuticals and Italfarmaco, and learning from their unique experiences in Duchenne research. With these new members, 2017 promises to be a year of sustained progress in the fight against Duchenne.”

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About cTAP

The Collaborative Trajectory Analysis Project, or cTAP, is enabling clinical experts to solve the most critical problems in drug development for Duchenne muscular dystrophy. The first community-wide coalition in Duchenne, cTAP has forged a dynamic alliance between clinical experts, drug companies developing therapies, patient advocacy organizations and collaborating registries and clinical centers across Europe and the US. Driven by a shared mission to overcome the challenges of developing drugs for a disease characterized by heterogeneous progression, cTAP brings advanced data science to the fight against Duchenne through a partnership with outcomes research experts at Analysis Group Inc (http://www.analysisgroup.com/). cTAP is curating and growing what is already the largest natural history database of patient data in Duchenne. This rich resource enables cTAP to develop solutions with the urgency necessary to enhance clinical trial design and analysis, near-term.
http://ctap-duchenne.org

About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy is a uniformly fatal, progressive muscle-wasting disease affecting about one in 3,500-6,000 male live births. Patients with Duchenne lack the ability to make dystrophin, a protein crucial to muscle function. As their muscles deteriorate, they progressively lose the ability to walk, feed themselves, turn over in bed, and ultimately to breathe. While there is no cure, the past decade has seen an explosion in research resulting in more than 15 therapies entering clinical development, with some receiving limited approval. Learn more about Duchenne at cureduchenne.org and parentprojectmd.org.

ABOUT DEFLAZACORT

DEFLAZACORT is a corticosteroid under FDA review for the treatment of Duchenne muscular dystrophy.

Deflazacort is not for patients who are allergic to deflazacort or any of the inactive ingredients in deflazacort.
Patients should not stop taking deflazacort, or change the amount they are taking, without first checking with their healthcare provider, as there may be a need for gradual dose reduction to decrease the risk of serious side effects.
Corticosteroids, such as deflazacort can cause:

- Hyperglycemia, altered glucose metabolism
- Increased risk of infection
- Changes in cardiovascular/kidney function that could lead to increases in blood pressure, salt, and water retention, and decreases in blood levels of potassium and calcium
- Behavioral and mood changes that could lead to potentially severe psychiatric adverse reactions
- Osteoporosis, decrease in bone mineral density
- Serious skin rashes
- May slow growth and development
- Cataracts or glaucoma

Vaccinations: The administration of live or live attenuated vaccines are not recommended while taking deflazacort. Killed or inactivated vaccines may be administered, but the responses cannot be predicted. Patients should discuss their vaccine history with their healthcare provider before starting deflazacort, and while taking deflazacort, patients should check with their healthcare provider before receiving any new vaccines or booster shots.

Common side effects that could occur with deflazacort include: Facial puffiness or Cushingoid appearance, skin redness, unwanted hair growth, weight gain, headache, central obesity, increased appetite, frequent daytime urination, constipation and abdominal pain.

Patients should tell their healthcare provider if they have had recent or ongoing infections, develop a fever, or experience any other side effects.