Muscular Dystrophy Canada commends US on the approval of Drug for Duchenne Muscular Dystrophy

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Toronto, Canada –Thursday, February 9, 2017 was an historic day for American Duchenne patients and their families. The US Food and Drug Administration (FDA) approved EMFLAZA™ (deflazacort). This is the first approval of a steroid for Duchenne and a therapy that applies to people with Duchenne five (5) years of age and older, regardless of genetic mutation.

"This is an exciting time in the world of neuromuscular disorders, like Duchenne. For the first ever, new drugs and treatments are coming to market and showing great promise. This is a result of the funds invested, by organizations like Muscular Dystrophy Canada, in neuromuscular research." commented Barbara Stead-Coyle, CEO of Muscular Dystrophy Canada.

Emflaza ™, developed by Marathon Pharmaceuticals, is a corticosteroid that demonstrates anti-inflammatory and immunosuppressant effects. In clinical studies, those treated with Emflaza ™ showed improved muscle strength and a slower decline in functional ability.

Duchenne muscular dystrophy is a genetic disorder, which usually affects boys (it is very rare in girls). Signs of weakness start when the boys are between 3 and 5 years of age - sometimes earlier. At first, the weakness is seen mostly in the legs and hips. The muscles become weaker as the boys get older. This is because the body cannot make the muscle protein called dystrophin. Eventually this weakness makes walking more difficult and a wheelchair is needed. Gradually, all the muscles become very weak, including the muscles used for breathing and the heart.

Corticosteroids, including prednisone and deflazacort (Emflaza ™), are used to slow down the progression of Duchenne muscular dystrophy. This medication allows boys and young men to walk for longer and can delay the need for a wheelchair by years. Corticosteroids reduce the likelihood of developing scoliosis (curvature of the spine) and improve lung and heart function.

"Deflazacort has been used for decades in many countries, including the European Union. Some physicians and families believe that deflazacort may be associated with less weight gain than prednisone, which is particularly important for people who already have weak muscles," commented Dr. Hugh McMillan, a Clinical Investigator and physician at the Children's Hospital of Eastern Ontario (CHEO).

"At this time, deflazacort is not approved for use in Canada. It can be accessed under a Special Access Program. Deflazacort is not covered under most private or public (provincial) insurance programs. This added expense is a barrier for many Canadian families who might otherwise have wanted to use this medication."

The recent approval of deflazacort by the US Food and Drug Administration (FDA) is encouraging and raises hopes that Canadian children may also have access to this medication in the near future.

About Muscular Dystrophy Canada
Muscular Dystrophy Canada's mission is to enhance the lives of those affected with neuromuscular disorders by continually working to provide ongoing support and resources while relentlessly searching for a cure through well funded research. To learn more about Muscular Dystrophy Canada – please visit www.muscle.ca or call our toll-free number at 1-866-MUSCLE-8 (1-866-687-2538).

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