MedicalXpress

Emflaza approved for duchenne muscular dystrophy

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(HealthDay) —Emflaza (deflazacort) has been approved by the U.S. Food and Drug Administration to treat Duchenne muscular dystrophy in people five years and older, the agency said Thursday in a news release.

The corticosteroid is designed to reduce inflammation and suppress the immune system in people with DMD, the most common type of muscular dystrophy. The FDA said it's the first corticosteroid approved to treat DMD.

The genetic disorder leads to ongoing deterioration of the muscles. It's caused by a lack of dystrophin, a protein designed to keep muscle cells intact. Symptoms commonly begin between ages three and five, and boys are affected much more often than girls. The disorder affects about one of every 3,600 male infants worldwide, the agency said.

Most people with DMD require a wheelchair by their early teens. The majority die in their 20s and 30s, the FDA said, although life expectancy varies.

Emflaza was evaluated in a clinical study of 196 males aged five to 15, all with documented mutation of the dystrophin gene and onset of DMD symptoms before age five. After 12 weeks, people taking the drug showed muscle strength improvement, compared with those who took a placebo.

Side effects, similar to those caused by other corticosteroid medications,

included facial puffiness, weight gain, increased appetite, upper respiratory tract infection, cough and increased need to urinate, the FDA said.

Emflaza is marketed by Marathon Pharmaceuticals, based in Northbrook, Ill.

More information: Learn more from the FDA.