Parent Project Muscular Dystrophy
JOIN THE FIGHT. END DUCHENNE.

May 21, 2021

State of West Virginia
Department of Health and Human Resources
Drug Utilization Review Board

To Whom It May Concern,

On behalf of Parent Project Muscular Dystrophy (PPMD) and Americans who live with the devastating diagnosis of Duchenne muscular dystrophy, we are writing today to urge you to support coverage for access to FDA approved therapies aimed at treating Duchenne muscular dystrophy, including **Emflaza,** which is under review by the DUR Board. **PPMD believes therapies for Duchenne should never be restricted for access based on age, function, or stage of disease.** Strict requirements for prior authorization and reauthorization should not limit access for patients in dire need of these therapies. In a slow, progressive, debilitating disease like Duchenne, every day marks another day of muscle cell death, patients should have access to these medications without delay.

Parent Project Muscular Dystrophy (PPMD) is the nation's leading patient advocacy organization dedicated to ending Duchenne. As you may know, Duchenne muscular dystrophy is a universally fatal, genetic disorder that affects approximately 1 in 5,000 live male births. People with Duchenne face a relentless deterioration of muscle strength leading to loss of mobility followed by severe cardiac and respiratory compromise in early adulthood. There is no escape.

PPMD has considerable concerns over the policy for **Emflaza**, requiring the following criteria ALL be met: *A documented history of at least 12-months continuous therapy with prednisone; AND 2) Documentation indicating the patient experienced significant adverse effects associated with prednisone therapy; AND 3) request must be accompanied with a baseline 6-minute walk distance (6MWD); AND 4) Initial authorizations shall be for 90 days. Continuation requests may be granted a 12-month approval if significant improvement is demonstrated in either the patient's adverse effect profile or 6MWD.*

We do not believe ambulation should be used as a criteria for continued use of Emflaza as current published care guidelines in Duchenne recommend use of steroids throughout the disease progression[1]. Steroids are aimed at slowing disease progression. Furthermore, the FDA label for Emflaza does not restrict use to only ambulatory patients[2]. We also believe the 12 month step requirement of prednisone is unnecessary. Published data suggests better outcomes and less side effects with Emflaza (deflazacort) over prednisone[3].

Given the high unmet medical need and identified preferences of the Duchenne community, PPMD strongly believes that the data supporting approval of **Emflaza** is sufficient to warrant coverage for patients who have been provided a prescription by their doctor.

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We thank you for your dedication to the wellbeing of patients and for all you do.

Sincerely,

Pat Furlong
President & CEO
Parent Project Muscular Dystrophy

Ryan Fischer
Chief Advocacy Officer
Parent Project Muscular Dystrophy

References

[1] Birnkrant DJ, Bushby K, Bann CM, Apkon SD, Blackwell A, Brumbaugh D, Case LE, Clemens PR, Hadjiyannakis S, Pandya S, Street N, Tomezsko J, Wagner KR, Ward LM, Weber DR; DMD Care Considerations Working Group. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. Lancet Neurol. 2018 Mar;17(3):251-267. doi: 10.1016/S1474-4422(18)30024-3. Epub 2018 Feb 3. Erratum in: Lancet Neurol. 2018 Apr 4;: PMID: 29395989; PMCID: PMC5869704.

[2] *FDA approves drug to treat Duchenne muscular dystrophy (Emflaza)* https://www.fda.gov/news-events/press-announcements/fda-approves-drug-treat-duchenne-muscular-dystrophy

[3] Griggs RC, Miller JP, Greenberg CR, et al. Efficacy and safety of deflazacort vs prednisone and placebo for Duchenne muscular dystrophy. *Neurology*. 2016;87(20):2123-2131. doi:10.1212/WNL.0000000000003217

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