

The Little Hercules Foundation

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The Little Hercules Foundation is a patient advocacy organization serving patients affected by Duchenne muscular dystrophy (DMD). We appreciate this opportunity to make public comment on the review of Emflaza. DMD is a fast-progressing, muscle wasting, neuromuscular disease diagnosed in childhood around the age of 3-5. Although there is some variability in disease progression, most individuals with DMD lose the ability to walk around age 10-13. There continues to be the need for treatment at all stages of progression, including after loss of ambulation.

We understand that West Virginia Medicaid has a policy for making coverage determinations of non-preferred products. In the case of Emflaza, patients are required to have ALL the following criteria:

- 1) 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.

In our experience with Medicaid policies for Emflaza nationwide, we see these requirements as overly burdensome. A history of Prednisone use of a period of at least 12 months is excessive. Adverse effects are common and can be seen much sooner than a twelve-month period. Adverse effects can be serious and can have a devastating impact on a patient's quality of life and that of their caregivers.

Of all the requirements, that of a 6-minute walk distance is the most concerning to us. The ongoing use of the 6-minute walk test as the only way to prove physical benefit in a disease where a significant number of individuals no longer walk is unacceptable. DMD is a progressive condition, therefore it is expected that patients will lose function even with treatment. The goal of treatment in DMD is to slow progression of this 100% fatal disease, not to reverse it.

We respectfully request that West Virginia Medicaid:

- 1) Lower the documented history of at least 12-months continuous therapy with prednisone to 3 months.
- 2) Consider alternative testing for non-ambulatory patients (e.g. Hammersmith Motor Ability, Medical Research Council muscle strength assessment, Brooke Upper Extremity Function Scale).
- 3) Eliminate the **AND** between time requirement on prednisone (replace with **OR**) side effect documentation.
- 4) Remove the requirement of significant improvement demonstrated on 6MWD for continuation of treatment.

Thank you for your consideration. We are available to discuss these issues affecting our patient community in greater detail with you at any time.

Sincerely,

A handwritten signature in black ink that reads "Amy Aikins".

Amy Aikins
Director, Government and Social Programs
Little Hercules Foundation