

The Little Hercules Foundation

5522 Avery Road, Dublin, Ohio 43016 | 614.389.0026 | littleherculesfoundation.org



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 Amondys 45. 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.

Amondys 45 targets DMD mutations that are amenable to skipping exon 45. Approximately eight percent of the Duchenne population in total have mutations that are amenable to this treatment. We are appreciative of West Virginia's recognition of the need for patients to access treatments approved via the accelerated approval pathway. This is evidenced by your policies on similar treatments approved via this pathway that target the skipping of other exons (Exondys 51 and Vyondys 53, respectively). These treatments allow an exon to be skipped over enabling the body unable to produce dystrophin to produce some. West Virginia's policies on these treatments allow access to the therapies across the spectrum of the disease from ambulatory to non-ambulatory, permitting alternate assessments on a case-by-case basis as necessary in meeting the coverage criteria. It is our sincere hope that your review of Amondys 45 results in similar coverage criteria.

Thank you for your time and consideration. As always, we are available at any time to discuss issues affecting our patient community in greater detail.

Sincerely,



Amy Aikins
Director, Government and Social Programs
Little Hercules Foundation