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 **AMONDYS 53**, which is under review by the DUR Board. PPMD thanks the review board for their favorable coverage criteria for a similar therapy, VYONDYS 53[1], which allows access for both ambulatory and non-ambulatory patients. We believe the same criteria should be applied to AMONDYS 53.

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.

Notwithstanding rising investments in research and development following the 1986 discovery of the Duchenne gene and the protein it specifies, there had been no FDA approved therapy to treat the underlying cause of Duchenne prior to September 2016 when the FDA approved market authorization for EXONDYS 51[2]. Following that, the Duchenne community enthusiastically celebrated FDA approvals of Emflaza[3] (February 9, 2017), VYONDYS 53[4] (December 12, 2019) and more recently VILTEPSO[5] (August 2020) and AMONDYS 45[6](February 2020). The fact that <u>all</u> dystrophin restoration therapies were advanced under the agency's accelerated approval pathway is a clear indication of the **high unmet medical need** in Duchenne.

The FDA package label inserts for drugs approved under accelerated approval including EXONDYS 51[7], VYONDYS 53[8], VILTEPSO[9], and AMONDYS 45[10] provided no restriction on administrating this therapy based on age or disease progression. We strongly recommend coverage for **AMONDYS 45** for all patients who have a confirmed genetic mutation amenable to therapy and have been issued a prescription by a treating physician. This would be in agreement with the Medicaid Drug Rebate Program Notice - For State Technical Contacts (Release No. 185) which states "as with any other drug, if the drug is labeled by a manufacturer that has signed a Medicaid National Drug Rebate Agreement, and the drug meets the definition of covered outpatient drug, then the drug is covered by the Medicaid Drug Rebate Program (MDRP) and is to be covered by state Medicaid programs" [11].

AMONDYS 45

The data contained in the NDA submission for **AMONDYS 45[12]** has met the standard for accelerated approval under 21 CFR 314.510 based on the surrogate endpoint of

increased dystrophin protein production, with the FDA concluding that this surrogate is *reasonably likely to predict* clinical benefit. The goal of these therapies is to slow the progression of the disease, which can be monitored through regular outcome and functional testing.

Given the high unmet medical need and identified preferences of the Duchenne community, PPMD strongly believes that the data supporting approval of **AMONDYS 45** is sufficient to warrant coverage for all amendable patients as the post-approval studies are conducted to further confirm clinical benefit in real world settings.

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] https://dhhr.wv.gov/bms/BMS%20Pharmacy/Documents/Vyondys%2053%202020.1a.pdf

[2] *FDA grants accelerated approval to first drug for Duchenne muscular dystrophy (EXONDYS 51)* https://www.fda.gov/news-events/press-announcements/fda-grants-accelerated-approval-first-drug-duchenne-muscular-dystrophy

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

[4] *FDA grants accelerated approval to first targeted treatment for rare Duchenne muscular dystrophy mutation (VYONDYS 53)*https://www.fda.gov/news-events/press-announcements/fda-grants-accelerated-approval-first-targeted-treatment-rare-duchenne-muscular-dystrophy-mutation

[5] *FDA Approves Targeted Treatment for Rare Duchenne Muscular Dystrophy Mutation (VILTEPSO)* https://www.fda.gov/news-events/press-announcements/fda-approves-targeted-treatment-rare-duchenne-muscular-dystrophy-mutation6

[6] FDA Approves Targeted Treatment for Rare Duchenne Muscular Dystrophy Mutation (AMONDYS 45) https://www.fda.gov/news-events/press-announcements/fda-approves-targeted-treatment-rare-duchenne-muscular-dystrophy-mutation-0

[7] *EXONDYS 51 FDA Label:* http://www.accessdata.fda.gov/drugsatfda_docs/label/2016/206488lbl.pdf

[8] *VYONDYS 53 FDA Label:* https://www.accessdata.fda.gov/drugsatfda_docs/label/2019/211970s000lbl.pdf

[9] *VILTEPSO FDA Label:* https://www.accessdata.fda.gov/drugsatfda_docs/label/2020/212154s000lbl.pdf

[10] *AMONDYS 45 FDA Label:* https://www.accessdata.fda.gov/drugsatfda_docs/label/2021/213026lbl.pdf

[11] *State Medicaid Coverage of Drugs Approved by the FDA under Accelerated Approval Pathway (Release 185)* https://www.medicaid.gov/medicaid-chip-program-information/by-topics/prescription-drugs/downloads/rx-releases/state-releases/state-rel-185.pdf

[12] *Poster Presented at the World Muscle Society Virtual Congress September 28−October 2, 2020* http://join.parentprojectmd.org/amondysposter *(publication pending)*

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