January 25, 2024

Oregon Health Authority
Drug Use Research & Management Program
Oregon Drug Use Review / Pharmacy & Therapeutics Committee

To the Committee;

My name is Brian Denger, and I am a member of Parent Project Muscular Dystrophy, a volunteer health organization focused on improving outcomes for those affected by Duchenne muscular dystrophy. I also have an adult son who has Duchenne muscular dystrophy. Supporting Patrick and individuals who live with Duchenne muscular dystrophy is the reason for my writing to the Oregon Drug Use Review / Pharmacy & Therapeutics Committee (Committee). I write to strongly recommend the Committee add the Food and Drug Administration approved gene therapy treatment, Elevidys (delandistrogene moxeparvovec-rokl) to the list of approved drugs for the treatment of eligible 4- and 5-year-old Duchenne muscular dystrophy patients as well as Agamree (Vamorolone) for eligible patients ≥2 years of age.

Duchenne muscular dystrophy (DMD) is an extraordinarily complex, progressive, degenerative muscle wasting disorder. Based on the advice of his expert clinical team, my son Patrick is treated with several drugs and therapies for his condition, including Exondys-51 (Exondys) as he has an amenable genetic variant. I realize Exondys-51 is not a gene therapy treatment yet serves as an appropriate comparator. His primary care is provided by an interdisciplinary team of DMD experts at Kennedy Krieger Institute (KKI) in Baltimore, MD. Patrick's KKI neurologist is a leading clinician/scientist in the muscular dystrophy field who is the Primary Investigator on over a dozen clinical trials for DMD and other neuromuscular disorders. On her recommendation Patrick decided to initiate use of Exondys-51.

My son, now 29 years old, has been treated with Exondys-51 since December 2016. I often read that the clinical benefit of Exondys-51 is "marginal" as the drug only produces small amount of dystrophin protein. For patients with DMD, marginal benefits change the natural history of disease progression. Preservation of function not only translates to continued ability, it delays the deleterious effects of disease progression which can increase survival. Forgive the frankness, yet lengthening survival without maintaining function and quality of life merely adds to patient and caregiver burden; we are fortunate that Patrick is experiencing both.

Patrick drives an adapted van, is self-employed as an online streamer, independently feeds himself and uses his computers and cell phone without assistance. (My wife and I provide support for all his activities of daily living.) I'm not sure you realize the significance for, especially for a man his age with DMD.

Recognizing that each person with DMD is unique and that the same interventions may lead to different results, providing a therapy that may help preserve function and survival to patients who have few viable alternatives is appropriate and vital. Patrick's example of continued independence bolsters my argument. Early treatment with Elevidys may significantly extend the time a treated individual is able to walk. In addition to increasing a child's ability to participate in similar activities as their unaffected

peers, later walking allows the trunk muscles to fully develop, eliminating the need for spinal intervention for scoliosis and helps in preserving upper body and limb function. The importance of upper body ability for selfcare and the use of computers and communication devices makes a significant difference for affected individuals regarding quality of life and independence. Replacing that independence with a team of state-funded personal care attendants to get him up, prepare him for work (if that were still possible) and assist him throughout the day becomes the alternative. As a parent of a disabled adult child and a former member on the Boards of Directors for local organizations that support people with that level of need, I'm fully aware of the expense and difficulty in obtaining staff to meet those obligations.

My request is that the Committee votes to provide coverage of Elevidys and Agamree to Oregon Medicaid covered patients with DMD who meet the FDA label criteria.

Thank you for your consideration.

Sincerely,

Brian Denger, Community Engagement Coordinator
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January 17, 2024

Oregon Health Authority
P&T Committee

RE: Drug Class Update with New Drug Evaluation: Drugs for Duchenne Muscular Dystrophy

To Whom It May Concern:

We are writing to provide feedback on the proposed updated OHA criteria for Duchenne muscular dystrophy (DMD) to be reviewed in the February 1, 2024 Pharmacy and Therapeutics meeting. As background, we are the neuromuscular clinicians in the only Muscular Dystrophy Association-supported pediatric multidisciplinary neuromuscular clinic in Oregon. Dr. Finanger, the program director, has fellowship training in Neuromuscular Medicine and is board certified in Neurology and Neuromuscular Medicine. Both Dr. Finanger and Ms. Leach each have more than 15 years of experience caring for individuals with DMD.

We have a few recommendations to consider with regards to the proposed criteria listed in Appendix 6 to be reviewed:

Duchenne Muscular Dystrophy Criteria:

1. Criteria 5: Standard CDC recommendations for MMR vaccine are for 2 doses at 12-15 months and 4-6 years of age. Thus, approval criteria 5 should be updated to reflect only 1 dose for those patients under age 4 at initiation of steroids.

2. Criteria 12: We would recommend starting exon skipping as soon as a patient is diagnosed (no age restriction in FDA labels).

    a. Given that many experts do not start corticosteroids, especially daily corticosteroids, in patients under age 4, this would lead to a delay in initiating exon skipping therapies. We often recommend high-dose twice weekly dosing for young patients, but this is not a universal practice. Thus, there should be a discussion of corticosteroid therapy with the neuromuscular specialist, but no requirement for daily therapy.
    b. In addition, referral to pediatric neurology/neuromuscular is often delayed such that many patients are not seen until 5 years of age. Thus, further delay of exon skipping therapies while patients initiate corticosteroids would be detrimental and unnecessary by medical standards. This enrollment criteria was included in the clinical trials only to exclude the improvement seen in the first 6 months of steroid treatment as this would have confounded the results of the trial.

Delanodystrogene moxeparvovec Criteria:

1. Criteria 6. The goal is stated as “restrict the use of this gene therapy to patients with the FDA-labeled indication”. However, criteria 6 excludes patients with deletions of exons 1-17 and 59-71. **Thus, the proposed OHA criteria does not match with FDA label**. In addition, it is important to note that the FDA was able to review safety information from the larger study 103 (ENDEAVOR; NCT04626674) which did not have any limitations on mutations. They concluded that only exons 8-9 should be excluded. Thus, I strongly support removing criteria 6 as this discussion would be part of the standard risk/benefit discussion had with the family by the prescribing neuromuscular specialist as part of standard of care.

Thank you for the opportunity to share our clinical experience and your continued partnership to provide excellent care for pediatric neuromuscular patients in Oregon.

Sincerely,

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