May 20, 2021

State of West Virginia
Health and Human Services
Bureau of Medical Services
Drug Utilization Review Board (DURB)

To the Board;

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 the Drug Utilization Review Board (Board). I write to strongly urge the Board to add the recently approved drug, Amondys-45, to the Department of Health and Human Services' list of approved drugs for the treatment of Duchenne muscular dystrophy patients who have an amenable gene variant, regardless of age or ambulatory status.

Duchenne muscular dystrophy (DMD) is an extraordinarily complex, progressive, degenerative muscle wasting disorder. Based on the advice of his expert clinical team, he is treated with several drugs and therapies for his condition, including Exondys-51 (Exondys) as he has an amenable gene variant. Patrick's primary care is provided by an interdisciplinary team of DMD experts at Kennedy Krieger Institute (KKI) in Baltimore, MD. His 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 26 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" benefit changes the natural history of disease progression. Preservation of function not only translates to continued ability, yet 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 of his activities of daily living.) Exondys also slows the progression of pulmonary decline. My son's Forced Vital Capacity is still in 40% range. He uses a Cough Assist to maintain healthy chest wall and respiratory muscles and began limited nighttime ventilatory support (Bi-Pap) beginning the end of 2019. He has no pulmonary deficiency symptoms. I'm not sure you realize the significance, especially for a man his age with DMD.

Why Amondys-45? Recognizing that each person with DMD is different and that the same interventions may lead to different results, providing a drug that may help preserve function and survival to patients who have no viable alternatives is appropriate and vital. Patrick's example of

continued independence bolsters my argument. 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. Furthermore, preventing a decline in pulmonary function also delays the need for skilled nursing support. As a former member of the Board of Directors for local organizations that supported people with that level of need, I'm fully aware of the expense and the difficulty in obtaining staff to meet those obligations.

Amondys-45 treatment requires weekly intravenous therapy. Due to fragile/impaired vasculature, a co-morbidity of DMD, some patients use an implanted venous access port (Medi-port). The decision to consider treatment with Amondys-45, including the additional requirements, requires a value judgment, best undertaken by patient and family with their physicians. That opportunity will only be afforded with the Board's approval of coverage for Amondys-45.

Finally, preservation of function is about the retention of quality of life. My request is that the committee votes to provide coverage of Amondys-45 to all patients with DMD who have an amenable deletion mutation. This would also place coverage in alignment 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". I thank you in advance for your thoughtful consideration of my request.

Sincerely,

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