October 13, 2023

The State of Nevada Department of Health and Human Services
Division of Healthcare Financing and Policy
Drug Utilization Review Board

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 to the Drug Utilization Review Board (DUR). I write to strongly recommend the DUR 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.

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 former member on the Boards 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.

My request is that the DUR votes to provide coverage of Elevidys to Nevada Medicaid covered patients with DMD who meet the FDA label criteria.

Thank you for your consideration.

Sincerely,

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