August 20, 2024

Oregon Health Authority
Drug Use Research & Management Program

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 my son and individuals who live with Duchenne muscular dystrophy is the reason for my writing to the Drug Use Research & Management Program (Committee). I write to strongly recommend the Committee adopt the draft policy for the Food and Drug Administration's recently expanded approval for gene therapy treatment, Elevidys (delandistrogene moxeparvovec-rokl). The expansion allows patients ≥4 years of age, irrespective of ambulatory status to be treated with Elevidys when prescribed by their treating physician. Additionally, adding Duvyzat (givinostat) as a covered therapy for patients ≥6 years of age is also encouraged.

Duchenne muscular dystrophy (DMD) is an extraordinarily complex, progressive, degenerative muscle wasting disorder. Preserving function at any stage of the disease process is important to the affected individual and their family. Recognizing that each person with DMD is unique and that the same interventions may lead to different results, the options for gene therapy and the HDAC treatment Duvyzat are important considerations that families and their experienced medical team need to discuss as ways to preserve function. Early treatment with Elevidys or Duvyzat may 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. Slowing the progression of the disease process isn't trivial to those living with DMD.

I am encouraged by the published draft policies. My request is that the Committee votes to provide coverage of Elevidys and Duvyzat to Oregon Medicaid covered patients with DMD who meet the FDA label criteria.

Thank you for your consideration.

Sincerely,

Brian Denger, Community Engagement Coordinator
Parent Project Muscular Dystrophy
Biddeford, ME 04005
brian@parentprojectmd.org

National
Multiple Sclerosis
Society

August 16, 2024

Oregon Pharmacy & Therapeutics Committee
College of Pharmacy, Oregon State University
Corvallis, Oregon

RE: Multiple Sclerosis drug class reviews for October 1, 2024; Pharmacy & Therapeutics Committee

Thank you for the opportunity to offer comments to the Pharmacy & Therapeutics Committee meeting for October 2024. The National Multiple Sclerosis Society (the Society) appreciates the time and care that is required to design a complete and cost-effective formulary for state plans. Our comments today relate to the preferred/non-preferred classification of multiple sclerosis disease modifying therapies (DMTs) and the need for continued access to the full suite of available and approved medications. **The National Multiple Sclerosis Society urges you to provide access to the full range of MS disease-modifying therapies** – with consideration to efficacy, route of administration, mechanism of action, and interchangeability or non – as well as other medications to treat the disease symptoms to ensure the needs of every individual are met.

MS is an unpredictable disease of the central nervous system. Currently, there is no cure. Symptoms vary from person to person and may include disabling fatigue, mobility challenges, cognitive changes, and vision issues. An estimated one million people live with MS in the United States. Early diagnosis and treatment are critical to minimize disability. Significant progress is being made to achieve a world free of MS.

No single agent is 'best' for all people living with MS, and, as MS presents differently in each person, every person's response to a DMT will vary. Early and ongoing treatment with a DMT is the only way to modify the course of the disease, slow the accumulation of disability and protect the brain from damage due to MS. People with MS and their treating clinicians require access to the full range of treatment options that represent different mechanisms of action and routes of administration with varying efficacy, side effects, and safety profiles.

It is common for people with MS to move through several different DMTs throughout their life with MS, as they "breakthrough" on medication - have disease activity or need to try a different DMT. Delays or disruptions in treatment can risk permanent, irreversible disease progression, worse health outcomes, and increased healthcare costs over time. Managing MS can be a difficult process that requires several "trial and error" changes to the medication before finding the one most effective at controlling disease progression with the fewest negative side effects for each individual.

National
Multiple Sclerosis
Society

Access to the full range of options is essential for optimal MS management.

Early and ongoing treatment with a DMT is the best way to modify the course of the disease, slow the accumulation of disability and protect the brain from damage due to MS. [1] According to a consensus paper by the MS Coalition, people with MS and their treating clinicians require access to the full range of treatment options for several reasons[2]:

- Individual differences related to tolerability and adherence may necessitate access to different medications within the same class.
- Individuals' access to treatment should not be limited by their frequency of relapses, level of disability, or personal characteristics such as age, sex, or ethnicity.
- Different mechanisms of action allow for treatment change in the event of a sub-optimal response.
- Potential contraindications limit options for some individuals.
- Risk tolerance varies among people with MS and their treating clinicians.
- Route of delivery and side effects may affect adherence and quality of life.
- Absence of relapses while on treatment is a characteristic of treatment effectiveness and should not be considered a justification for discontinuing treatment.
- Treatment should not be withheld to allow for determination of coverage by payers as this puts the patient at risk for recurrent disease activity.

Considering this information, **we ask the Oregon Pharmacy and Therapeutics Committee to lessen the number of restrictions placed on prescribing DMTs and provide access to the full range of MS DMTs and other medications used to treat symptoms of the disease.** We strongly believe people with MS should be at the center of their healthcare decision-making, and that they and their physicians must be allowed to determine the most appropriate medication through a shared decision-making process that considers individual factors.

Thank you for your work on behalf of all Oregonians. The Society is here to serve as a resource and advocate for medications and services critical for people living with and affected by MS. Please contact Seth Greiner, Senior Manager of Advocacy, with any questions or clarifications.

Sincerely,

Seth M. Greiner
Senior Manager, Advocacy
Seth.Greiner@NMSS.org

[1] The Multiple Sclerosis Coalition has published an evidence-based consensus paper entitled The Use of Disease-Modifying Therapies in Multiple Sclerosis: Principles and Current Evidence. This document was developed and endorsed by the eight Coalition member organizations and subsequently endorsed by the Americas Committee for Treatment and Research in Multiple Sclerosis (ACTRIMS). The paper is designed to: summarize current evidence about disease modification in MS; highlight the importance of early and ongoing treatment; and provide support for broad access to FDA-approved MS disease-modifying therapies for people with MS in the United States.

[2] https://www.nationalmssociety.org/NationalMSSociety/media/MSNationalFiles/Brochures/DMT_Consensus_MS_Coalition.pdf

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Multiple Sclerosis Association of America

August 27, 2024

Dr. Sejal Hathi, MD, MBA
Director
Oregon Health Authority
500 Summer Street, NE, E-20
Salem, OR 97301-1097

Re: October 2024 Draft MS Policy Guidance

Dear Dr. Hathi:

On behalf of the Multiple Sclerosis Association of America (MSAA), a patient advocacy organization dedicated to Improving Lives Today for individuals affected by MS, we are writing to provide comments on the upcoming MS Policy Guidance Review taking place in October 2024.

Access to the appropriate DMT for people living with MS should not be dependent on failing other MS DMTs. Research has found that delaying appropriate MS treatment can have a significant impact on short- and long-term outcomes for people living with MS. The decision of what DMT is appropriate for people living with MS should be decided by the person living with MS and their healthcare provider and should not be contingent upon failing other DMTs that may not be the most appropriate. MS is a heterogeneous chronic illness, and choosing the right DMT at the right time is critical for delaying progression and ensuring positive health outcomes. Having to fail two therapies that may not be appropriate before having access to the DMT that a healthcare provider and the person living with MS believe will optimize outcomes may lead to disease progression that cannot be reversed.

Multiple sclerosis is a chronic, incurable disease of the central nervous system with a high likelihood of progressive disability over time. A large body of evidence indicates that early and persistent treatment with an FDA-approved MS disease-modifying treatment (DMT) reduces the accumulation of damage in the brain and spinal cord, thus reducing relapses and disease progression. As the MS disease process is highly individualized, treatments must be carefully chosen for the highest efficacy, adherence, and long-term benefit. This requires access to a wide range of MS DMT's, with differing mechanisms of action and modes of administration. While cost is a critical factor, we believe that the Oregon Health Authority must consider additional factors in the

shared decision-making process to ensure that Oregonians living with MS have access to the MS DMT's that address their individual needs. Shared decision-making must also include the patient's voice, the MS provider's voice, and consideration of the evidence supporting the personal choice of an MS treatment option.

We want to express our gratitude specifically for the opportunity provided to stakeholders to voice concerns and make comments. We are available to provide additional information and insight as the Oregon Health Authority needs.

Sincerely,

Gina Ross Murdoch

Gina Ross Murdoch
President and CEO
Multiple Sclerosis Association of America'

CAN DO MULTIPLE SCLEROSIS

Oregon Health Authority
500 Summer Street, NE, E-20
Salem, OR 97301-1097
osupharm.di@oregonstate.edu

Dear Oregon Health Authority:

Thank you for the opportunity to provide comment on the Oregon Health Authority draft MS policy guidance. I am writing on behalf of Can Do Multiple Sclerosis, a non-profit patient advocacy organization that provides education, coaching, and support for people living with MS and their care partners.

We appreciate the extensive review process of the available MS disease modifying therapies, including efficacy and safety as well as the efforts in Oregon to contain cost. However, we also have concerns about the prior authorization requirements presented in the draft document.

Multiple sclerosis is chronic, incurable, inflammatory, and degenerative disease of the central nervous system (CNS). It is highly heterogeneous and thus requires individualized treatment planning. Treatment is directed at reducing new CNS inflammation, clinical relapses, and disease progression. It is well established that early initiation and persistence with an MS disease modifying therapy (DMT) is crucial for reducing disease activity and disability. Delays in treatment initiation have been shown to negatively impact long-term outcomes. Given the variability in disease presentation and the risk of disability among individuals with MS, research increasingly supports the early use of high-efficacy DMTs as first-line treatments. Studies have shown that early intervention with these therapies can significantly reduce the risk of relapses and slow disease progression, ultimately preserving neurological function[1-5].

Ideally, the decision-making process for Multiple Sclerosis (MS) disease-modifying therapies (DMTs) is a collaborative effort between the neurology provider and the person living with MS. This process considers the individual's disease history, disease activity, drug efficacy, potential risks, and side effects, alongside personal goals, risk tolerance, family planning, and lifestyle needs.

Given this information, we have the following concerns about the 2024 draft approval and renewal criteria.

- In the injectable drug and natalizumab criteria sections of the 2024 draft document, there is a requirement for trial and failure of two approved drugs for MS before an injectable drug or natalizumab can be approved. While we are not sure if this means any two DMTs or two drugs

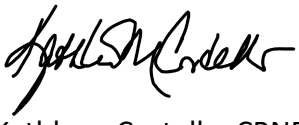
from the PDL, any trial/failure requirement could be detrimental to outcomes. In MS, where "time is brain," treatment delays associated with escalation therapy can result in irreversible damage. This could be particularly true in clinically isolated syndrome (CIS), where there is the opportunity to slow disease activity very early in the disease process. Therefore, all DMTs, including high-efficacy treatments should be available, without the delays often necessitated by stepwise escalation or approval processes.

- It is unclear if the trial/failure requirement applies to people with a diagnosis of primary progressive MS (PPMS), where there is only one FDA approved treatment. A separate approval criterion for PPMS would be helpful for the PA process.
- The current renewal criteria asks the question: “Has the patient’s condition improved as assessed by the prescribing physician and physician attests to patient’s improvement? The MS DMTs are not intended to improve MS, as previous damage largely irreversible. However, the expectations of the DMTs are reduction in relapse rate relative to pretreatment rate, clinical stability without significant worsening of existing symptoms and no MRI evidence of new activity. Please keep in mind that MS is progressive, and while the MS DMTs are effective, they are not a cure and there may be some change in symptoms over time. We recommend success be measured similarly to clinical trials - through reduction in new MRI activity, fewer relapses and minimal or no progression.

It is our hope that you consider the above points when finalizing the MS policy guidance. We believe that MS requires individualized treatment plans and to that end, we believe that people with MS will benefit from access to the full range of FDA approved DMTs, without treatment delays.

Thank you again for the opportunity to provide comments on the draft guidance document. Should you have any questions, please do not hesitate to contact me by email or phone, listed below.

Sincerely,



Kathleen Costello, CRNP, MSCN
Chief Operating Officer
Can Do Multiple Sclerosis
Avon, CO 81620
Email: kcostello@cando-ms.org
Mobile: 410-652-7822

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