January 6, 2022

Dana Hittle
Interim Deputy State Medicaid Director
500 Summer St. NE, E65
Salem, OR 97301

Re: Oregon Health Plan 1115 Demonstration Waiver Application for Renewal

Dear Deputy Director Hittle:

The National Organization for Rare Disorders (NORD) appreciates the opportunity to submit comments on the Oregon 1115 Demonstration Waiver for the Oregon Health Plan. NORD is a unique federation of voluntary health organizations dedicated to helping the 25-30 million Americans living with a rare disease. We believe that all patients should have access to quality, accessible, and affordable health coverage that is best suited to their medical needs.

The Medicaid program serves as lifeline for many people living with one of the 7,000 known rare diseases. Patients with rare disorders often find their financial lives upended by the debilitating nature of their diseases, and on their behalf, NORD is committed to ensuring that the Oregon Health Plan provides quality and affordable health care coverage to all low-income individuals and families. The state is well aware of the financial and clinical benefits of access to health insurance. Oregon's seminal 2008 health insurance experiment remains the only randomized study that has ever been used to evaluate the Medicaid program, and produced significant, robust data demonstrating the importance of public health insurance coverage. [1] We applaud Oregon's focus on health equity in this waiver application and are supportive of the state's request to provide multi-year continuous enrollment for children under six and two-year continuous eligibility for all beneficiaries ages six and over. We believe that these policies will improve continuity of care and reduce gaps in coverage for individuals with serious, chronic, and rare health conditions in the state of Oregon.

Unfortunately, this waiver also contains multiple proposals that would impede access to care for people with rare disorders. NORD opposes Oregon's plan to limit retroactive coverage for nearly all Medicaid beneficiaries and to waive the Early and Periodic Screening, Diagnostic and Treatment (EPSDT) benefit for beneficiaries over the age of one. In addition, we are strongly opposed to the state's proposal to create a closed formulary with as few as one drug per class and to exclude certain prescription drugs entirely, such as those approved through the Food and Drug Administration's (FDA) accelerated approval pathway. These provisions will make it harder for our patients to access the medications they need to stay healthy.

NORD offers the following comments and suggested changes on the 1115 Demonstration Waiver for the Oregon Health Plan:

[1] Finkelstein A, Taubman S, Wright B, Bernstein M, Gruber J, Newhouse JP, Allen H, Baicker K; Oregon Health Study Group. The Oregon Health Insurance Experiment: Evidence from the First Year. Q J Econ. 2012 Aug;127(3):1057-1106. doi: 10.1093/qje/qjs020. Epub 2012 May 3. PMID: 23293397; PMCID: PMC3535298.

Expansion of Continuous Eligibility

NORD supports Oregon's request for continuous enrollment for children under the age of six and two-year continuous eligibility for all beneficiaries aged six and over. Continuous eligibility reduces gaps in insurance coverage. Research has shown that individuals with disruptions in coverage during a year are more likely to delay care, receive less preventive care, refill prescriptions less often, and have more emergency department visits.[2] For rare disease patients who require daily, weekly, or monthly medications and/or health care provider engagement, reducing interruptions in coverage can have a significant and positive effect on health outcomes. Multi-year continuous enrollment for children has the potential to be especially impactful for the rare disease community, as rare diseases disproportionately affect children, adolescents, and young adults.[3] Finally, continuous eligibility will improve equitable access to care, as studies show that children of color are more likely to be affected by gaps in coverage.[4]

This continuous eligibility proposal is an example of how the state *should* be using the power and authority of the Medicaid program to positively impact access to health care services and, ultimately, health outcomes for low-income Oregonians. Unfortunately, other provisions of this waiver renewal application would undermine the benefits gained by expanded continuous eligibility by restricting access to necessary services and treatments. NORD encourages the state to proceed with expanding continuous enrollment for Oregon Health Plan beneficiaries and to revise other portions of the waiver renewal application to align with the same spirit and intent displayed in this section.

Adoption of a Closed Formulary

NORD is deeply concerned by the state's proposal to transition to a closed formulary for adult beneficiaries, with guaranteed coverage for only one drug per therapeutic class. Prescription drugs have different indications, different mechanisms of action and different side effects, depending on the person's diagnosis and comorbidities. Coverage of one drug per class is not sufficient to provide comprehensive options to patients with common conditions, let alone the vast variety of rare disorders.

Diseases present differently in different patients, and rare diseases often are particularly difficult to diagnose and treat. From the onset of symptoms, it takes on average six years for a patient with a rare disorder to receive an accurate diagnosis, with many patients experiencing misdiagnoses and receiving incorrect treatment at some point.[5] A rare disease patient, who has already been through a lengthy diagnostic odyssey and is finally on a stable treatment regimen should not be forced to change medications to something less effective so that the state can reduce prescription drug costs. Furthermore, this detrimental proposal is made worse by the fact it does not include an appeals process for patients to access non-formulary medications, even though such a process would not alleviate the burden for patients facing a closed formulary.

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[2]Sugar S, Peters C, De Lew N, Sommers, D. Medicaid Churning and Continuity of Care. ASPE. April 12, 2021. https://aspe.hhs.gov/sites/default/files/private/pdf/265366/medicaid-churning-ib.pdf.

[3] Tisdale, A., Cutillo, C.M., Nathan, R. et al. The IDeaS initiative: pilot study to assess the impact of rare diseases on patients and healthcare systems. Orphanet J Rare Dis 16, 429 (2021). https://doi.org/10.1186/s13023-021-02061-3

[4] Osorio, Aubrianna. Alker, Joan, "Gaps in Coverage: A Look at Child Health Insurance Trends", Center for Children & Families (CCF) of the Georgetown University Health Policy Institute, November 21, 2021. Gaps in Coverage: A Look at Child Health Insurance Trends – Center For Children and Families (georgetown.edu)

[5] Blöß S, Klemann C, Rother AK, Mehmecke S, Schumacher U, Mücke U, et al.Diagnostic needs for rare diseases and shared prediagnostic phenomena: Results of a German-wide expert Delphi survey. PLoS ONE. 2017; 12(2):e0172532. Available from: http://dx.plos.org/10.1371/journal.pone.0172532.

Without proper prescription drug coverage, a patient who may have been asymptomatic on their current medication could relapse and begin to experience symptoms again. For example, patients diagnosed with Neurotrophic Keratitis, a rare disorder that affects the retina, depend on prescription medications to prevent the further degeneration of the cornea that could lead to blindness. If a Neurotrophic Keratitis patient's prescription coverage were to change and the medication was no longer covered, the patient could go blind, requiring additional assistance to survive. A closed formulary limits the ability of providers to make the best medical decisions for the care of their patients, effectively taking the clinical care decisions away from the doctor and patient and giving them to the state.

NORD requests that the Oregon Health Authority amend this portion of their proposal and instead maintain a robust, open formulary for all beneficiaries that will allow patients to access the medications that their providers believe are best for them.

Limiting Access to FDA-Approved Treatments

NORD is also opposed to Oregon's proposal to limit Medicaid recipients' (both children and adults) access to prescription drugs that the state deems to have "limited or inadequate evidence of clinical efficacy," including those approved through FDA's accelerated approval pathway. The state claims that drugs which come onto market through the accelerated approval pathway "have not yet demonstrated clinical benefit and have been studied in clinical trials using only surrogate endpoints." Further, the state implies that the FDA is allowing drugs to come onto the market that have not been "fully clinically proven." This proposal is clearly premised on an inaccurate understanding of the FDA's approval process for therapies on the market via the accelerated approval pathway, and if implemented, would cause significant harm to rare disease patients by restricting access to many novel and lifesaving therapies.[6]

NORD recently issued a report that details the history and development of the accelerated approval pathway and its historical importance for rare disease patients and the treatments they need.[7] As Oregon notes in its application, the accelerated approval pathway was codified by Congress in 2012. What the state fails to describe in its application, however, is that FDA had actually enshrined the pathway in law when it implemented regulations in 1992. At that time, the HIV/AIDS epidemic had drastically altered the landscape for drug development.[8] The epidemic—with its staggeringly high death toll and commensurate urgency around developing treatments—catalyzed a reconsideration, by patients and regulators alike, of more traditional clinical trial requirements. New thinking was necessary for what was essential to the demonstration of efficacy, and in response, scientists sought ways to streamline and expedite clinical trials for HIV/ AIDS drugs to focus on the utility of surrogate endpoints which were known to demonstrably correlate with improved outcomes.[9] For example, improved T-cell count was determined to reliably predict fewer infections in AIDS patients and was accepted as a surrogate endpoint that could be

[6] U.S. Food & Drug Admin., CDER Drug and Biologic Accelerated Approvals Based on a Surrogate Endpoint (June 30, 2021). https://www.fda.gov/media/151146/download

[7] Temkin E, Trinh, J. FDA's Accelerated Approval Pathway: A Rare Disease Perspective. October 2021. https://rarediseases.org/wp-content/uploads/2021/06/NRD-2182-Policy-Report_Accelerated-Approval_FNL.pdf

[8] The Centers for Disease Control and Prevention ("CDC") published its first report on HIV/AIDS in 1981. See James W. Curran & Harold W. Jaffe, AIDS: The Early Years and CDC's Response, 60 Morbidity & Mortality Weekly Rep. 64 (Oct. 7, 2011), available at https://www.cdc.gov/mmwr/preview/ mmwrhtml/su6004a11.htm.

[9] See U.S. Food & Drug Admin., Guidance for Industry: Human Immunodeficiency Virus-1 Infection: Developing Antiretroviral Drugs for Treatment 3 (Nov. 2015), www.fda.gov/files/drugs/published/Human-Immunodeficiency-Virus-1-Infection--Developing-Antiretroviral-Drugs-for-Treatment.pdf

used to demonstrate the efficacy of HIV/AIDS drugs.[10] This scientific advancement seemingly resolved the debate about modifying requirements regarding clinical efficacy: if approval of HIV/AIDS treatments (among others) could be predicated successfully on the use of surrogate endpoints, FDA's "substantial evidence of efficacy" standard did not need to be compromised to get treatments to patients sooner. As consensus grew about the utility of surrogate endpoints in clinical trial design,[11] FDA embraced drug approval reform and promulgated regulations formalizing the accelerated approval pathway.[12] Under accelerated approval, FDA could expedite the approval of and patient access to drugs that were intended to treat serious and life-threatening diseases and conditions for which there were unmet medical needs.

As noted in NORD's report, it is critical to understand that accelerated approval—both as it is set forth in law and in regulations—does not alter FDA's gold standard of substantial evidence of safety and effectiveness. To the contrary, accelerated approval is granted based on FDA's finding that a drug is safe and effective for its intended use— the same approval standard used for traditional approval. Traditional approval relies on a direct demonstration of clinical benefit, while accelerated approval relies on surrogate endpoints and intermediate clinical endpoints that can be measured earlier than irreversible morbidity or mortality.

Oregon's proposal states that such drugs are studied in clinical trials using "*only surrogate endpoints."* This language demonstrates a fundamental misunderstanding of the nature of surrogate endpoints and the rigor FDA applies in accepting such endpoints in the context of an accelerated approval drug. Such endpoints are chosen because FDA, in its scientific discretion, has decided that they are expected to predict clinical benefit. FDA can make the risk benefit calculation that an accelerated approval drug's benefits outweigh its risks—just as the agency does for traditional approval—and then confirm the clinical benefit in post marketing confirmatory studies conducted after (and as a condition of) the accelerated approval. Both the FDA and Congress have considered – and rejected – the notion that accelerated approval is a different or lesser standard than traditional approval. [13] In fact, while codifying the accelerated approval pathway in 2012, Congress acknowledged the vital role the accelerated approval pathway serves for patients with rare diseases and expressed their hope that it would bring life-saving drugs to the market expeditiously.[14] Congress also affirmed FDA's conclusion that accelerated approval did not create a different standard for drug approval, stating in a "Sense of Congress" Congressional understanding that accelerated approval "may result in fewer, smaller, or shorter clinical trials... without compromising or altering the high standards of the FDA for the approval of drugs."[15]

Today, accelerated approval is crucial to facilitate the development of drugs indicated to treat rare diseases. As stated above, 25-30 million Americans (or 1 in 10 people) suffer from rare disorders, which are

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[10] *Id*

[11] Approval of the first statin drug, for example, was predicated on the validated surrogate endpoint of lower cholesterol, which was accepted as a proxy for reduced risk of heart disease. Editorial, Biomarkers: The Next Generation, 9 Nature Reviews: Drug Discovery 415 (June 2010), https:// www.nature.com/articles/nrd3196.pdf

[12] FDA also created the fast track, breakthrough therapy, and priority review designations to advance the development and review of new drugs and address unmet needs in the treatment of a serious medical condition. See U.S. Food & Drug Admin., Guidance for Industry: Expedited Programs for Serious Conditions – Drugs and Biologics 1 (May 2014) ("Expedited Programs Guidance"), https://www.fda.gov/media/86377/download.

[13] 57 Fed. Reg. at 58944

[14] *See* H.R. Rep. 112-495, *35–36 (2012).

[15] *See* 158 Cong. Rec. H3825-01, H3848 (2012).

particularly likely to be serious and life-threatening diseases with unmet medical needs.[16] Of the 7,000 rare diseases that have been identified, more than 90% have no FDA-approved treatment.[17] Many facets of rare diseases make them particularly difficult to study in clinical trials targeting direct clinical benefit. For example, the number of patients with any one condition can be small and heterogeneous, with highly diverse clinical manifestations and a long timeframe for disease progression. Furthermore, there is often a lack of prior clinical studies and a limited number of clinical investigators and treatment centers knowledgeable about a given rare disorder.[18] This makes accelerated approval a particularly important tool for the development of treatments for rare diseases.[19] Limiting coverage of drugs that come onto market through this pathway means that patients with a rare disease who *do* have an FDA-approved therapy will be unable to access vital and lifesaving treatment. NORD believes that this provision will disproportionately and unfairly limit access to care for patients with rare disorders.

Oregon's proposal ultimately puts the state in the position of second-guessing FDA's expert scientific judgment and role in the drug review process. The Oregon Health Authority lacks the capacity and expertise to overrule FDA decisions regarding the safety and efficacy of new medicines. NORD acknowledges that the high cost of many drugs, including accelerated approval products, present significant affordability challenges to the state. Indeed, we have long advocated at the federal level for state Medicaid programs to be given the resources that they need to maintain broad and accessible coverage for patients. However, it is inappropriate to attempt to resolve drug pricing challenges through the differential treatment of accelerated approval drugs or other drugs that have been FDA approved through one of the FDA's expedited programs. The core premise of FDA's expedited programs, as established by Congress, is that these drugs are exceptionally important to patients and should be provided to them in a manner that is as expedient as possible. Restrictions in patients access to these products runs counter to the clear intent of the laws that establish these programs. We urge the state to remove this request from the draft waiver renewal application.

Retroactive Eligibility

NORD opposes the state's proposal to continue to eliminate retroactive coverage for nearly all beneficiaries, excluding the aged, blind and disabled. Retroactive eligibility in Medicaid prevents gaps in coverage by covering individuals for a set amount of time prior to the month of application, assuming the individual is eligible for Medicaid coverage during that time frame. In many cases, individuals are not aware that they are eligible for Medicaid until they have an unexpected medical event or receive a new diagnosis. Eligible applicants may also delay necessary health care until the Medicaid enrollment process is complete, which can increase their health risks and exacerbate any health conditions that they may have.

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[16] Jennifer Huron, *New Study Investigates the Number of Available Orphan Products, Generics and Biosimilars*, Nat'l Org. for Rare Disorders (Mar. 25, 2021), https://rarediseases.org/new-study-investigates-the-number-of-available-orphan-products-generics-and-biosimilars/.
[17] *Id.*
[18] Food and Drug Administration. Report: Complex Issues in Developing Drugs and Biological Products for Rare Diseases and Accelerating the Development of Therapies for Pediatric Rare Diseases Including Strategic Plan: Accelerating the Development of Therapies for Pediatric Rare Diseases. July 2014. https://www.fda.gov/media/89051/download
[19] U.S. Food & Drug Admin., CDER Drug and Biologic Accelerated Approvals Based on a Surrogate Endpoint (Jan. 14, 2021), https://www.fda.gov/ media/88907/download

Retroactive eligibility allows patients who are newly diagnosed with a serious illness, such as a rare disease, to access treatment immediately without being burdened by medical debt prior to their official eligibility determination. In Indiana, for example, Medicaid recipients were responsible for an average of $1,561 in medical costs with the elimination of retroactive eligibility.[20] Without retroactive eligibility, Medicaid enrollees could then face substantial costs at their doctor's office or pharmacy. The financial concerns are particularly acute for patients with rare conditions, as data supplied by the National Institute of Health has shown that people with rare diseases typically incur much higher health care costs over the course of year or during a hospitalization than people without a rare disease.[21]

Patients with underlying health conditions who are unable to access regular care are often forced to go to emergency rooms and hospitals if their conditions worsen, leading health systems to provide more uncompensated care. For example, when Ohio was considering a similar provision in 2016, a consulting firm advised the state that hospitals could accrue as much as $2.5 billion more in uncompensated care as a result of the waiver.[22] Increased uncompensated care costs are especially concerning as safety net hospitals and other providers continue to deal with limited resources and capacity during the COVID-19 pandemic. Limiting retroactive coverage increases the financial hardships to rural hospitals that absorb uncompensated care costs.

NORD opposes the continued limitations to retroactive coverage and encourages the state to expand retroactive coverage to include all Medicaid beneficiaries.

EPSDT Benefit

NORD is opposed to restricted coverage for treatment under Early and Periodic Screening, Diagnosis and Treatment (EPSDT). The EPSDT statute is of enormous importance to the rare disease community. EPSDT provides a comprehensive array of prevention, diagnostic, and treatment services for low-income infants, children, and adolescents, and is the mechanism through which many children are diagnosed with a rare disease. By identifying rare disorders early, the EPSDT benefit allows almost immediate intervention for conditions that, if left undiagnosed and untreated during the early stages of their progression, could cause severe physical and developmental impairment or death.

The purpose of the EPSDT benefit is to ensure that children receive appropriate health care and limiting that care to a prioritized list of services leaves families vulnerable should their child need services beyond those offered on the list. As stated before, there are more than 7,000 different rare disorders, many of which are not well studied or understood. There is no way to ensure that the states list of prioritized services will be sufficient for rare disease patients.

The state has demonstrated through other efforts a desire to increase equitable access to health care. However, the continued restriction of the EPSDT benefit is a step in the opposite direction. For example,

[20] Healthy Indiana Plan 2.0 CMS Redetermination Letter. July 29, 2016. Available at: https://www.medicaid.gov/Medicaid-CHIP-Program-Information/By-Topics/Waivers/1115/downloads/in/Healthy-Indiana-Plan-2/in-healthy-indiana-plan-support-20-lockouts-redetermination-07292016.pdf

[21] See Tisdale, *supra* note 3

[22] Virgil Dickson, "Ohio Medicaid waiver could cost hospitals $2.5 billion", Modern Healthcare, April 22, 2016. (http://www.modernhealthcare.com/article/20160422/NEWS/160429965)

children of color are enrolled in Medicaid at disproportionately higher rates[23] and as mentioned before, are more likely to be affected by gaps in coverage.[24] These children are likely to be disproportionately affected by limitations to the EPSDT benefit. NORD urges the state to remove restrictions to EPSDT to allow children full and equitable access to health care, in keeping with the purpose of the benefit.

Conclusion

Thank you for the opportunity to provide comments. For questions about NORD or our comments please contact Corinne Alberts at calberts@rarediseases.org.

Sincerely,

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Policy Manager, Western Region
National Organization for Rare Disorders

[23] Brooks, Tricia. Whitener, Kelly. "At Risk: Medicaid's Child-Focused Benefit Structure Known as EPSDT," Center for Children & Families (CCF) of the Georgetown University Health Policy Institute, June 2017. EPSDT-At-Risk-Final.pdf (georgetown.edu)
[24] Osorio, Aubrianna. Alker, Joan, "Gaps in Coverage: A Look at Child Health Insurance Trends", Center for Children & Families (CCF) of the Georgetown University Health Policy Institute, November 21, 2021. Gaps in Coverage: A Look at Child Health Insurance Trends – Center For Children and Families (georgetown.edu)