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Oregon Health Authority

Health Policy and Analytics Medicaid Waiver Renewal Team

Attn: Michelle Hatfield

500 Summer St. NE, 5th Floor, E65

Salem, OR 97301

RE: Application for Renewal and Amendment Oregon Health Plan, Section 1115 Demonstration Waiver

On behalf of the EveryLife Foundation for Rare Diseases, which is dedicated to empowering the rare disease patient community to advance laws and policies to improve lives of all people and families impacted by rare diseases and disorders, we are pleased to provide comments to inform the Oregon Health Authority's (OHA) Section 1115 Waiver Application for the period of 2022-2027.

EveryLife's policy priorities are informed by the needs of our community and our shared mission of advancing the equitable development of, and access to, lifesaving diagnoses, treatments, and cures. To inform our policy work, we convene the Community Congress, a forum for collaboration across stakeholders, representing over 200 individual rare disease patient advocacy organizations in addition to over 90 other healthcare and biotechnology organizations.

The EveryLife Foundation appreciates the many worthy goals laid out by OHA in the Waiver planning process, however we are concerned that the policies proposed under Strategy 3: *Increase predictability of costs and ensure value for spending through closer management of pharmacy costs by adopting commercial-style closed formularies and by excluding drugs with limited or inadequate evidence of clinical efficacy* will have serious adverse consequences for rare disease patients and families. These proposed policies stand in direct conflict with the overall goals that the Waiver request is premised on.

Over 30 million Americans live with one or more rare diseases, 95% of which have no approved treatment¹. Rare disease patients and families navigate how to manage expenses from multiple inpatient and outpatient encounters, costs for prescription therapies and medical devices, and the support services that are critical for managing their health and wellbeing. For millions of these patients and families, Medicaid is their lifeline. It is imperative that policies support timely access to all care and treatments recommended by clinical providers, including innovative therapies that address the significant unmet need, without restrictive formularies that do not reflect the individualized nature of rare disease care.

Patients and families need relief from the high costs associated with managing a rare disease. But such relief must take into account the total cost of patient care, not just the cost of pharmaceutical therapies. [EveryLife Foundation's National Economic Burden of Rare Disease Study in the U.S.²](#) included 379 rare diseases affecting 15.5 million people and found the overall economic impact in 2019 exceeded \$966 billion. Non –medical and In-direct costs, costs absorbed directly by affected families, accounted for nearly 60 percent of overall costs. Relief is needed. But such relief must not come at the expense of access to life changing therapies or the next generation of innovation that 95% of rare diseases are still awaiting.

To reflect the realities of the need to lower costs across stakeholders, the EveryLife Foundation engaged with Community Congress members to create the Rare Disease Community Statement on Drug Pricing Policy Priorities. Principles from this statement are reflected throughout our comments and the full statement can be found at <https://everylifefoundation.org/drug-pricing/>.

Policy solutions should recognize FDA's statutory authority in determining a medical product's safety and effectiveness and promote timely access to approved therapies.

OHA's proposal to "allow exclusion of drugs with limited or inadequate evidence of clinical efficacy" and to establish their own review procedure to make this determination undermines the FDA's statutory authority, their expertise and understanding of the complexities involved in bringing therapies to the market for complex rare diseases, and the high level of rigor in the FDA regulatory review process.

In 1992, in response to the HIV/AIDS crisis, the U.S. Food and Drug Administration (FDA) instituted the Accelerated Approval pathway to improve the time to approve drugs that treat serious conditions that fill an unmet medical need based on substantial evidence of safety and efficacy and a surrogate endpoint that is reasonably likely to predict outcomes like irreversible mortality or morbidity. In the years since establishing the accelerated approval pathway, it has also facilitated the transformation of oncology care and enabled rare disease treatments to reach patients who otherwise had limited or no options.

OHA's proposal is predicated on several misperceptions about the use of the accelerated approval pathway in rare diseases and will serve to undermine the purpose and future use of the pathway.

During the EveryLife Foundation's Annual [Scientific Workshop in December 2021](#), rare disease scientific, clinical, patient and regulatory experts examined the use of the Accelerated Approval Pathway in rare disease to identify common challenges and opportunities for optimizing the pathway to transform rare disease care in the way that is has been transformational for HIV/AIDS and oncology.

A summary of the Workshop's proceedings is pending release, however several key themes emerged that address the concerning misperceptions of accelerated approval that underly OHA's decision to pursue exclusionary policies based on the use of this pathway.

- ❖ Therapies approved via the accelerated approval pathway are not the main driver of costs to state Medicaid programs. A 2021 study showed that spending on drugs approved through AAP accounted for less than one percent of annual Medicaid spending between 2007 and 2018³.
- ❖ Surrogate biomarkers are often superior to traditional clinical endpoints. In many cases, because surrogate biomarkers are intermediate markers of disease progression or improvement, they offer a way to more accurately capture patient data in real-time and be more accurate due to the complex and variable nature of rare diseases as compared to traditional clinical endpoints.
- ❖ The FDA has rigorous requirements for biomarker qualification that result in only a small fraction of biomarker based rare disease therapy approvals. In fact, less than 20 rare disease therapies have been approved using accelerated approval.⁴
- ❖ The safety of rare disease treatments approved via the accelerated approval pathway is evaluated in the same rigorous manner as in traditional drug approvals.

We urge OHA to reconsider their problematic proposals related to the accelerated approval pathway and to instead consider engaging in efforts prioritized by the Scientific Workshop participants as essential to ensuring the optimization of accelerated approval to transform rare disease outcomes and to consider the actions suggested within the Rare Disease Community Statement on Drug Pricing Policy Priorities. These actions include;

- ❖ The need for payer entities such as Medicaid programs to engage early and often with patient, scientific, clinical and regulatory stakeholders to develop the knowledge and evidence available for rare disease biomarkers. As payers engage early in the process, the understanding of the biomarkers being used and the rationale for their use will produce outcomes that payers can meaningfully incorporate into their decision-making process.
- ❖ Reject policies that treat all AAP medications as experimental, thus exacerbating health inequality among communities eligible for life-saving medications.
- ❖ Support the robust collection of real-world outcomes to enhance the evidence for AAP therapies in the post-market setting.
- ❖ Seek new and alternative payment models, including outcomes-based contracting, that allow rare disease patients to have access to novel therapies and ensure reimbursement policies encourage development of future curative therapies
- ❖ Exclude rare disease therapies from policy experiments or demonstrations (such as those proposed under Strategy 3) until or unless it is proven that the changes do not threaten patient access and medical innovation.

Oregon's proposal to implement a closed formulary, including the exclusion of therapies approved via the accelerated approval pathway, will have serious adverse consequences for rare disease patients and families. These proposed policies stand in direct conflict with the overall goals the Waiver request is premised on. Further, the proposed policies will create further disparities in care between adults and children and will result in increasing burdensome utilization management barriers which lead to delays

accessing life sustaining and life-saving therapies, while also not addressing the biggest cost drivers in rare disease care for state Medicaid programs.

Meaningful policy solutions must focus on the total cost of patient care. Policy reforms that focus on only one aspect of healthcare costs and that threaten access to the few treatment options available to those with rare diseases will result in negative repercussions for patient health and have detrimental economic outcomes for families and society overall. We urge OHA to consider the extensive unmet needs and scientific challenges inherent in the rare disease community and ensure policy solutions reflect these complexities by removing Strategy 3 from the 1115 Waiver Extension and investing in long-term actions that will support future policy innovation to ensure the sustainable delivery of life-saving treatments to those most vulnerable Oregonians.

Thank you for the opportunity to provide comments on Oregon's 1115 Waiver Extension application. Please contact Jamie Sullivan, Director of Policy at jsullivan@everylifefoundation.org if we can provide any additional information to inform your process.

Sincerely,



Julia Jenkins
Executive Director
EveryLife Foundation for Rare Diseases



Annie Kennedy
Chief of Policy, Advocacy, & Patient Engagement
EveryLife Foundation for Rare Diseases

¹ <https://www.fda.gov/patients/rare-diseases-fda>

² https://everylifefoundation.org/wp-content/uploads/2021/02/The_National_Economic_Burden_of_Rare_Disease_Study_Summary_Report_February_2021.pdf

³ <https://www.fightchronicdisease.org/sites/default/files/FINAL%20Quantifying%20Impact%20-%20White%20Paper%20v6.pdf>

⁴ <https://www.fda.gov/media/151146/download>