



January 7, 2022

Mr. Patrick Allen
Director
Oregon Health Authority
Health Policy and Analytics Medicaid Waiver Renewal Team
Attn: Michelle Hatfield
500 Summer St. NE, E65
Salem, OR 97301

Dear Director Allen,

Traverse Therapeutics, Inc. (Traverse) appreciates the opportunity to provide comments on Oregon Health Authority's draft Section 1115 waiver and applauds the state for its innovative approaches. However, if the waiver is submitted and approved by the Centers for Medicare and Medicaid (CMS) as proposed, Traverse is concerned about two provisions: 1) limiting drug access to patients for therapies approved through the Federal Food and Drug Administration's (FDA) accelerated approval pathway; 2) enacting a closed formulary. We urge the state to revise its waiver request and remove these provisions before formally submitting to CMS.

Traverse is a small biotechnology company based in San Diego, exclusively focused on identifying, developing, and delivering life-changing therapies to people living with rare disease. We have three approved products that are used by patients each day to treat rare nephrology and hepatology conditions. We also have late-stage research and development (R&D) programs with first-in-class potential for rare kidney conditions, and ongoing discovery efforts partnered with the National Institutes of Health (NIH) and leading patient advocacy organizations.

While millions of Americans have a rare disease or live with someone who does, the patient population living with any given rare disease is very small, even miniscule. By definition, rare disease patient populations are less than 200,000 people residing in the U.S., and in many cases are much smaller – one of our therapies serves under 100 U.S.-based patients. People with rare disease face unique challenges and often have very

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limited treatment options.¹ The complex nature of rare diseases combined with small patient populations presents a unique set of challenges to developing treatments for rare diseases and facilitating their access. In fact, 95% of the approximately 7,000 known rare diseases still have no treatment available.² Yet, for the 5% of patients that have the hope that comes with an FDA-approved treatment for their condition, it is likely that treatment was approved via the accelerated approval pathway due to the unmet need and burden of the disease.

Traverse writes to express concerns and ask the state to reconsider the following two provisions included in its draft waiver request:

Limits to Drugs Approved Via Accelerated Approval Would Decrease Access for Rare Disease Patients That Already Have Very Limited Options

Traverse, and others, are deeply concerned about the state's proposal to use its own review process to determine coverage of new drugs. If this proposal moves forward, Oregon could exclude drugs approved through FDA's accelerated approval process from its formulary. This would be devastating for the state's rare disease community as "accelerated approval offers a valuable source of hope," according to the National Organization for Rare Disorders.³ Additionally, the draft waiver proposes to use discretion of drugs when certain conditions, like only "surrogate endpoints have been reported." Surrogate endpoints are a crucial aspect of the FDA accelerated approval pathway that help allow patients access to life-changing medicine when there is an unmet need, without sacrificing safety and efficacy. In sum, Traverse disagrees with the state's interpretation of the intent of the 21st Century Cures Act.

In 2018, CMS notes in *State Release 185* that these drugs must be covered by the Medicaid program if there is a signed Medicaid National Rebate Agreement, but it also reaffirms that these drugs go through the same rigorous approval as drugs through the traditional approval process. *State Release 185* notes,

"Drugs granted accelerated approval by FDA under the process described in 506(c) of the FDCA are approved under section 505(c) of the FDCA and must meet the same statutory evidentiary standards for safety and effectiveness as those granted traditional approvals. See section 506(e)(2) of the FDCA. Thus, as noted above, at the time a product is granted accelerated approval, FDA has based such an approval on a determination that the drug has an effect on a

¹ See, e.g., Gina Kolata, 'It Will Consume Your Life': 4 Families Take On Rare Diseases, N.Y. TIMES, July 7, 2020, <https://www.nytimes.com/2020/07/07/health/rare-diseases.html>.

² Rare Diseases Clinical Research Network, National Institutes of Health, available at <https://report.nih.gov/nihfactsheets/ViewFactSheet.aspx?csid=126>.

³ https://rarediseases.org/wp-content/uploads/2021/06/NRD-2182-Policy-Report_Accelerated-Approval_FNL.pdf



surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint other than survival or irreversible morbidity.”⁴

Drugs approved via the accelerated approval pathway are a lifeline for the more than 95% of rare disease patients that do not yet have access to an FDA-approved treatment. If Oregon moves forward with circumventing the FDA process, which is considered to be the “gold standard” for review and efficacy of drugs, there would likely be unequal treatment and worsened health outcomes for patients already dealing with serious, life-threatening diseases. More is explained in the extensive findings and sense of Congress provisions of the *Food Drug Administration Safety and Innovation Act*, §901:

“[FDA] serves a critical role in helping to assure that new medicines are safe and effective. Regulatory innovation is 1 element of the Nation’s strategy to address serious and life-threatening diseases or conditions by promoting investment in and development of innovative treatments for unmet medical needs.”

Drugs approved through the accelerated approval pathway are subject to a demanding standard of review — demonstration of “substantial evidence” of effectiveness.⁵ In fact, studies have found that certain drugs reviewed under the accelerated approval processes have offered greater medical gains than drugs reviewed through the FDA’s traditional, lengthier process.⁶ Importantly, for drugs granted accelerated approval, post-approval confirmatory trials or studies are required as part of the regulatory process to verify and describe the anticipated clinical benefit.⁷ If the confirmatory trial fails to verify benefit, the FDA has the authority to withdraw approval and has done so when needed.⁸

Furthermore, if the goal of this section of the waiver is to save costs or improve care, we believe that is misguided. According to Medicaid claims data from 2007 to 2018, accelerated approval drugs only accounted for less than 1 percent of Medicaid spending.⁹ For the impacted families living with rare disease, “...downstream access concerns have emerged that could be chilling the appetite for investing in accelerated approval programs and [leave] patients without access to beneficial treatment,” according to the EveryLife Foundation for Rare Diseases.¹⁰

⁴ State Release 185, CMS, June 27, 2018.

⁵ 21 U.S.C § 355(d)(5).

⁶ Chambers, et al., *Drugs Cleared Through the FDA’s Expedited Review Offer Greater Gains Than Drugs Approved by Conventional Process*, Health Affairs Vol. 36, No. 8, 2017.

⁷ FDA. Guidance for Industry: Expedited Programs for Serious Conditions – Drugs and Biologics. May 2014.

⁸ FDA. Delivering Promising New Medicines Without Sacrificing Safety and Efficacy. FDA Voices: Perspectives from FDA Leadership and Experts. August 2019.

⁹ <https://www.fightchronicdisease.org/sites/default/files/FINAL%20Quantifying%20Impact%20-%20White%20Paper%20v6.pdf>

¹⁰ <https://everylifefoundation.org/events-schedule/scientific-workshop/>

Closed Drug Formularies Harm Rare Disease Patient Care

Traverse is also concerned about the state using a closed formulary approach and the impact that would have on rare disease patients that rely on Medicaid. If Oregon proceeds with covering one drug per therapeutic class, patient care and well-being would be severely compromised. It is not uncommon for rare disease patients to take multiple drugs to manage their condition. The decision on the best course of treatment should remain between the provider and patient, not the state's Medicaid program.

Medicaid is a lifeline for many rare disease patients and families. There could be many unintended consequences to the overall health of patients in Oregon if access is limited to only certain medications through a closed formulary approach. For example, medication adherence would likely decrease leading to worsened health outcomes.¹¹ Additionally, we believe a closed formulary approach will only further exacerbate disparities in care that could lead to delays and worsened health outcomes. We urge Oregon to reconsider this approach and prioritize rare disease patients living in the state.

Conclusion

Traverse strongly encourages the Oregon Health Authority to protect the core objectives of the Medicaid program and refrain from moving forward with the proposed outlined provisions above. Rare disease patients in the state rely on the Medicaid program for access to their needed treatments and care. We urge Oregon to revise its proposal before submitting to CMS and we look forward to working with you to advocate for better outcomes for all rare disease patients and families living in the state. If you have any questions, please do not hesitate to contact me or Rose Gallagher at 315-263-8463 or by email at rose.gallagher@traverse.com.

Sincerely,

A handwritten signature in black ink, appearing to read 'Chris Porter', written over a light blue grid background.

Christopher Porter
Vice President
Government Affairs and Policy
Traverse Therapeutics, Inc.

¹¹ <https://www.imcp.org/doi/pdf/10.18553/imcp.2014.20.7.677>