



BY ELECTRONIC DELIVERY

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, 5th Floor, E65
Salem, OR 97301

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

Dear Director Allen:

The Biotechnology Innovation Organization (BIO) and Oregon Bioscience Association (OR Bio) appreciate the opportunity to comment on the Oregon Health Authority's (OHA) proposed renewal and amendment of its Section 1115 waiver, which among other things, would be a waiver of compliance with essential provisions of §1927 of the Social Security Act (SSA). We urge the State to abandon this attempt and work with biopharmaceutical manufacturers, patient groups, and other key stakeholders to accomplish the goals of stability and predictability in the Medicaid pharmacy budget without jeopardizing patient access and care.

BIO is the world's largest trade association representing biotechnology companies, academic institutions, state biotechnology centers, and related organizations across the United States and in more than thirty other nations. OR Bio, a state affiliate of BIO, represents biotech companies throughout Oregon developing critical lifesaving drugs, advanced health-care technologies, and cutting-edge medical devices. OR Bio supports Oregon's bioscience community through networking, educational programs, enterprise support, advocacy, and the enhancement of research collaboration.

Our organizations represent thousands of large and small biotech companies throughout the country—including in Oregon. Our members span from those engaging in foundational research, to entrepreneurs, to major manufacturers and developers of therapies, cures, and devices. Our collective members develop medical products and technologies to treat patients afflicted with serious diseases, delay the onset of these diseases, or prevent them in the first place. In that way, our members' novel therapeutics, vaccines, and diagnostics yield not only improved health outcomes, but also reduced health care expenditures due to fewer physician office visits, hospitalizations, and surgical interventions.

The focus of our comment's centers on the OHA's request to waive §1927 of the SSA in order to adopt a "commercial-style" closed formulary for adults, leaving

many Medicaid beneficiaries with only one drug per formulary class, as well as its proposal to deny access to drugs approved via an accelerated pathway which will restrict access to drugs that address serious or life-threatening diseases with limited or no treatment options. The Centers for Medicare and Medicaid Services (CMS) has already soundly rejected previous such approaches because of its violation of Section 1927. To predictably manage drug expenditures, we recommend that Oregon focus on alternative, innovative payment strategies based on the value that balance patient care and positive health outcomes against the needs and limitations of the state's finite resources.

We support the OHA's purported goal of ameliorating disparities in the health care system but are deeply concerned that the policies being proposed in this waiver renewal and amendment application will have the opposite effect and instead exacerbate the inequities engrained in our health system. One of the primary tenets of BIO's own health equity agenda serves to promote health equity through:

- The enhancement of clinical trial diversity by partnering with contract research organizations and minority-serving institutions;
- Promotion of access to vaccines and therapeutics for uninsured and underserved populations, especially related to COVID-19; and
- Fostering enhanced nutritional, environmental, and mental wellness opportunities in economically disadvantaged communities.

We do agree that the state should leave in place an open formulary for children, a vulnerable patient group that already faces narrowed treatment options and for whom we must ensure access to medically necessary care, including therapies approved via accelerated pathways, where applicable.

Our specific comments on the OHA's Section 1115 Waiver Renewal and Amendment Application with respect to the proposal to waive §1902(a)(54) of the SSA, insofar as it incorporates §1927 are summarized as follows:

- **A closed formulary hinders access and jeopardizes the quality of care of the most vulnerable patients, especially those with rare, life-threatening diseases;**
- **Denial of access to drugs approved through one of FDA's expedited programs (accelerated approval) harms the most vulnerable patients;**
- **A closed formulary violates Section 1927 of the SSA, and Section 1115 of the SSA does not permit such a waiver; and**
- **Oregon could achieve better financial predictability in its pharmacy program by using its Preferred Drug List. Another option is to align payment to value through alternative payment mechanisms that emphasize value and outcomes.**

Our more detailed comments are outlined on the following pages:

A Closed Formulary Hinders Access and Jeopardizes the Quality of Care of the Most Vulnerable Patients

We have grave concerns that a waiver of compliance with SSA § 1927's coverage requirements would harm patient health by restricting access to medically necessary drugs. Consequently, in covering as few as one drug per therapeutic class, Oregon would seriously jeopardize the health of many patients, which will exacerbate growing inequities in access to health care. This is contrary to the application's purported goal: "to eliminate inequitable access with strategies to extend and stabilize coverage to every eligible child and adult in Oregon."¹

Studies show that restricting access to drugs through closed formularies results in non-adherence or poor adherence to prescribed medication regimens, worsened health outcomes and hospitalizations, not to mention other costs to the state such as additional costs to the state correctional system.^{2,3,4,5} One study found a causal relationship between formulary restrictions of schizophrenia drugs and increased incarcerations.⁶ For many therapeutic classes, such a culling of select drugs from overall drug classes would leave some Medicaid beneficiaries in a far worse position. Providing access to a wide variety of drug agents remains the cornerstone to improved patient care and health outcomes since one formulary agent may not produce the intended therapeutic outcome across all patient types. Also, side effect profiles can vary across patient subgroups, and a closed formulary design may lead to therapy discontinuation due to side effects. These concerns are even more heightened as science and innovation move toward personalized medicine, particularly in rare and chronic diseases. Below we highlight only a few examples in which a closed formulary is detrimental to patients.

Example: Epilepsy. Despite the availability of a variety of drug treatments for epilepsy, approximately 30% to 40% of all epilepsy patients still do not have the ability to adequately manage their seizures,⁷ and those patients who are able to manage their condition often must take up to three to five drugs at a time to

¹ Application for Renewal and Amendment: Oregon Health Plan Section 1115 Demonstration Waiver, Oregon Health Authority.

² Happe LE, Clark D, Holliday E, Young T. A systematic literature review assessing the directional impact of managed care formulary restrictions on medication adherence, clinical outcomes, economic outcomes, and health care resource utilization. *J. Managed Care Spec Pharm.* 2014;20(7):677-84. <https://www.jmcp.org/doi/pdf/10.18553/jmcp.2014.20.7.677> Accessed: December 10, 2021.

³ Zullig, LL, Bosworth, H, "Engaging patients to optimize medication adherence." NEJM Catalyst, May 14, 2017. <https://catalyst.nejm.org/doi/full/10.1056/CAT.17.0489> Accessed: December 10, 2021.

⁴ Seth A. Seabury, et al., "Formulary restrictions on atypical antipsychotics: impact on costs for patients with schizophrenia and bipolar disorder in Medicaid," 20 AM. J. MANAGED CARE e52 (2014). <https://www.ajmc.com/view/formulary-restrictions-on-atypical-antipsychotics-impact-on-costs-for-patients-with-schizophrenia-and-bipolar-disorder-in-medicaid> Accessed: December 10, 2021.

⁵ Yujin Park, et al., "The Effect of Formulary Restrictions on Patient and Payer Outcomes: A Systemic Literature Review," *Journal of Managed Care Pharmacy.* 2017 Aug; 23(8):893-901. <https://www.jmcp.org/doi/10.18553/jmcp.2017.23.8.893> Accessed: December 10, 2021.

⁶ Seabury, et al., Formulary Restrictions on atypical antipsychotics, 2014.

⁷ NIH Epilepsy Data: <https://www.ninds.nih.gov/Disorders/Patient-Caregiver-Education/Hope-Through-Research/Epilepsies-and-Seizures-Hope-Through>. Accessed: December 9, 2021.

control their seizures. According to the Centers for Disease Control and Prevention (CDC), there are roughly 42,900 people in Oregon suffering from epilepsy, 5,400 of which are children.⁸ For those on Medicaid, once these children reach 18 years of age, they would be subject to the closed formularies, potentially destabilizing their care. If the Oregon Health Plan were to limit coverage to a single drug in a therapeutic class, many epilepsy patients would be left without adequate therapy to manage their condition leading to costly acute care episodes and hospitalizations. Of additional concern, because epilepsy is more prevalent in Hispanic than non-Hispanic individuals, a closed formulary is likely to increase the health inequities that are already prevalent in this population.⁹

Example: Chronic Respiratory Diseases. Furthermore, access to necessary medications for appropriate care is especially important for adults and children with chronic conditions such as chronic lower respiratory disease, which often requires multiple medications to treat. This disease, which includes chronic obstructive pulmonary disease (COPD) and asthma is the fourth leading cause of death in the United States, with over 160,000 deaths in 2016.¹⁰ According to the CDC, approximately 369,735 Oregon adults (11.1%)¹¹ were affected by asthma, while another 229,000 (6.9%) of Oregonians were affected by COPD.¹² According to the Oregon Asthma Leadership Plan 2014-2019, at least 7% of children in Oregon are affected by Asthma.¹³ Again, these children would age into the Oregon Health Plan and be subject to a destabilizing closed formulary. Furthermore, the same report indicates that those on the Oregon Health Plan are disproportionately affected by Asthma.¹⁴ Given the prevalence of COPD and asthma in Oregon and the fact that the State has one of the highest rates of Asthma in the country,¹⁵ a drug formulary should accommodate standard medical science, accept treatment guidelines, and support new innovations to safeguard the medication needs of the State's population. A commercial style closed formulary design with one drug per class policy would not ensure patient access to current and innovative therapies for the appropriate treatment of respiratory diseases. It would further exacerbate health inequities in the State. According to the American Lung Association, African Americans and Native Americans have a higher prevalence of Asthma than other racial backgrounds. In fact, African Americans are 42% more likely to have Asthma than whites.¹⁶

⁸ <https://www.cdc.gov/epilepsy/data/index.html> Accessed: December 9, 2021.

⁹ <https://www.epilepsy.com/start-here/about-epilepsy-basics/who-gets-epilepsy>

¹⁰ CDC 2017 Most recent Asthma Data: https://www.cdc.gov/asthma/most_recent_data.htm Accessed: December 10, 2021.

¹¹ Id.

¹² American Lung Association, 2018. <https://www.lung.org/research/trends-in-lung-disease/copd-trends-brief/data-tables/copd-prevalence-rates-by-state-gender> Accessed: January 1, 2022.

¹³ Oregon Asthma Leadership Plan 2014-2019, Oregon Health Authority. <https://www.oregon.gov/oha/PH/DISEASES/CONDITIONS/CHRONICDISEASE/ASTHMA/Documents/alplan.pdf>. Accessed: December 10, 2021.

¹⁴ Id.

¹⁵ American Lung Association, 2018. <https://www.lung.org/research/trends-in-lung-disease/asthma-trends-brief/current-demographics> Accessed: December 29, 2021

¹⁶ Id.

Example: HIV. This is also true for people living with HIV. HIV treatments are not one-size-fits-all, and anti-retroviral therapy (ART) options may not be easily substituted for all patients. Health care providers work closely with patients to select treatment with great specificity for each patient. Patients may respond differently to the same ART. People living with HIV rely on open formularies because the effective treatment of HIV is highly individualized and accounts for a wide variety of factors. In fact, the HHS clinical treatment guidelines¹⁷ state that,

*"Selection of a regimen should be individualized based on virologic efficacy, potential adverse effects, childbearing potential and use of effective contraception, pill burden, dosing frequency, drug-drug interaction potential, comorbid conditions, cost, access, and resistance test results. . ."*¹⁸

The guidelines also recognize that "[i]t is important to consider the patient's daily schedule; patient tolerance of pill number, size and frequency; and any issues affecting absorption."¹⁹ Medical challenges for people living with HIV also include an increased risk for, and prevalence of, comorbidities that require additional drug treatment such as depression and substance use disorders,²⁰ as well as cardiovascular disease, hepatic and renal disease, osteoporosis, metabolic disorders, hypertension, hyperlipidemia, and endocrine disease and several non-AIDS-defining cancers.^{21,22,23,24} In addition, HIV impacts people of all ages and demographics, including children, adolescents, adults, women, men, etc. However, different HIV medicines may be more appropriate for different patient types. For example, not all treatment regimens are appropriate for pediatrics, pregnant mothers, or patients with resistance. For context, some drugs have low dose options available for younger/lower weight children; some drugs are not recommended for women of childbearing potential; patients with resistance to a certain class of HIV drugs would need to use drugs from a different class(es) to suppress their HIV. Accordingly, we must focus on maintaining access to all ART instead of limiting the number of HIV medicines. Closing the Oregon Health Plan formulary would restrict access to high-quality treatment for many people living with HIV at a time when ART can lower viral load to undetectable levels. This means that the virus is suppressed to levels that cannot be detected in blood tests and cannot be transmitted to others. Therefore, maintaining patient access to ART

¹⁷ DHHS Guidelines for the Use of Antiretroviral Agents in HIV-1-Infected Adults and Adolescents, 2021.

<https://clinicalinfo.hiv.gov/sites/default/files/guidelines/documents/AdultandAdolescentGL.pdf>. Accessed December 10, 2021.

¹⁸ Id.

¹⁹ Id.

²⁰ CDC, Medical Monitoring Project, United States, 2013 Cycle (June 2013–May 2014)

²¹ Gallant, Joel, Priscilla Y Hsue, Sanatan Shreay, Nicole Meyer; Comorbidities Among US Patients with Prevalent HIV Infection—A Trend Analysis, *The Journal of Infectious Diseases*, Volume 216, Issue 12, 19 December 2017, Pages 1525–1533, <https://doi.org/10.1093/infdis/jix518> Accessed: December 10, 2021.

²² Rodriguez-Penney, Alan T. et al. "Co-Morbidities in Persons Infected with HIV: Increased Burden with Older Age and Negative Effects on Health-Related Quality of Life." *AIDS Patient Care and STDs* 27.1 (2013): 5–16. PMC. Web. 21 June 2018. <https://www.ncbi.nlm.nih.gov/pmc/articles/PMC3545369/> Accessed: December 10, 2021.

²³ Joint HHS, CMCS, HRSA, and CDC Informational Bulletin, Opportunities to Improve HIV Prevention and Care Delivery to Medicaid and CHIP Beneficiaries, p. 9 (December 1, 2016), <https://www.medicaid.gov/federal-policy-guidance/downloads/cib120116.pdf>. Accessed December 10, 2021.

²⁴ Gallant, Joel, et al., December 19, 2017, <https://doi.org/10.1093/infdis/jix518>

options that are most clinically appropriate based on patients' individual factors is critical not just for the health of people living with HIV but also for the prevention of HIV for those at risk. The types of short-sighted policies, such as a closed formulary, can have poor public health consequences, increase health disparities, and can result in increased costs elsewhere in the budget.

Example: rare genetic diseases. Rare diseases can affect multiple organ systems and present with severe symptoms. The US Pharmacopeia (USP) category for genetic diseases lists many therapies that are the only therapy available for a particular disease state. Restricting access to only one, sole drug within a USP category (note: there are no "classes" offered for genetic disease, only "categories") would severely limit access to medically necessary therapy for patients with rare genetic diseases, a population that makes up some of the state's most vulnerable patients, who already experience incredibly limited treatment options.

Moreover, the State's assertion that a "closed formulary" would offer Medicaid beneficiaries coverage comparable to that of Medicare Part D beneficiaries is inaccurate. Unlike many Oregon Health Plan beneficiaries, Medicare Part D beneficiaries have a choice among multiple coverage options, with transparency into the drugs included on any individual formulary and protections against mid-year formulary changes. As a result, Medicare Part D beneficiaries can choose the formulary that best suits their medical needs with reasonable certainty that they will have such coverage over the next year. And, if their needs change, they can choose a new Part D plan during the annual open enrollment period. Furthermore, as the application notes, Medicare Part D formularies have two drugs per class, yet the State intends to limit coverage to just one drug per class. In addition, the Part D program has an exception and reconsideration process that provides access to off-formulary therapies, as well as six protected classes of drugs, for which Part D plans must include all, or substantially all, drugs in the class on-formulary — namely, the classes of anti-convulsants, anti-depressants, anti-psychotics, anti-neoplastics (oncology), immunosuppressants, and anti-retrovirals (HIV/AIDS). The OHA has described no such protections in its draft Waiver Application.

It is likewise the case that patients who obtain coverage through the health insurance exchanges can choose plans with a formulary best suited to their individual needs and can also change their plan during the annual open enrollment period as their health care needs change. In addition, these patients would have access to an exceptions process to obtain their medically necessary medications.

The Health Equity Committee, a subcommittee of the Oregon Health Policy Board (OHPB) was tasked with coordinating and developing policy that proactively promotes the eradication of health disparities and the achievement of health equity for all people in Oregon and the state has a goal of "eliminating health inequities by 2030."²⁵ This is an admirable goal for the state, one with which we support.

²⁵ Oregon Health Policy Board, Health Equity Committee, <https://www.oregon.gov/oha/OEI/Pages/Health-Equity-Committee.aspx> (Accessed: January 1, 2022).

However, we are concerned that the proposed closed formulary is incongruent with this policy.

Individuals with rare diseases tend to report common concerns that result from being underserved, such as a long road to diagnosis, limited treatment options, and a need for research to better understand their medical condition.²⁶ Additionally, rare diseases are disproportionately prevalent among some racial and ethnic minority groups. These patients may also face greater disease burden, earlier age of disease onset, and/or complex sets of comorbidities that limit the safety and effectiveness of older treatment options.

Rather than making progress toward Oregon's goal of eradicating health disparities, this draft Waiver Application's proposed closed formulary would likely create additional health disparities for rare disease patients, especially those who are also part of racial and/or ethnic minority groups. As part of two underserved populations, these individuals face substantial burdens which can cause economic hardship, difficulty accessing care, and poorer health outcomes for themselves and their caregivers. Without the ability to have coverage of a medicine that may not be included in the proposed closed formulary, these issues will be accentuated, and could result in poor health outcomes and increased costs of care, rather than achieving the state's goals of "eliminating health disparities and the achievement of health equity for all people in Oregon."

The suggestion that the Oregon Health Plan would maintain a high quality of care – while denying access to many medically necessary drugs – seems highly unlikely. The State did not even specify in its proposal whether patients might have access to an exceptions process or appeals process. Whether or not there is an exceptions process or appeals process, there are likely to be delays in treatment for thousands of Oregonians. Delays in access to treatment have been shown to lead to worsened patient outcomes. A recent study assessing time to treatment initiation (TTI) for cancer patients showed that increased TTI was associated with worsened overall survival for stages I and II breast, lung, renal, and pancreas cancers, and stage II colorectal cancers.²⁷

Today, meaningful therapies for people living with rare diseases are only available for 5% of more than 7,000 rare diseases, and the overall economic burden of just 379 rare diseases – including all direct medical costs as well as indirect nonmedical costs – is estimated at \$966 billion.²⁸ If the Oregon Health Plan succeeds in adopting its proposed closed formulary in Medicaid, patients will continue to struggle for access to high-quality health care and may delay care or forgo important aspects of life (e.g., getting married, buying a home, starting a family). This will be an especially acute problem for rare disease patients, for whom multiple

²⁶ "Barriers to Rare Disease Diagnosis, Care and Treatment: A 30-Year Comparative Analysis," *NORD Rare Insights*, NORD, November 19, 2020.

²⁷ Khorana AA, Tullio K, Elson P, et al. Increase in time to initiating cancer therapy and association with worsened survival in curative settings: A U.S. analysis of common solid tumors. *Journal of Clinical Oncology* 2017 35:15_suppl, 6557-6557. https://ascopubs.org/doi/abs/10.1200/JCO.2017.35.15_suppl.6557 Accessed: December 10, 2021.

²⁸ "The National Economic Burden of Rare Disease Study," Every life Foundation and National Health Council. 2021.

therapeutic options for treatment rarely exist. Furthermore, the accelerated approval pathway is a mechanism for expediting innovative treatments for patients with rare diseases too challenging to study using a traditional pathway, without compromising FDA's stringent, science-based approval standards. By denying access to new therapies approved through this pathway, Oregon could make it nearly impossible for rare disease patients who sometimes wait years to access a transformative medicine for their condition – concerns that we further detail in the following section.

Lastly, it is important to note that OHA currently lacks authority to enforce prior authorization for non-preferred drugs. Oregon has a long history of protecting access to needed medicines while also allowing OHA to manage utilization and cost through prior authorization. These protections were originally established in 1977 and were later developed as part of the Oregon Health Plan's Practitioner-Managed Prescription Drug Program (PMPDP).²⁹ Stakeholders negotiated changes to the PMPDP in 2009, allowing OHA limited prior authorization capabilities but prohibiting prior authorization criteria or requirements for unreviewed classes of drugs, mental health drugs, or refills of immunosuppressants and drugs to treat HIV/AIDS, cancer, or seizures.³⁰ These changes preserved the long-standing "prescriber prevails" protections allowing a provider to prescribe medicines subject to PA requirements.

Due to the ongoing attempts to appropriately balance access and costs, the Oregon Legislature has not granted OHA permanent authority to establish and enforce prior authorization requirements. After portions of statutory authority establishing the PMPDP sunset in early 2018, the Legislature has declined to re-implement that authority.³¹ OHA supported legislation in 2021 to re-institute prior authorization authority, with the historic provider and patient protections, but that effort failed to pass, continuing to leave OHA without statutory authority to enforce prior authorization requirements for non-listed drugs.³² Even if CMS were to authorize a closed formulary as part of a Section 1115 Demonstration Waiver, the OHA would still need explicit authority under state law to require prior authorization for those drugs not listed on the formulary.

Denial of Access to Drugs Approved through FDA's Accelerated Approval Pathway Harms the Most Vulnerable Patients

The State proposes that it should have the ability to exclude drugs approved through FDA's accelerated approval process from its formulary because they have limited or inadequate clinical efficacy.³³ CMS notes in *State Release 185* that these drugs must be covered by the Medicaid program if there is a signed Medicaid

²⁹ See ORS 414.325, enacted in 1977 and ORS 414.334 "Oregon Practitioner Managed Prescription Drug Program," enacted in 2001.

³⁰ See HB 2126 of 2009, which created ORS 414.337

³¹ Portions of ORS 414.337 expired on January 2, 2018. See also HB 2678 of 2019.

³² See SB 848 of 2021.

³³ Oregon Health Plan Section 1115 Waiver Renewal and Amendment.

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,

“Section 506(c) of the FFDCA allows the FDA to grant accelerated approval to a drug for a serious or life-threatening disease or condition. Part of the criteria for accelerated approval under section 506(c) is a demonstrated effect on either:

- “a. A surrogate endpoint that is reasonably likely to predict a clinical benefit, taking into account severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments, or
- b. A clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments.

“Drugs granted accelerated approval by FDA under the process described in 506(c) of the FFDCA are approved under section 505(c) of the FFDCA and **must meet the same statutory evidentiary standards for safety and effectiveness as those granted traditional approvals.** See section 506(e)(2) of the FFDCA. 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 surrogate endpoint that is reasonably likely to predict clinical benefit or on a clinical endpoint other than survival or irreversible morbidity.”³⁴

The FDA, the scientific community, and Congress³⁵ have all deemed surrogate endpoints as an appropriate marker of clinical efficacy for serious and life-threatening diseases and conditions for which there are no other meaningful alternatives. In the case of diseases that take course over a long period of time (e.g., nephrology disease or respiratory disease), surrogate endpoints are critical because without the pathway for researchers to feasibly study the ultimate, long-term impact on clinical outcomes through clinical trials it would require years, or even decades, of study, denying seriously ill patients medicines during the long wait. Additionally, the pathway is a lifeline for patients who have severe life-threatening diseases, including those with rare diseases, cancers, or HIV/AIDS. By attempting to limit Medicaid beneficiaries’ access to therapies for which only surrogate endpoints have been reported, Oregon would deprive patients of access to important therapy options approved by the FDA as safe and efficacious.

For nearly 30 years, FDA and Congress have both been clear in affirming that accelerated approval does not dilute or otherwise compromise FDA’s approval standards. FDA similarly responded to concerns that the accelerated approval

³⁴ *State Release 185*, CMS, June 27, 2018. (Emphasis added.)

³⁵ *Food Drug Administration Safety and Innovation Act*, §901.

process was inconsistent with the substantial evidence requirement of section 505(d) of the Food, Drug, and Cosmetic Act (21 U.S.C. § 355(d)):

“Approval under this rule requires ... that the effect shown be, in the judgment of the agency, clinically meaningful, and of such importance as to outweigh the risks of treatment. This judgment does not represent either a ‘lower standard’ or one inconsistent with section 505(d) of the act, but rather an assessment about whether different types of data show that the same statutory standard has been met.”³⁶

The State appears to suggest that Oregon can determine the safety and clinical efficacy of a drug in a manner superior to that of the FDA, which is considered the worldwide gold standard in the review and efficacy of drugs. The State is attempting to thwart the goals of the Federal Food, Drug and Cosmetic Act (FDCA), which tasks the FDA with applying its expertise to speed the development of medicines for serious diseases while maintaining its rigorous approval standards. Furthermore, this new type of decision-making outside the FDA could lead to unequal treatment access for patients already dealing with serious, life-threatening diseases. As 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 one 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.”

As specified by Congress, the FDA may consider the use of accelerated approval to

“a product for a serious or life-threatening disease or condition . . . upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments.”³⁷

We also believe the State makes an erroneous misinterpretation of the 21st Century Cures Act, incorrectly stating, “the 21st Century Cures Act was intended to expedite the drug approval process by reducing the level of evidence required for drugs to reach the market and allowing doctors, patients, and payers to decide whether to purchase them.”³⁸ Drugs approved through the accelerated approval pathway are subject to a demanding standard of review —demonstration of “substantial

³⁶ 57 Fed. Reg. at 58944.

³⁷ 21 U.S.C. § 356(a)(1).

³⁸ Oregon Health Plan Section 1115 Waiver Renewal and Amendment Application.

evidence” of effectiveness.³⁹ 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.⁴⁰ 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.⁴¹ If the confirmatory trial fails to verify the benefit, the FDA has the authority to withdraw approval and has done so when needed.⁴²

We strongly object to the exclusion of such categories of drugs in Medicaid not just because the statute and regulations demand it, but because these drugs must go through the same rigorous clinical review as other drugs. These drugs provide treatment for unmet medical needs, and most patients have limited or no current treatment options available to them. If the State excludes accelerated approval drugs from coverage, these patients will no longer have any treatment options.

For patients with rare diseases, this policy would have a devastating impact. Of the 7,000 rare diseases that exist, only 5% have treatment options available. Many of these patients have waited years for treatments only to not be able to access it, however, a patient living next door that has commercial insurance likely can. This would greatly exacerbate inequities in the State. In addition, exclusion from coverage removes a strong incentive for biopharmaceutical companies to research and develop these important innovative therapies, further exacerbating the inequities that are inherent in rare disease patients. In addition, studies have shown that accelerated approval drugs have accounted for less than 1% of Medicaid pharmacy costs between 2007 and 2018.⁴³ It would be deeply troubling to deny care to rare and chronic disease patients, who have few or no treatments, simply to gain a negligible financial impact.

Furthermore, studies have found that certain therapies reviewed under expedited programs, including the accelerated approval pathway, have offered greater medical gains than drugs reviewed through the FDA’s traditional, lengthier process.⁴⁴ A further consideration is that this approval pathway was created principally in response to the HIV/AIDS crisis of the 1980s as well as in response to calls that cancer drugs were not being developed quickly enough. We have seen the trajectory of those diseases change remarkably due, in large part, to the therapies developed under this FDA accelerated approval pathway. Should the State decide

³⁹ 21 U.S.C § 355(d)(5).

⁴⁰ 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.

⁴¹ FDA. Guidance for Industry: Expedited Programs for Serious Conditions – Drugs and Biologics. May 2014.

⁴² FDA. Delivering Promising New Medicines Without Sacrificing Safety and Efficacy. FDA Voices: Perspectives from FDA Leadership and Experts. August 2019.

⁴³ Thorpe, Kenneth E., Ph. D, and Douglas Holtz-Eakin, Ph.D., “Limiting Medicaid Access to Accelerated Approval Drugs: Costs and Consequences,” *American Journal of Managed Care*, March 30, 2021. Accessed: December 30, 2021. <https://www.ajmc.com/view/limiting-medicare-access-to-accelerated-approval-drugs-costs-and-consequences>

⁴⁴ 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.

not to cover these drugs until their FDA-required confirmatory studies are complete, then it will jeopardize the incentives for the research and development of these treatments, once again leaving these vulnerable patients with few, if any, options.

In the waiver renewal and amendment application, the State also appears to indicate its intention to restrict coverage for accelerated approval drugs and beyond, which would functionally exclude most drugs from coverage because the FDA's drug approval framework does not require evidence of "incremental benefit" over existing therapies to demonstrate safety and efficacy. This concept is deeply troubling as it fundamentally undermines the FDA's ability to evaluate and approve a safe and efficacious drug for the US public, leaving the general public questioning whether their own medical therapies are really safe and effective.

The Closed Formulary Violates Section 1927 and Section 1115 Does Not Permit Such a Waiver.

The OHA stated intent is to waive §1927 of the SSA to adopt a "commercial-style" closed formulary and circumvent the formulary requirements of §1927(d)(4). The waiver amendment application also suggests the Oregon Health Plan would utilize the "new flexibility" it seeks under the waiver to deny access to new innovative drugs approved through the FDA accelerated approval process, because "they have not yet demonstrated actual clinical benefit and have been studied in clinical trials using only surrogate endpoints." Accelerated approval is reserved for drugs that address serious or life-threatening diseases with limited or no treatment options and, *importantly*, are proven safe and effective by the same rigorous evidentiary standards used by the FDA to approve all other medicines.⁴⁵

The Medicaid rebate provisions of the SSA represent a carefully balanced compromise made by Congress to ensure the Government has access to the lowest available price for covered outpatient prescription medicines – via a statutorily mandated rebate – while also ensuring that manufacturers' products would be accessible to Medicaid recipients if medically necessary and subject to statutorily defined access restrictions.

[Section 1927] sets forth requirements for covered outpatient drugs, whereby drug manufacturers must pay statutorily defined rebates to the states through the Medicaid drug rebate program. In return, any state that provides payment for drugs **must cover all covered outpatient drugs**, which may include appropriate limitations on amount, duration, and scope, for the drug manufacturers that participate in the Medicaid drug rebate program.⁴⁶

⁴⁵ 21 U.S.C. §356(e)(2).

⁴⁶ 78 Fed. Reg. 4594, 4631 (Jan. 22, 2013). (Emphasis Added)

The Medicaid program is guaranteed a manufacturer's "best price," as defined in the statute, and in addition, receives an inflationary rebate to protect states from price increases that rise above the consumer price index.

In 2017, the Commonwealth of Massachusetts proposed a plan to reform its Medicaid pharmacy program to waive §1902(a)(54) of the SSA, insofar as it incorporates §1927, in an attempt to circumvent the Medicaid drug formulary requirements of §1927(d)(4). It proposed a closed formulary with at least one drug per class, with the intent to exclude drugs approved through the FDA's accelerated approval process. Both policies were firmly rejected by CMS, indicating that a state cannot simply opt out of §1927 and not provide access to "covered outpatient drugs" for which a manufacturer has a signed National Rebate Agreement.⁴⁷ The same day that CMS responded to the Massachusetts waiver amendment, the agency issued "State Release No. 185," which underscored the fact that drugs approved through the FDA's expedited approval processes "must be covered by state Medicaid programs, if the drug meets the definition of "covered outpatient drug" as found in Section 1927 of the Social Security Act"⁴⁸ and the Manufacturer has a signed Medicaid National Rebate agreement.⁴⁹

Given the nearly identical components of the Oregon and Massachusetts proposals, we urge the State to abandon this attempt and work with manufacturers and CMS on other alternatives, which are discussed later in this letter.

Furthermore, the Medicaid bargain is a favorable one for state Medicaid programs, because states can already negotiate rebates higher than what is statutorily mandated, it is dubious that states can negotiate better rebates by opting out of the statute. According to the Georgetown University Health Policy Institute, "Medicaid obtains rebates that are far larger than those in Medicare Part D and in private insurance."⁵⁰ According to the June 2021 Report to Congress, the Medicaid and CHIP Payment and Access Commission noted that the Medicaid program received \$37.1 billion in rebates from pharmaceutical manufacturers in 2019.⁵¹ This resulted in a net reduction in pharmacy spending of 55.6%.⁵² The Medicare Part D

⁴⁷ CMS letter to Asst. Secretary Tsai, MassHealth, June 27, 2018.

⁴⁸ CMS State Release No. 185, June 27, 2018.

⁴⁹ Tennessee also sought and obtained approval for a Section 1115 demonstration, which includes authority to implement a commercial style closed drug formulary, with certain exceptions. CMS letter to Dir. Stephen Smith, TennCare, January 8, 2021. Following an Administrative Procedures Act (APA) challenge to CMS' decision, CMS issued and opened a new comment period regarding the Tennessee demonstration and will issue a decision with respect to whether it will make any changes to its approval of the TennCare III demonstration. CMS letter to Dir. Stephen Smith, TennCare, August 10, 2021. The Massachusetts and Oregon proposed plans are distinct from the TennCare waiver, which was tied to a block grant. However, BIO has concerns that the TennCare demonstration also presents risks for Medicaid beneficiaries who rely on prescription drugs to treat acute conditions or manage chronic health needs.

⁵⁰ "How to Strengthen the Medicaid Drug Rebate Program to Address Rising Medicaid Prescription Drug Costs," Issue Brief, Georgetown University Health Policy Institute, January 2019.

⁵¹ Report to Congress, MACPAC, June 2021. <https://www.macpac.gov/wp-content/uploads/2021/06/June-2021-Report-to-Congress-on-Medicaid-and-CHIP.pdf> Accessed: December 9, 2021.

⁵² BIO Calculation from: MACPAC Report to Congress 2021.

program only received 26.5% in rebates in 2020.⁵³ In short, the Medicaid Drug Rebate Program works to achieve both the intended goals, ensuring patient access to much-needed medicines at the lowest prices in the commercial marketplace.

The Proposed Waiver Amendment Does Not Further the Objectives of the Medicaid Program and Therefore is Not Authorized by SSA § 1115

Under SSA § 1115(a), a state's proposed waiver must set forth an "experimental, pilot, or demonstration project," that, in the judgment of the Secretary, is "likely to assist in promoting the objectives of title XIX [i.e., the Medicaid program]." ⁵⁴ A waiver of compliance with SSA § 1927 would fail to satisfy these criteria.

In the first instance, the State has not specified a research proposition that it seeks to test to improve patient care for Medicaid enrollees. It proposes only to cut costs by restricting coverage of covered outpatient drugs that it would otherwise be required to cover under SSA § 1927. As the Court of Appeals for the Ninth Circuit has emphasized, "[SSA § 1115] was not enacted to enable states to save money or to evade federal requirements, but to test out new ideas and ways of dealing with the problems of Medicaid recipients"⁵⁵...such "[a] simple benefits cut, which might save money but has no research or experimental goal, would not satisfy th[e] criteria [of] ha[ving] a research or demonstration value."⁵⁶ SSA § 1115 demonstration projects must test innovative approaches aimed at furthering the objectives of the Medicaid program, for example, by enhancing the quality of care or promoting efficient administration. A demonstration project may not operate as a mere benefit cut with no actual experimental value.

Additionally, a waiver of compliance with SSA § 1927 would fail to promote the objectives of title XIX, which was enacted by Congress to provide medical care to the needy and medically needy.⁵⁷ By denying access to otherwise-covered and potentially life-saving therapies, the State would do precisely the opposite – strip away medical care for the needy and medically needy, exacerbating health disparities in the process. Such a waiver would also fail to promote Congress's clear objectives in enacting SSA § 1927 in particular – indeed, it would contradict these objectives. Congress enacted SSA § 1927 in order to guarantee that "[s]tates that elect to offer prescription drugs ... cover all the products of any manufacturer that agrees to provide price rebates."⁵⁸ If CMS were to approve a waiver of compliance that enables a state to avoid its drug coverage obligations under SSA § 1927, the agency would undermine the primary objective of SSA § 1927, as stated by

⁵³ 2021 Annual Report of the Board of Trustees of the Federal Hospital Insurance and the Federal Supplementary Insurance Trust Funds, 2021.

⁵⁴ SSA § 1115(a).

⁵⁵ *Beno v. Shalala*, 30 F.3d 1057, 1069 (9th Cir. 1994).

⁵⁶ *Id.*

⁵⁷ Staff of H. Comm. on Ways and Means, 89th Cong., Summary of Major Provisions of H. R. 6675, The "Social Security Amendments of 1965" 1 (Comm. Print 1965).

⁵⁸ *Id.*

Congress itself. On top of this, the State would fail to ensure that “Medicaid beneficiaries have access to the same range of drugs that the private patients or their physicians enjoy,” as intended by Congress.⁵⁹ CMS confirmed this understanding in its June 27, 2019, letter to the Commonwealth of Massachusetts that rejected the nearly identical closed formulary proposal.

Oregon can achieve better financial predictability in its pharmacy program by using its Preferred Drug List (PDL). Oregon can also align payment to value through alternative payment mechanisms that emphasize value and outcomes.

We believe that Oregon would be better served by utilizing its PDL to manage drug costs and enacting a permanent structure for enforcing prior authorization. Lack of explicit statutory authority and a permanent, predictable system for patients, providers, and others have hindered effective implementation. As described above, Medicaid programs already obtain rebates that are far larger than those in Medicare Part D and in private insurance. Oregon could negotiate further, supplemental Medicaid rebates from manufacturers as a condition of product placement on the PDL.

Oregon’s current proposal also does not take advantage of potential structures to align the State’s payment for medicines with the value they deliver to patients and the Medicaid program. The State could seek a State Plan Amendment or waiver amendment that would allow the State the flexibility to negotiate voluntary alternative payment models that emphasize value, quality, and health outcomes. The success of the voluntary, innovative contracting approaches such as those approved in Oklahoma, Michigan, Colorado, Washington, and several more states have the potential to demonstrate beneficial outcomes, stability, and predictability in the financing, and continued future innovation.

- For example, some contracts are strictly linked to outcomes. If a drug does not produce certain metrics, such as a reduction in hospitalizations by patients using the drug, the manufacturer would receive a reduced payment or no payment.
- Other examples such as an amortized payment contract, or pay-over-time, would allow for the drug to be delivered up front for the patient and then the state would stretch out the payment over a set period of payments, with payments that could be tied to a health outcome (referred to as milestone payments); or alternatively, the subscription model in which manufacturers partner with states to agree upon pricing to treat a larger number of patients. These options can provide more predictable, value-driven financing for the appropriate disease states.

⁵⁹ H. Rep. No. 101-881, at 96-97 (1990).

Furthermore, we expect the new regulation due to take effect on July 1, 2022, permitting manufacturers to report multiple best prices, could increase the options states and manufacturers will have to enter into these novel agreements.

We strongly support innovative, voluntary negotiation between states and biopharmaceutical companies, which will, in turn, help ensure patient access to necessary therapies. We believe that value-, outcomes- or indication-based arrangements, and alternative payment models, can all have merits to both states and biopharmaceutical companies. BIO and Oregon Bio look forward to working with OHA to help the State understand the variety of arrangements that exist and flexibility they can provide to ensure new models can be advanced as health care evolves, and new medications are developed.

Thank you for the opportunity to submit comments on the OHA's Section 1115 Demonstration Waiver Renewal and Amendment application. BIO and Oregon Bio strongly urge the state to work with all stakeholders including the biopharmaceutical industry to ensure new policies do not severely jeopardize patient access to care, given our belief that OHA can achieve its objectives without any waiver of §1927.

Should you have any questions, please do not hesitate to contact me at (202) 962-9200 or at jgeisser@bio.org.

Sincerely,

/s/
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