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BY ELECTRONIC DELIVERY

April 1, 2022

The Honorable Chiquita Brooks-LaSure
Administrator, Centers for Medicare and Medicaid Services (CMS)
7500 Security Boulevard
Baltimore, MD 21244

RE: Application for Renewal and Amendment to the Oregon Health Plan, §1115 Demonstration Waiver

Dear Administrator Brooks-LaSure:

The Biotechnology Innovation Organization (BIO) appreciates the opportunity to comment on the Oregon Health Authority's (OHA) proposed Oregon Health Plan §1115 Demonstration Waiver Application (Waiver Application), which among other things, would be a waiver of compliance with essential provisions of §1927 of the Social Security Act (SSA) to exclude drugs approved through the FDA's accelerated approval pathway (AAP).[1] We urge CMS to reject those provisions of the proposed waiver that would exacerbate health disparities and jeopardize patient access and care, especially for patients with rare and chronic diseases.

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. BIO's members develop medical products and technologies to treat patients afflicted with serious diseases, to delay the onset of these diseases, or to 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.

We appreciate that Oregon has narrowed the scope of its waiver with respect to pharmacy benefits and eliminated its proposal for a closed formulary because it violates the coverage and access protections of §1927 of the SSA. However, the focus of our comments centers on another of the OHA's requests to waive §1927 of the SSA in order to deny access to drugs approved via the AAP, 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 such approaches because of their violation of §1927.

We support CMS' goal of ameliorating disparities in the health care system. One of the primary tenets of BIO's own health equity agenda serves to promote health equity through:

[1] 2022-2027 Oregon Health Plan § 1115 Waiver Application, Oregon Health Authority, February 18, 2022.

- 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.

Unfortunately, despite Oregon's stated intention to reduce health care disparity, we are deeply concerned that the policies proposed in the Waiver Application will have the opposite effect and instead exacerbate inequities that are deeply engrained in our health system.

While the Waiver Application addresses a variety of changes to the Medicaid program, the State proposes that it should have the ability to exclude coverage of drugs approved through FDA's AAP that have not had benefit confirmed with conversion to full FDA approval in the expected time interval.[2] The State incorrectly asserts that these drugs' evidence of effectiveness at approval is less than other drugs approved through the traditional FDA approval pathway. While many details are not delineated in the Waiver Application and the current language seems obscure, the State of Oregon also appears to be requesting the ability to cover AAP drugs until the FDA timeline for a confirmatory trial has expired. If a confirmatory trial has not been completed, the State would like the option to exclude these FDA-approved drugs from coverage. In addition, the State seems to intend to use its own undefined process to review drugs that have been previously approved by the FDA and have not completed their studies within the timeline estimated by the FDA to determine whether or not to cover them. The language in some parts of the Waiver Application is ambiguous and suggests that the State may want the ability to review drugs prior to the timeline expiring or the confirmatory trial being completed to determine whether to cover them, also by using peer-reviewed literature.

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

- **Denial of access to drugs approved through FDA's AAP jeopardizes patient care and exacerbates existing health care disparities while raising costs.**
- **Oregon's proposed Waiver Application does not further the objectives of the Medicaid program and therefore is not authorized by SSA § 1115.**
- **The denial of access to drugs approved through the AAP violates § 1927 and would undermine the FDA's authority to determine which drugs are safe and effective.**

[2] Oregon Health Plan § 1115 Waiver Renewal and Amendment Application.

- **Oregon's use of the prioritized list rations care and uses discriminatory Quality-adjusted Life Years (QALYs), which exacerbates health inequities and results in additional violations of §1927.**
- **Exploring alternative payment mechanisms would enhance Oregon's goal of aligning payment to value.**

Our more detailed comments are outlined on the following pages:

Denial of access to drugs approved through FDA's AAP jeopardizes patient care and exacerbates existing health care disparities while raising costs.

BIO strongly objects to the exclusion of drugs approved through the AAP 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. The pathway is a lifeline for patients who have severe life-threatening diseases, including those with rare diseases, cancers, or HIV/AIDS. BIO is deeply concerned that such a proposal to deny coverage will disrupt the continuity of care to vulnerable patients, many of whom may have been taking these drugs safely and with clinical benefit for years. These patients may have just one treatment available to them in many cases; this proposal would allow the state to abruptly stop coverage of a medication without opportunity for an appeal, which could have devastating, if not life-threatening, consequences. Disruptions in continuity of care can jeopardize patient health, potentially causing hospitalizations and acute care episodes, which also result in higher health care costs. For example, according to the US Government Accountability Office recently reviewed,

> "a comparison of costs incurred by people with pulmonary arterial hypertension (a disease affecting the heart and lungs) in the year after initiation of drug therapy to an estimate of their costs in the year before initiation of drug therapy suggests potential cost savings. Results indicated that, despite the increase in costs for drugs (from an average of $6,440 to $38,514 per person, in 2011 U.S. dollars), overall direct, all-cause medical costs decreased after initiation of drug therapy (from an average of $116,681 to $98,243 per person)."[3]

A reversal of drug therapy would see medical costs rise for these patients.

For patients with rare diseases, this policy would have a devastating impact. Of the 7,000 rare diseases that exist, only 5% have FDA-approved treatment options available.[4] Many of the approved treatments came through the AAP. If it were not

[3] "Rare Disease: Although Limited, Available Evidence Suggests Medical and Other Costs Can Be Substantial," US Government Accountability Office (GAO). October 2021.
[4] Rare Disease Day Fact Sheet, NORD, 2019.

for the pathway, many rare disease patients may have waited years longer for treatments. If this waiver is approved, a new scenario in Oregon would start, where only after a patient waits years for a potentially life-saving drug, the patient covered by Medicaid may not be able to access it, while a patient living next door who has commercial insurance likely can. It is even more troublesome when you consider that many rare diseases or chronic diseases with unmet needs have a higher prevalence in certain minorities than the general population, and a higher prevalence on Medicaid, 61.1% of enrollees on Medicaid identify as a minority,[5] a number that is roughly the same proportion in Oregon.[6] This would greatly exacerbate health care inequities in the State, which would have the opposite effect of both CMS and OHA's goal of ameliorating health disparities.

By attempting to limit Medicaid beneficiaries' access to these therapies, even if only for those where a confirmatory trial has not been completed in the established timeline, many of these patients will no longer have any treatment options.

The State of Oregon seems to suggest that an incomplete confirmatory trial deems the drug ineffective. There are a variety of reasons why a confirmatory trial may not be completed by the timetable established by the FDA that have nothing to do with the actual efficacy of a drug. Establishing hard triggers that prevent flexibility would have deleterious effects. By their nature, confirmatory studies for accelerated approval treatments are time-consuming and challenging. It is exactly because of these obstacles that the AAP is valuable in making lifesaving and life-changing drugs available to patients sooner. The challenges in conducting confirmatory post-marketing studies are real—randomized trials in a post-marketing setting can create ethical challenges; ultra-rare diseases present unique challenges in patient recruitment, given the small number of patients eligible to participate; and clinical outcomes for some conditions take many years to develop. An FDA analysis of its own data provides some "real world" examples of why a trial can be delayed:

- **Enrollment:**
  - "The applicant requested revised Trial Completion and Final Report Submission milestone due dates because of **difficulty in enrollment**. Revised milestones were acknowledged in a letter dated 10/01/2019."

- **Protocol:**
  - "The final protocol milestone was missed because the onset of this trial is **based on the results from another study**. Revised milestones were acknowledged in a letter issued in March 2017."[7]

The State's assertion that it needs to "incentivize" manufacturers to complete confirmatory trials is unfounded. FDA's own analysis of drugs approved from 1992 to 2012, demonstrates that the program is working as intended, and balancing risks

[5] "Racial and Ethnic Disparities in Medicaid: An Annotated Bibliography," *FactSheet*, MACPAC, April 2021.
[6] Monthly Medicaid Population Report, Oregon Health Authority, October 2020.
[7] Reagan-Udall Foundation for the FDA. Accelerated Approval Program: 30 Years On – Insights and Experiences. Virtual Public Meeting, pgs. 40-41. March 11, 2022.

against the value these important therapies bring to patients. Indeed, 94 out of 118 (80%) of drugs approved by the Center for Drug Evaluation and Review (CDER) converted to traditional approval, demonstrating clinical benefit, while 14% were withdrawn. Further, 14 of 16 biologics approved by the Center for Biologics Evaluation and Review in that same period were converted to traditional approval and one was withdrawn, while one has yet to be converted to traditional approval.[8] Further, a recent study of FDA-approved accelerated approval drugs before 2020 indicates that 75% demonstrated clinical benefit and were converted to traditional approval.[9]

Moreover, the supposed need for this proposal is dubious. The Waiver Application states that AAP therapies "tend to be specialty drugs" representing a "significant portion" of pharmacy costs. However, this seems to be more hyperbole than fact. In an analysis of CMS Data, the Partnership to Fight Chronic Disease found that, "in 2020, accelerated approval drugs accounted for only .4% of total Medicaid spending in Oregon and, between 2015 and 2020, the category represented just .5% of total Medicaid spending growth, relative to the 31% growth in total Medicaid spending in that state during that same span."[10] Permitting the State to deny necessary care to rare and chronic disease patients, who have few or no treatments available, simply to gain a negligible financial benefit.

Again, the State's suggestion that this policy approach would provide incentives for manufacturers to complete studies is not only unfounded, as mentioned previously, it is not necessarily the case. The FDA established specific guidelines on how studies need to be conducted by the biopharmaceutical manufacturer, are the only provisions that determine when a confirmatory study is in fact complete, not the State's opinion. The State is leveraging the health of its own population to advance a policy for which: it has no authority, or expertise; there is no evidence the policy drives the stated outcome; and there is negligible benefit to the budget. Further, the 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 and chronic disease patients.

Oregon's proposed Waiver Application 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]."[11] A

[8] Reagan-Udall Foundation for the FDA. Accelerated Approval Program: 30 Years On – Insights and Experiences. Virtual Public Meeting, pgs. 24-25. March 11, 2022.
[9] GK Raju, PhD., Presentation at the Rare Disease Legislative Advocates Congressional Caucus Briefing. The Accelerated Approval Pathway: Reflecting the Rare Disease Community's Priorities of Rigor, Safety and Urgency. February 22, 2022. Available at: https://www.youtube.com/watch?v=FoScXVVBVdw
[10] Thorpe, Kenneth, "Debunking Oregon's Cost Argument in Denying Access to Accelerated Approval Drugs, Partnership to Fight Chronic Disease. 2022. https://www.fightchronicdisease.org/blog/debunking-oregon%E2%80%99s-cost-argument-denying-access-accelerated-approval-drugs
[11] SSA § 1115(a).

waiver of compliance with SSA § 1927 would fail to satisfy these criteria. The Waiver Application states that, "[t]hrough this process, the state could *incentivize*[12] drug sponsors to complete their regulatory obligations to demonstrate clinical benefit as laid out by the FDA upon approval."[13] First, it is not the responsibility of the state to ensure a manufacturer fulfills its regulatory obligations to the FDA. Secondly, Oregon fails to describe and explain why this provision would promote the objectives of the Medicaid program.

Indeed, the State has not specified a research proposition that it seeks to test to improve patient care for Medicaid enrollees. Oregon proposes only to cut benefits and 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"[14]…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."[15] 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 are to provide medical care to the needy and medically needy.[16] By denying access to otherwise-covered and potentially life-saving therapies for rare and chronic diseases that have few or no treatments available, the State would do precisely the opposite – strip away medical care for the needy and medically needy, exacerbating health disparities in the process. 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."[17] If CMS were to approve a waiver that enables a state to avoid its drug coverage obligations under SSA § 1927, the agency would undermine this primary objective of SSA § 1927. 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.[18]

[12] Emphasis added.
[13] 2022-2027 Oregon Health Plan Waiver Application, Oregon Health Authority, February 18, 2022.
[14] *Beno v. Shalala,* 30 F.3d 1057, 1069 (9th Cir. 1994).
[15] Id.
[16] 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).
[17] Id.
[18] H. Rep. No. 101-881, at 96-97 (1990).

The denial of access to drugs approved through the AAP violates § 1927 and would undermine the FDA's authority to determine which drugs are safe and effective.

The Waiver Application also suggests the Oregon Health Plan would utilize the "new flexibility" it seeks under the waiver to deny access to innovative drugs approved through the FDA AAP, because "they have not yet demonstrated actual clinical benefit and have been studied in clinical trials using only surrogate endpoints."[19] 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.[20]

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.[21]

The Medicaid program is guaranteed a rebate of 23.1% or the manufacturer's "Best Price," whichever price is lower, and in addition, receives an inflationary rebate to protect states from price increases that rise above the consumer price index. In return for the Best Price, patients are granted access to all medically accepted covered outpatient drugs for which the manufacturer has a signed Medicaid National Rebate Agreement. AAP therapies are subject to these same requirements. If CMS permits the State of Oregon to pick and choose which specific drugs it decides to cover, it will leave the access and coverage protections of §1927 toothless.

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). Massachusetts proposed a closed formulary with at least one drug per class, with the additional intent to exclude drugs approved

[19] OHP 1115 Waiver Application, February 2022.
[20] 21 U.S.C. §356(e)(2).
[21] 78 Fed. Reg. 4594, 4631 (Jan. 22, 2013). (Emphasis Added)

through FDA's AAP. Both requests were firmly rejected by CMS.[22] The Agency issued "State Release No. 185," which reinforced to state Medicaid programs 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 §1927 of the Social Security Act"[23] and the Manufacturer has a signed Medicaid National Rebate agreement.[24] Oregon's proposed waiver is directly at odds with CMS's prior decisions and guidance, as well as the language and structure of §1927 of the SSA.

Such a targeted approach to specific types of drugs based upon FDA approval pathways has never been done before and undermines the FDA's authority as well as the intent of the Medicaid Drug Rebate Program. It differs from the closed formulary proposals because it is not a scenario where the state is trying to negotiate rebates by driving purchasing volume in a commercial-style formulary. The State makes no mention of trying to negotiate different rebates for AAP therapies. This is simply the State of Oregon cutting benefits and trying to circumvent §1927, which has clearly defined what is a "covered outpatient drug" for the purposes of the program.

AAP Therapies Go Through the Same Rigorous Approval Process as Drugs Approved in the Traditional Review Process.

The State asserts that drugs approved through the AAP have not been shown to be clinically effective, and it suggests that the accelerated approval drugs have not gone through the same approval process as drugs that go through the traditional approval pathway. However, as CMS also noted in *State Release 185,* these drugs go through the same rigorous approval process as other covered outpatient drugs. *State Release 185* specifically adds that,

> "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

[22] CMS letter to Asst. Secretary Tsai, MassHealth, June 27, 2018.
[23] CMS State Release No. 185, June 27, 2018.
[24]Tennessee also sought and obtained approval for a § 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.

> 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."[25]

The FDA, the scientific community, and Congress[26] 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 lengthy period of time (e.g., nephrology or respiratory disease), surrogate endpoints are critical because it would otherwise require years, or even decades, for researchers to feasibly study the ultimate, long-term impact on clinical outcomes through clinical trials, denying seriously ill patients medicines during the long wait. This period of waiting could mean the difference between life and death for many patients, including those with rare and chronic diseases.

For nearly 30 years, FDA and Congress have both been clear in affirming that the AAP does not dilute or otherwise compromise FDA's approval standards. FDA similarly responded to concerns that the AAP was inconsistent with the substantial evidence requirement of § 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."[27]

The State appears to suggest that it 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. In doing so, the State

[25] *State Release 185*, CMS, June 27, 2018. (Emphasis added.)
[26] *Food Drug Administration Safety and Innovation Act*, §901.
[27] 57 Fed. Reg. at 58944.

provides no detail as to how it would conduct this rigorous review, and as submitted the provides for no process for stakeholder comment or CMS review. The State effectively asks CMS for permission to thwart the goals of the Federal Food, Drug and Cosmetic Act (FDCA), which tasks the FDA –and only 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. Indeed, if approved, Oregon could have different coverage rules than neighboring Washington State, or Alabama, or New York. This dis-uniformity is inconsistent with Congress' mandate in the Medicaid statute, which was to provide a broad set of national coverage rules, and also could usurp the FDA's vital role in approving medicines. 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 the AAP for

> "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."[28]

The State also misinterprets *the 21st Century Cures Act*, asserting, "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."[29] Drugs approved through the AAP are subject to the same demanding standard of review —demonstration of "substantial evidence" of effectiveness.[30] In fact, studies have found that certain drugs reviewed under the AAP have offered greater medical gains than drugs reviewed through the FDA's traditional, lengthier process.[31] 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

[28] 21 U.S.C. § 356(a)(1).
[29] Oregon Health Plan § 1115 Waiver Renewal and Amendment Application.
[30] 21 U.S.C § 355(d)(5).
[31] 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.

clinical benefit.[32] If the confirmatory trial fails to verify the benefit, the FDA has the authority to withdraw approval and has done so when needed.[33] Indeed, only 8% of the 269 drugs approved through the AAP have been withdrawn, ten (10) of them for safety concerns, ten (10) were withdrawn for failing to demonstrate efficacy in the confirmatory trial, and notably, four (4) were withdrawn because confirmatory trials could not be completed due to low enrollment,[34] which again, highlights the difficulty in completing some confirmatory trials.

Oregon's use of the prioritized list rations care and uses discriminatory QALYs, which exacerbates health inequities and violates §1927.

We are deeply concerned that the State of Oregon continues to use its "Prioritized List of Health Services" (the List). The List is a set of services the State will pay for in Medicaid. If a service or treatment falls below a certain cut-off line, as established each year by the Health Evidence Review Commission (HERC), then it is denied, effectively rationing care. Unfortunately, in the Waiver Application, the State indicates that it fully intends to continue this practice. It has used this system in the past to conduct reviews of medications. The HERC still utilizes QALYs to come to its conclusion about many medications it will cover. Should the State be granted the flexibility to determine which AAP drugs would be covered, they will use this power to engage in expanded drug utilization review with the potential for discriminatory rationing of care.

QALYs are used by many technology assessment organizations, such as the Institute for Clinical and Economic Review (ICER), despite the federal government recognizing QALYs are inherently discriminatory to patients with chronic disease and disability. In its November 2019 report on QALYs, the National Council on Disability (NCD) "found sufficient evidence of QALYs being discriminatory (or potentially discriminatory) to warrant concern."[35] Further, studies have shown that countries that use QALYs have severe restrictions on patient access to innovative medicines. For example, one study has shown that, between 2002 and 2014, 40% of medicines that treat rare diseases were rejected for coverage in the United Kingdom.[36]

Indeed, Congress saw fit to ban the use of QALYs by the Patient-Centered Outcomes Research Institute (PCORI) when it created the organization in 2010. The NCD Report called on Congress to pass legislation prohibiting the use of QALYs in Medicare and Medicaid. In addition, it encouraged CMS to use alternative

[32] FDA. Guidance for Industry: Expedited Programs for Serious Conditions – Drugs and Biologics. May 2014.
[33] FDA. Delivering Promising New Medicines Without Sacrificing Safety and Efficacy. FDA Voices: Perspectives from FDA Leadership and Experts. August 2019.
[34] https://www.fda.gov/drugs/nda-and-bla-approvals/accelerated-approvals
[35] "Quality-Adjusted Life Years and the Devaluation of Life with Disability," National Council on Disability, November 6, 2019.
[36] Mardiguian, S., Stefanidou, M., et al. "Trends and key decision drivers for rejecting an orphan drug submission across five different HTA agencies." Value in Health Journal. 2014.
https://www.valueinhealthjournal.com/article/S1098-3015(14)03070-8/fulltext

measurements of value when "the exact cost and benefits of a drug or treatment are not known."[37]

We believe CMS should follow the recommendations in the NCD report and reject the use of QALYs when considering the Oregon Waiver Application.

Exploring alternative payment mechanisms would enhance Oregon's goal of aligning payment to value.

Oregon's Waiver Application emphasizes the willingness to align its payment for medical services with the value they bring to the patient and the Oregon Health Plan. Yet, Oregon's proposal fails to take advantage of alternative structures that have the potential to better align the State's payment for medicines with the value they deliver to patients and the Medicaid program, and simply tries to cut available benefits by demanding the authority not to cover AAP drugs.[38] CMS saw fit to allow State Plan Amendments[39] and promulgate regulations due to take effect on July 1, 2022, permitting manufacturers to negotiate voluntary value-based purchasing agreements. Oregon could pursue its stated interest in aligning payment to value under this regulatory structure and seek voluntary agreements that ensure coverage of transformative therapies, when appropriate.

BIO strongly supports innovative, voluntary negotiation between states and biopharmaceutical companies, which we believe, in turn, has the potential to help ensure patient access to necessary therapies. We believe that voluntary value-, outcomes-, or indication-based arrangements, and alternative payment models, can all have merits to both states and biopharmaceutical companies.

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[37] QALYs and the Devaluation of Life, NCD. November 2019.
[38] 2022-2027 OHP Waiver Application, February 18, 2022.
[39] Value-based State Plan Amendments approved in: Alabama, Arizona, Colorado, Louisiana, Massachusetts, Michigan, Oklahoma, Texas, and Washington. (Several other states are reportedly considering.)

Thank you for the opportunity to submit comments on the OHA's 2022-2027 Oregon Health Plan § 1115 Demonstration Waiver Application. BIO strongly urges CMS to disapprove of the proposed policies regarding AAP therapies so they 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/

Jack Geisser
Sr. Director, Healthcare Policy, Medicaid, & State Initiatives