

January 7, 2022

Director Patrick Allen
Oregon Health Authority
Health Policy and Analytics Medicaid Waiver Renewal Team
Attn: Michelle Hatfield
500 Summer St. NE, 5th Floor, E65
Salem, OR 97301

BY ELECTRONIC DELIVERY

Re: Comments on the Oregon Health Plan (OHP) 1115(a) Draft Demonstration Waiver Renewal Application

Dear Director Allen and Team:

Novartis Services, Inc. is submitting this letter on behalf of Novartis Pharmaceuticals Corporation, Advanced Accelerator Applications USA, Inc. (AAA), and Sandoz Inc., collectively referred herein as "Novartis."

Novartis appreciates the opportunity to comment on the Oregon Health Authority's (OHA) Oregon Health Plan (OHP) 1115(a) Demonstration Waiver Renewal Application (proposed waiver),¹ which is intended for submission to the Centers for Medicare & Medicaid Services (CMS). Novartis provides healthcare solutions that address the evolving needs of patients and societies worldwide. Our broad portfolio of medicines includes treatments in the areas of: ophthalmics; neuroscience; immunology, hepatology and dermatology; respiratory and allergy; cardiovascular, renal and metabolism; oncology, including targeted therapies, immuno-oncology, chimeric antigen receptor T cells (CAR-T) and radioligand therapy; and gene therapies. We are also a global leader in generic and biosimilar medicines, committed to playing a leading role in driving access to medicine worldwide.

At Novartis, we are united by a single purpose to reimagine medicine to improve and extend people's lives. Through innovative science and technology, we address some of society's most challenging healthcare issues. Every day we work to discover and develop breakthrough treatments and find new ways to deliver them to as many people as possible. Our vision is to be a trusted leader in changing the practice of medicine.

Novartis applauds Oregon for addressing the significant unmet need of patients through health equity initiatives. Novartis believes an important way we can achieve greater health equity is to address persistent disparities in the way healthcare is approached, accessed, and delivered. However, the proposed waiver presents several fundamental legal and policy-based concerns that may harm Medicaid beneficiaries. Specifically, the proposed waiver:

- Undermines the bargain that Congress struck in enacting the Medicaid Drug Rebate Program (MDRP) and contradicts the basic terms of the Medicaid rebate agreement (MDRA), which have contributed to the MDRP's success over the past

¹ OHA, Application for Renewal and Amendment, Oregon Health Plan 1115 Demonstration Waiver 30–32 (as revised Dec. 7, 2021).

- three decades;
- Excludes safeguards that Congress has affirmed as essential in the provision of services within state Medicaid programs;
- Seeks to limit drug coverage in a manner inconsistent with federal law and that would fundamentally harm Medicaid beneficiaries;
- Fails to satisfy the statutory requirements for a Section 1115 demonstration program; and
- Restricts access to life-saving medications which leads to worse outcomes and runs counter to the OHA's mission of health equity.

We firmly believe that OHA should revise its approach, and not proceed with the proposed waiver as written on several grounds. The proposed waiver should instead pursue implementation of alternative payment methodologies and stay focused on health equity for all OHP beneficiaries.

I. The Proposed Waiver Violates the Bargain Underlying the Medicaid Drug Rebate Program Enshrined in the Medicaid Drug Rebate Agreement

First, the proposed waiver violates the bargain struck by Congress in enacting the MDRP, Social Security Act (SSA) § 1927. SSA § 1927 codifies a carefully constructed compromise between manufacturers and states, which is memorialized in the MDRA that manufacturers enter into with the Secretary of Health and Human Services (HHS). Under the MDRA, manufacturers agree that, in exchange for federal approval of the payment by state Medicaid programs for all of their Food and Drug Administration (FDA) approved covered outpatient drugs, the manufacturers will make generous rebate payments to the states on such drugs “for as long as an agreement with the Secretary is in force and state utilization data reports that payment was made for that drug.”²

As a result of this agreement between states and manufacturers, over the past several decades SSA § 1927 has helped provide Medicaid beneficiaries with access to critical, innovative, life-saving therapies and has helped ensure that states receive substantial rebates on the cost of those therapies. The proposed waiver would introduce an unprecedented and inappropriate departure from this long-standing arrangement. In simple terms, OHA is proposing to waive only its obligations under SSA § 1927 — to provide coverage of all covered outpatient drugs of a manufacturer that has entered into a MDRA. The state, however, appears to anticipate that manufacturers continue paying rebates on the state Medicaid program's utilization of its drugs.

This proposal is fundamentally inconsistent with the Congressional intent underlying SSA § 1927 and with the rebate agreement memorializing the terms of the § 1927 bargain.

II. The Proposed Waiver Fails to Include Congressionally Designed and Mandated Safeguards

Second, the proposed waiver fails to include essential safeguards that Congress incorporated into SSA § 1927 and has since affirmed. In the plain text of SSA § 1927, Congress narrowly (and exhaustively) outlines the ways in which states may limit access to covered outpatient drugs. For example, states may limit coverage of covered outpatient drugs by creating a formulary. However, Congress requires that the formulary have various safeguards for Medicaid beneficiaries, including: (i) a drug may only be excluded from a formulary on the basis of a clinical determination based on the drug's label, (ii) the state must provide a written explanation of its decision to exclude a drug in such a manner, and (iii) the state must still make such a drug available through a federally compliant prior

² OHA, Application for Renewal and Amendment, Oregon Health Plan 1115 Demonstration Waiver 30–32 (as revised Dec. 7, 2021). 12785 (Mar. 23, 2018).

authorization process.³

The proposed waiver would disregard the safeguards that Congress instituted under § 1927. OHA has proposed to introduce a “closed formulary” that is not, a formulary as that term is recognized under SSA § 1927(d)(4). The “closed formulary” would restrict coverage in a manner not permissible under federal law. For example, OHA’s proposal neither limits clinical determinations regarding a drug’s therapeutic advantage based on a review of the drug’s label, nor make available to the public a written explanation of the state’s decision to exclude a particular drug from the formulary. Instead, OHA’s proposal is to include as few as one drug per therapeutic class, irrespective of whether additional drugs qualify as “covered outpatient drugs” under § 1927 or would be medically appropriate - and superior, from a patient health standpoint - to the single available drug.

Novartis is also concerned with OHA’s proposal to exclude from coverage various therapies that have been approved by the FDA through the accelerated approval pathway. OHA would thereby substitute its judgment regarding whether products meet certain clinical efficacy criteria over the judgments of the key federal regulatory agency tasked with making such determinations – the FDA. Indeed, a decision by the FDA to approve a drug based on surrogate endpoints, or contingent on confirmatory trials, does not reflect a judgment by the agency that the drug has no “demonstrated actual clinical benefit”; it is improper for OHA to make such a judgment when the federal agency with the legal and regulatory authority and clinical expertise to make such determinations has not done so. The FDA has allowed for approval of a particular therapy based on a reported surrogate endpoint, rather than a clinical outcome, where it would be too challenging or inappropriate to measure a defined clinical outcome for that therapy. As an example, the surrogate endpoint of bone mineral density might be utilized, rather than the clinical outcome of hip fractures, because requiring the clinical outcome would require an unreasonably large and lengthy clinical trial given the relatively low incidence of hip fractures. In instances where the FDA has approved a drug based on trials utilizing surrogate endpoints, OHA should not exclude coverage of these therapies merely because only surrogate endpoints have been reported.

Also concerning is the attempt by OHA to subvert the FDA’s policy priority of accelerating patient access to innovative therapies. In this age of pharmaceutical innovation, FDA has created an accelerated approval pathway in order to expedite the process for the agency’s approval of safe and effective drugs for patients with serious and/or unmet medical needs. We submit that OHA’s proposed waiver will likely harm patients with serious and unmet medical needs by undermining this important policy objective.

Notably, CMS has affirmed that such therapies qualify as covered outpatient drugs that are subject to coverage under the MDRP. Indeed, on June 27, 2018, CMS sent a notice to the states regarding Medicaid coverage for FDA approved drugs under the accelerated approval pathway stating:

... this release clarifies that drugs that are granted “accelerated approval” are drugs approved by FDA under section 505(c) of the [Federal Food, Drug, and Cosmetic Act], and are able to satisfy the definition of covered outpatient drug, and if used for a medically-accepted indication, then the drug must be covered by state Medicaid programs if the manufacturer has an applicable signed Medicaid national drug rebate agreement for participation in the MDRP. States can use utilization management mechanisms such as prior authorization to assure appropriate use of these medications.⁴

³ See SSA § 1927(d)(4).

⁴ See CMS, Medicaid Drug Rebate Program Notice, Release No. 185, State Medicaid Coverage of Drugs Approved by the FDA under Accelerated Approval Pathway (June 27, 2018).

Thus, consistent with § 1927, states must cover drugs approved under this pathway. While states may utilize prior authorization to assure appropriate use of such therapies, the wholesale denial of access to such potentially life-saving therapies would be detrimental to the health and well-being of Medicaid beneficiaries and a violation of the law.

III. The Proposed Waiver Would Potentially Harm Medicaid Beneficiaries

The proposed waiver would deprive Medicaid beneficiaries in Oregon of medically appropriate therapies and therapeutic advances, including personalized medicines based on patients' unique medical needs and biological make-up, that would be available to non-Medicaid beneficiaries in Oregon and Medicaid beneficiaries in other states. Indeed, the proposed "closed formulary" threatens to prevent the state's most vulnerable residents from accessing such innovative therapies without regard to the appropriateness of the therapy and the possibility of better health outcomes and fewer side effects. Medicaid beneficiaries, unlike persons with greater financial means, are not afforded an opportunity to select an alternative coverage policy when a drug is not available through the Medicaid program. As such, the proposed waiver threatens to create a profoundly inequitable brand of second-class healthcare in Oregon.

We note that, should OHA wish to limit coverage of covered outpatient drugs, it can do so by establishing a "formulary" that comports with § 1927(d)(4) requirements. Under the proposed waiver, it does not appear that OHA will attempt to design appropriate coverage policies consistent with this federally permitted option. OHA should avail itself of the opportunities that already exist under federal law rather than deprive Medicaid beneficiaries of access to innovative therapies in contravention of § 1927.

We urge OHA to consider beneficiary access as a guiding principle for designing any waiver or model, especially with regard to the Medicaid population. Limitations on access to drugs could adversely affect beneficiary health and lead to increased overall costs for the Medicaid program, and thus, for taxpayers. Every waiver or other model that is approved for Medicaid should be designed in a way that protects beneficiary access to prescription drugs, incentivizes better health outcomes, and aligns payment with value. Additionally, we encourage the state to follow a collaborative approach in working with relevant stakeholders and focus on initiatives that drive value-based care while not restricting access to medicines.

IV. The Proposed Waiver Fails to Satisfy the Federal Criteria for a Section 1115 Waiver

The proposed demonstration program fails to satisfy foundational requirements of demonstration projects under SSA § 1115. Under federal law, a demonstration program must be "likely to assist in promoting the objectives of [the Medicaid program]" – i.e., it must assist in providing support to low-income individuals who, in the absence of the Medicaid program, may lack coverage for health services. The State's proposed "closed formulary" manifestly fails to satisfy this requirement. It would not assist Medicaid beneficiaries in accessing health services. Rather, it would unquestionably lead to narrower drug coverage and, for many patients, could deprive them of appropriate therapies to which patients would have had access in the absence of the proposed waiver. As CMS has previously acknowledged, drug coverage restrictions "could result in recipients being treated with alternate therapies that may not be in their best interest. This could result in increased program costs if other medical services, such as inpatient hospital services, are necessary because a drug therapy is made less accessible under the State Medicaid program."⁵

The proposed waiver also fails to set forth a true "experimental, pilot, or demonstration

⁵ 60 Fed. Reg. 48442, 48454 (Sept. 19, 1995).

project” – another federal requirement for Section 1115 waivers. At least one court has affirmed that, in reviewing a proposed demonstration project, HHS “must make some judgment that the project has a research or a demonstration value. A simple benefits cut, which might save money but has no research or experimental goal, would not satisfy this requirement.”⁶ The proposed demonstration would institute such a “simple benefits cut,” without serving a research or experimental goal. Accordingly, it lacks an essential requirement of SSA § 1115.

V. CMS Rejected the Massachusetts 1115 Waiver Demonstration that Sought to Limit Medication Access

On June 27, 2018, CMS sent a letter to Massachusetts, denying its request to create a closed formulary for the state Medicaid (MassHealth) population. CMS said that it would consider a similar demonstration project that included a closed formulary if Massachusetts (or another state) instead directly negotiated with pharmaceutical manufacturers and agreed to forgo rebates under the MDRP. According to CMS, “(t)he state could then be provided flexibility to exclude specific drugs from coverage based on cost effectiveness or other approved criteria, or to employ a closed formulary structure similar to Medicare Part D or commercial plan formularies. Under such an approach, the state would have to ensure that federal expenditures under the demonstration would not exceed federal expenditures incurred without the demonstration.”⁷ As previously discussed, the proposed waiver seeks to exclude certain covered outpatient drugs from coverage while continuing to collect manufacturers rebates under § 1927. Accordingly, the OHA proposed waiver is impermissible under the standard set forth by CMS in rejecting the Massachusetts waiver.⁸

VI. The Proposed Waiver Should Consider Alternative Payment Models

We appreciate that OHA has significant concerns regarding prescription drug spending. However, in fiscal year 2019, only 2.9 percent of the state Medicaid budget was spent on retail medications, inclusive of all rebates.⁹ As an alternative to severe access restrictions, a number of states are experimenting with new methods of Medicaid prescription drug contracting through alternative payment models (APMs) for higher cost medications and/or for medications that meet an urgent public health need. APMs aim to promote value via savings on the total cost of care, total savings for a specific population, improved access, and/or better outcomes.

States that pursue market-based alternatives such as these can improve access to therapies, may lower overall spending, and/or improve health outcomes while incentivizing manufacturers to compete on attributes that include access, cost, quality, and value. Alabama, Arizona, Colorado, Louisiana, Massachusetts, Michigan, North Carolina, Oklahoma, and Texas have all been approved by CMS for Medicaid supplemental rebate agreements (SRAs) that allow for value-based agreements (VBAs) with pharmaceutical manufacturers for a variety of treatments. Louisiana and Washington have received CMS approval of SRA arrangements that allow for Hepatitis C “subscription agreements,” and at least 14 states use risk distribution models including high-risk pools, reinsurance, and risk corridors to help manage program costs. These states have developed approaches that address spending concerns while also maintaining, and

⁶ *Beno v. Shalala*, 30 F.3d 1057, 1069 (9th Cir. 1994).

⁷ See CMS, 11-W-00030/1 <https://www.medicaid.gov/Medicaid-CHIP-Program-Information/By-Topics/Waivers/1115/downloads/ma/ma-masshealth-ca.pdf>.

⁸ Novartis recognizes that in early 2021, CMS approved a demonstration proposal from Tennessee to create a closed formulary under its Medicaid program. Since then, CMS has decided to reopen implementation of the demonstration proposal and has requested a new round of public comments. Novartis firmly believes that CMS does not have the legal authority to approve Tennessee’s proposal to implement a closed formulary. Oregon should therefore not base its decision to implement the proposed waiver on CMS’s initial approval of the Tennessee waiver, especially since CMS has reopened the approval determination by seeking a new comment period.

⁹ PhRMA, “The Facts About Medicaid in Oregon”, https://www.phrma.org/-/media/Project/PhRMA/PhRMA-Org/PhRMA-Org/PDF/Medicaid-2020/OR-One-Pager_20.pdf.

even improving, Medicaid beneficiary access to innovative medicines.

Novartis has, for instance, entered into VBAs with a number of these states for the spinal muscular atrophy (SMA) gene therapy, Zolgensma, whereby the state receives additional rebates if the desired clinical outcomes are not achieved in the first five years post-administration. Furthermore, a CMS final rule becomes effective in July of this year that will make developers' commercial VBAs more accessible to state Medicaid programs and generally reduce current burdens for states pursuing VBAs.¹⁰ OHA should pursue these types of program reforms and explore new CMS VBA opportunities rather than changes that threaten Medicaid beneficiary access to potentially life-saving therapies.

VII. The Proposed Waiver Restricts Access to Life-Saving Medications, Leading to Poorer Outcomes, and Runs Counter to the OHA's Mission of Health Equity

Novartis believes an important way we can achieve greater health equity is to address persistent disparities in the way healthcare is approached, accessed, and delivered. We are confronting these disparities in innovative ways, such as promoting greater diversity in biopharmaceutical clinical trials and supporting educational programs that raise awareness about diseases and treatments. Often, we work in partnership with patient and community organizations that share our commitment to health equity.

Novartis appreciates the state's focus on health equity. We commend its efforts to improve coverage for vulnerable populations, addressing social determinants of health, assessing health equity quality metrics, and focusing on improving health outcomes. The proposed waiver, however, may have the opposite effect as intended, as it is well documented that restricted access to medicines reduces adherence to prescribed medication regimens, worsens health outcomes, drives up long-run costs, and exacerbates healthcare disparities. A systematic literature review concluded that "formulary restrictions were most frequently negatively correlated" with desirable outcomes, including a consistent negative effect on medication adherence.¹¹

A closed formulary ignores and detracts from real opportunities to improve health equity. Underserved communities, who are disproportionately burdened by chronic disease, often already face barriers to accessing medicines and are often treated later for many diseases, such as certain cancers.^{12, 13, 14} Timely access to provider-recommended medicines is central to reversing that trend, improving health outcomes, decreasing avoidable healthcare utilization and costs, and reducing mortality.^{15,16,17} Improving health equity must begin with addressing the obstacles to accessing care that are most significant to underserved communities.

A closed formulary involves "one size fits all" determinations about clinical efficacy, fails to accommodate the diverse needs of a heterogeneous patient population like in Oregon. In fact, a

¹⁰ 85 Fed Reg. 87000 (Dec. 31, 2020).

¹¹ 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 Manag Care Spec Pharm*. 2014;20(7):677-84.

¹² Minority Population Profiles. Office of Minority Health. <https://minorityhealth.hhs.gov/omh/browse.aspx?lvl=2&lvlid=26>.

¹³ Gracia JN. COVID-19's Disproportionate Impact on Communities of Color Spotlights the Nation's Systemic Inequities. *Journal of Public Health Management and Practice* 2020; 26(6):518-521. doi: 10.1097/PHH.0000000000001212.

¹⁴ Halpern MT, Holden DJ. Disparities in timeliness of care for US Medicare patients diagnosed with cancer. *Curr Oncol*. 2012;19(6):e404-e413. doi:10.3747/co.19.1073

¹⁵ Lloyd JT, Maresh S, Powers CA, Shrank WH, Alley DE. How Much Does Medication Nonadherence Cost the Medicare Fee-for-Service Program? *Med Care*. 2019 Mar;57(3):218-224. doi: 10.1097/MLR.0000000000001067. PMID: 30676355.

¹⁶ Sokol MC, McGuigan KA, Verbrugge RR, Epstein RS. Impact of medication adherence on hospitalization risk and healthcare cost. *Med Care*. 2005 Jun;43(6):521-30. doi: 10.1097/01.mlr.0000163641.86870.af. PMID: 15908846.

¹⁷ Khunti K, Seidu S, Kunutsor S, Davies M. Association Between Adherence to Pharmacotherapy and Outcomes in Type 2 Diabetes: A Meta-analysis. *Diabetes Care*. 2017 Nov;40(11):1588-1596. doi: 10.2337/dc16-1925. Epub 2017 Aug 11. PMID: 28801474.

recent study found if the Medicaid program implemented rigid cost-effectiveness criteria for a set of chronic conditions treated with long-term drug regimens, a significant percentage of beneficiaries would be required to change their current prescriptions.¹⁸ As a result of the OHA's proposal, patients with chronic conditions may lose access to their current treatments or experience interruptions in care. The state should take this opportunity to revise its harmful formulary policy proposal that, if implemented, would exacerbate existing healthcare inequalities for vulnerable patients in Oregon.

Conclusion

In sum, the proposed waiver poses a significant risk to the health and well-being of Medicaid beneficiaries in Oregon. It would also mark a serious departure from the principles undergirding SSA § 1927, and from CMS' long-standing policy of "remain[ing] committed to Medicaid beneficiaries continuing to have access to needed prescribed medications."¹⁹ It also fails to satisfy the essential criteria for a § 1115 demonstration program. As such, Novartis strongly objects to the prescription drug restrictions in the proposed waiver and encourages OHA to revise its OHP proposal to ensure appropriate access to prescription drugs and the inclusion of beneficiary protections.

Moving forward, Novartis would like to work with OHA to develop meaningful solutions to meet its budgetary and health equity goals while upholding the current Medicaid rebate statute. We stand ready to work with the state if there is interest in applying for a state plan amendment to implement VBAs and health equity strategies to ensure that OHP beneficiaries have access to life-saving and life-enhancing medications.

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We appreciate the opportunity to comment and would be happy to provide further information regarding our comments above. Please feel free to contact me at 862-778-3284 if we can provide further assistance.

Sincerely,

Leigh Anne Leas
Vice President and Head, North America Public Policy
Novartis Services, Inc.

¹⁸ Xcenda, Impact Analysis of ICER Formulary Implementation in Medicaid, https://www.xcenda.com/-/media/assets/xcenda/english/content-assets/white-papers-issue-briefs-studies-pdf/icer-medicaid-analysis_march-2019.pdf?la=en&hash=03590A12822FB95144692F0BF6FFF846E2E26F1A. The conditions studied were: multiple sclerosis (99% of prescriptions would need to be changed), rheumatoid arthritis (87%), non-small cell lung cancer (78%), psoriasis (77%), and multiple myeloma (42%).

¹⁹ See CMS, Medicaid Drug Rebate Program Notice, Release No. 172, Assuring Medicaid Beneficiaries Access to Hepatitis C (HCV) Drugs (Nov. 5, 2015).