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VIA ELECTRONIC DELIVERY

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: Comments of Pfizer Inc. Regarding Draft Application for Renewal and
Amendment Oregon Health Plan, Section 1115 Demonstration Waiver**

Dear Director Allen,

Pfizer, Inc. (“Pfizer”) appreciates the opportunity to comment on the Oregon Health Authority’s (OHA) proposed renewal and amendment of its Section 1115 waiver (“proposed 1115 waiver”). Pfizer is committed to saving and improving lives through the development of medicines and vaccines, applying the latest science and technology to meet the most demanding healthcare challenges of today. Pfizer believes that the proposed 1115 waiver would jeopardize the ability of Medicaid beneficiaries—among the neediest of patients—to access life-saving therapies, among other troubling outcomes.

Pfizer is particularly concerned by the OHA’s proposal to implement a closed formulary and exclude drugs approved via FDA’s accelerated approval pathway—which the proposal calls “drugs with limited or inadequate evidence of clinical efficacy.”¹ Pfizer agrees with other commenters, including the Pharmaceutical Research and Manufacturers of America (“PhRMA”) and the Biotechnology Innovation Organization (“BIO”), that these proposals fail to meet certain basic requirements for Medicaid waivers under Social Security Act (“SSA”) section 1115,² and thwart the Congressional intent underlying the Medicaid rebate statute,³ which reflects a pact

¹ Oregon Health Authority, Oregon Health Plan, Section 1115 Demonstration Amendment Draft Application for Public Comment (Dec. 1, 2021), <https://www.oregon.gov/oha/hsd/medicaid-policy/pages/waiver-renewal.aspx> (“Waiver Request”).

² Codified at 42 U.S.C. § 1315.

³ Codified at 42 U.S.C. § 1396r-8.

between government and industry to provide deep rebates in exchange for patient access. Further, the proposed formulary exclusions (even if allowable) would block access to therapeutic advances for serious and life-threatening conditions approved as safe and effective by the FDA for the most vulnerable and neediest group of patients.

I. The Commonwealth’s Proposal Fails to Satisfy the Requirements for Section 1115 Waivers.

Pfizer shares the objections raised by PhRMA, BIO, and other organizations that OHA’s proposal falls outside the scope of waivers allowed by section 1115 and upends key requirements of the Medicaid rebate statute. First, the proposal flies in the face of PhRMA v. Thompson, in which the D.C. Circuit held that SSA section 1115 does not authorize waivers of the Medicaid rebate statute.⁴ Second, such a waiver would destroy an essential element of the legislative compromise codified by Congress in the Medicaid rebate statute. Under the rebate statute, manufacturers of innovator drugs pay deep rebates to Medicaid programs, in exchange for Medicaid beneficiaries receiving the drug coverage protections. For innovator drugs, the statute provides Medicaid programs with a price net of the rebate that is at least as low as the manufacturer’s best price to any commercial customer. In fact, the price to Medicaid is often lower than any commercial customer’s price, because the Medicaid rebate statute guarantees Medicaid at least a 23.1% discount on most innovator drugs and an additional rebate that pays dollar-for-dollar the amount by which the average manufacturer price of the drug increases faster than the inflation rate.⁵ Many cases have held that when a statute reflects such a legislative compromise, the interpretation of the law should uphold the compromise—not tear it apart.⁶ As other commenters have argued, OHA’s proposal would fall short of section 1115 waiver requirements in other significant ways. For example, the proposal is not, in fact, a demonstration, test, pilot, or study—it does not involve any investigation of the impacts on healthcare outcomes, access, delivery, or secondary healthcare costs—but instead represents a mere benefits cut, which is not a permissible subject for a section 1115 waiver.⁷

Other commenters have also addressed the concerns for patient access under the proposal as well as the need for OHA to use the many tools already at its disposal (rather than this problematic proposed 1115 waiver). These themes are explored in more depth in the remainder

⁴ 251 F.3d 219, 222 (D.C. Cir. 2001) (“Although the Act authorizes the Secretary to waive certain Medicaid requirements for such demonstration projects, it does not authorize him to waive any requirements of section 1396r–8’s [the Medicaid rebate statute’s] rebate provision or the requirement that Medicaid beneficiaries contribute no more than a ‘nominal’ amount to the cost of medical benefits they receive.”).

⁵ Specifically, the Medicaid rebate for innovator drugs equals the higher of the drug’s average manufacturer price (AMP) minus the drug’s best price, or the mandated minimum percentage discount based on product type (23.1 percent for most innovator drugs, 17.1 percent for clotting factors and drugs approved only for pediatric indications, and 13 percent for non-innovator/generic drugs). An additional dollar-for-dollar rebate, referred to as the CPI Penalty, is also required for price increases greater than the inflation rate (and applies to both innovator and non-innovator drugs).

⁶ See, e.g., *General Motors Corp. v. Romein*, 503 U.S. 181, 191 (1992) (upholding statutory provisions necessary to “preserve[] the delicate legislative compromise that had been struck by [prior] laws”).

⁷ *Beno v. Shalala*, 30 F.3d 1057, 1069 (9th Cir. 1994) (“[Section 1115] requires that the state project be an ‘experimental, demonstration or pilot’ project. The statute 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 public welfare recipients.’”).

of this comment, in keeping with Pfizer’s support for policies that appropriately promote patient access to prescribed products. We also explain below how granting OHA’s request would be inconsistent with FDA’s authority to grant accelerated approvals.

II. OHA’s Proposal Would Jeopardize Access for the Neediest Patients and Block Coverage of Cutting-Edge Treatments for Life-Threatening Conditions.

A. The Proposal Would Exclude Important Medicines That Have Met FDA Standards of Safety and Efficacy

OHA proposes to “[a]dopt a commercial-style closed formulary with at least one drug available per therapeutic class” and to “[e]xclude from the formulary drugs with limited or inadequate evidence of clinical efficacy,”⁸ under sections 3a and 3b of its waiver request, respectively.

Regarding closed formularies, studies suggest that allowing more choice of medications has positive results for patients, including lowering the chances of drug interactions and adverse events, and increasing the efficacy of treatment.⁹ Years of research have also shown that limiting formularies correlates to poor medication adherence outcomes.¹⁰ Studies featuring Medicaid recipients with severe health conditions indicate that in many instances, these restrictions can result in negative health outcomes and other outcomes without generating program savings or other intended benefits (and sometimes increasing overall state costs).¹¹

Regarding drugs with “limited or inadequate clinical efficacy,” OHA defines this term “as when one or more of the following conditions exist”:

- “Primary endpoints in clinical trials have not been achieved;
- Only surrogate endpoints have been reported;

⁸ Waiver Request at 30-31.

⁹ See, e.g., DiMasi, “Competitiveness in follow-on drug R&D: a race or imitation?” 10 Nat. Rev. Discov. 23-27 (Jan. 2011); Turner et. al, “Parsing Interindividual Drug Variability: An Emerging Role For Systems Pharmacology,” 7 Wiley Interdiscip. Rev. Syst. Biol. Med. 221-41 (2015); Mullins et. al, “Persistence, Switching, And Discontinuation Rates Among Patients Receiving Sertraline, Paroxetine, And Citalopram,” 25 Pharmacotherapy 660-67 (2005).

¹⁰ See, e.g., Happe et. al, “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,” 20 Manag. Care Spec. Pharm. 677-84 (2014).

¹¹ See, e.g., USC Schaffer, “Medicaid Access Restrictions on Psychiatric Drugs: Penny-Wise or Pound Foolish?” (Feb. 2015), <http://healthpolicy.usc.edu/documents/USC%20Issue%20Brief%20No.%202%20Final.pdf> (indicating increased incarceration rates associated with certain access restrictions); Lu, et. al, “Unintended Impacts of a Medicaid Prior Authorization Policy on Access to Medications for Bipolar Illness,” 48 Medical Care 4 (Jan. 2010) (finding that while a prior authorization policy in Maine Medicaid was associated with a marked decrease in rates of initiation of bipolar treatments associated with reduction in initiation of nonpreferred agents, the policy had no discernable impact on rates of switching therapy among patients currently on treatment); Farley, et al., “Retrospective Assessment of Medicaid Step-Therapy Prior Authorization Policy for Atypical Antipsychotic Medications,” 30 Clinical Therapeutics 1524 (April 2008) (showing, for a group of Medicaid patients with schizophrenia who were subject to a prior authorization policy for atypical antipsychotic medications, significant increases in per member per month outpatient expenditures far exceeded the associated savings in a typical antipsychotic expenditures).

- Clinical benefits have not been assessed;
- The drug provides no incremental clinical benefit within its therapeutic class, compared to existing alternatives.”¹²

This language (contained in section 3b of the waiver request) reflects OHA’s misguided attempt to target for exclusion drugs that enter the market through FDA’s accelerated approval pathway.¹³ This statutory pathway authorizes FDA to approve a product that treats “a serious or life threatening condition . . . upon a determination that the product has an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit . . . taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative therapies.”¹⁴

Under the law, FDA must find “substantial evidence” of the drug’s effectiveness, and the applicant must have conducted one or more “adequate and well-controlled studies,”¹⁵ just like drugs approved outside this pathway. Indeed, medicines approved through the accelerated pathway are those that FDA has determined meet the key requirements of safety and efficacy, and that FDA believes should be approved on an expedited basis because they are needed to treat “serious and life-threatening” diseases and conditions.

Existing literature supports this characterization. Researchers at Tufts University, for example, found that drugs cleared through FDA’s expedited review process “offered larger health gains, compared to drugs approved through conventional review processes.”¹⁶ Notwithstanding these health benefits, OHA wishes to target these drugs for formulary exclusion.

While accelerated approval drugs represent the cutting edge of treatment—and meet the exacting FDA approval standards required under law—the proposal would permit OHA to exclude these drugs from coverage on the grounds, for example, that their clinical benefit was established through studies that rely on surrogate endpoints.¹⁷ This exclusion criterion reflects OHA’s misunderstanding of the nature and purpose of validated surrogate endpoints. Under the Food, Drug, and Cosmetic Act (FDCA), FDA can base an accelerated approval “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

¹² *Id.* at 10.

¹³ *Id.* (“Many drugs coming to market through the FDA’s accelerated approval pathway have not yet demonstrated clinical benefit and have been studied in clinical trials using only surrogate endpoints.”).

¹⁴ 21 U.S.C. § 356.

¹⁵ 21 U.S.C. § 355.

¹⁶ James D. Chambers et al., “Drugs Cleared Through the FDA’s Expedited Review Offer Greater Gains Than Drugs Approved By Conventional Process,” 36 Health Affairs 1408-1415 (Aug. 2017), <http://content.healthaffairs.org/content/36/8/1408.abstract>.

¹⁷ The Commonwealth also includes another criterion, “Primary endpoints in clinical trials have not been achieved.” This criterion is somewhat confusing, since primary endpoints are endpoints that FDA deems essential to establish effectiveness, thus it is not clear that any approved drugs exist for which “primary endpoints in clinical trials have not been achieved.” *See, e.g.*, FDA, Draft Guidance for Industry, Multiple Endpoints in Clinical Trials, at 2 (January 2017).

condition and the availability or lack of alternative treatments.”¹⁸ FDA explains in its guidance that the accelerated approval process 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 [i.e. FDA criteria for refusing applications, including the “substantial evidence” standard].”¹⁹ FDA is the expert agency for determining products’ safety and efficacy, including those receiving accelerated approvals. CMS should not undermine its sister agency by allowing OHA to second-guess FDA by broadly rejecting coverage for products used for their FDA-approved indications.

If the waiver were granted, it would allow OHA to exclude many drugs that received accelerated approval and that thousands of patients, including Oregon Health Plan beneficiaries, depend on for the treatment of severe and life-threatening conditions.

In addition to noting the serious risks for patients who rely on these innovative medications, it is worth pointing out additional problems with OHA’s exclusion criteria. For example, the proposed criterion that “[c]linical benefits have not been assessed” actually does not apply to any FDA approved drug (under either the accelerated or traditional pathways); the criterion is a misguided attempt to target drugs that have been approved based on clinical studies using surrogate endpoints to predict a clinically meaningful benefit. Also, the criterion that that “drug provides no incremental clinical benefit within its therapeutic class, compared to existing alternatives” is overly broad and could in fact be applied by OHA to exclude from coverage almost any drug. U.S. law does not require a demonstration of “incremental benefit” for product approval. Instead, drugs are approved if they are “safe and effective,” whether the drug receives approval under the traditional or the accelerated pathway.

B. An Exceptions Process is No Panacea

The proposal would allow patients to obtain excluded drugs through an exceptions process. However, exceptions procedures for non-formulary drugs, like utilization management restrictions for on-formulary drugs, can lead to a decrease in patient adherence and an increase in overall system cost and inefficiencies. Such burdensome administrative processes can deter or prevent patients from taking medication altogether. Moreover, several studies conducted with Medicaid beneficiaries suggest other ways in which utilization management controls like prior authorization can backfire—for example, by increasing certain costs of outpatient care that outweigh the per-member savings gained by limiting drug utilization.²⁰ Even where utilization

¹⁸ 21 U.S.C. 356(c)(1)(A).

¹⁹ FDA, “New Drug, Antibiotic, and Biological Drug Product Regulations; Accelerated Approval,” 57 Fed. Reg. 58942, 58944 (Dec. 11, 1992).

²⁰ Farley, et al., “Retrospective Assessment of Medicaid Step-Therapy Prior Authorization Policy for Atypical Antipsychotic Medications,” 30 Clinical Therapeutics 1524 (April 2008) (“Among patients with schizophrenia, the PA policy was associated with a \$19.62 per member per month (PMPM) decrease in atypical antipsychotic expenditures and a \$2.20 PMPM increase in typical antipsychotic expenditures (both, $P < 0.001$). Among the same patients with schizophrenia, however, the reduction in atypical antipsychotic expenditures was accompanied by a \$31.59 PMPM increase in expenditures for outpatient services ($P < 0.001$).”).

management is successful in switching patients to lower-cost sales channels or lower-tier medications, such changes do not always result in greater cost savings overall.²¹

Clinical judgement exercised within the bounds of accepted medical practice ought to take precedent over a payer's cost considerations in decisions about an individual's care. This is especially true because there is no conclusive evidence that administrative barriers such as prior authorization or step therapy reduce long-term health care costs—and such restrictions may in fact result in higher costs due to possible adverse reactions or other treatment that may be required as the result of nonadherence. For these reasons, applying a blanket exclusion restriction to FDA approved therapies used for their approved indications, not to mention cutting-edge therapies approved through accelerated approval, and forcing patients and providers to rely on an exceptions process to access medically necessary drugs would be a step in the wrong direction, creating new barriers for patients rather than supporting prescriber decision making and pioneering drug development. Pfizer believes that existing utilization management tools provide OHA sufficient control over pharmacy spending. Given the limitations noted above, we believe these tools ought to be applied efficiently and judiciously, in a manner consistent with sound policy and the framework of existing law.

III. Granting OHA's request would be inconsistent with FDA's authority to grant accelerated approvals.

Congress explained how it envisioned FDA's role in the extensive "findings" and "sense of Congress" provisions included in Section 901 of the Food and Drug Administration Safety and Innovation Act (FDASIA), a bipartisan reform in 2012 that amended the federal Food Drug and Cosmetic Act (FDCA) to codify the accelerated approval pathway. Congress addressed both how it saw FDA's role as a steward of medical innovation as well as the importance of the expedited approval framework to realizing FDA's duty and purpose:

[FDA] serves a critical role in helping to assure that new medicines are safe and effective. Regulatory innovation is 1 element of the Nation's strategy to address serious or life-threatening diseases or conditions by promoting investment in and development of innovative treatments for unmet medical needs. [. . .] FDA should be encouraged to implement more broadly effective processes for the expedited development and review of innovative new medicines intended to address unmet medical needs for serious or life-threatening diseases or conditions, including those for rare diseases or conditions, using a broad range of surrogate or clinical endpoints and modern scientific tools earlier in the drug development cycle when appropriate.²²

FDA has recognized this statutory mandate through public statements, such as in a 2015 white paper in which FDA declared: "finding effective treatments for rare diseases is a public health priority, and FDA has brought to bear all of its drug review and technical assistance tools

²¹ See, e.g., Martin, et. al, "Impact of a Novel Cost-Saving Pharmacy Program on Pregabalin use and Health Care Costs," 22 J. Managed Care & Specialty Pharmacy 132 (Feb. 2016).

²² Pub. L. 112–114, title IX, §901(a), July 9, 2012, as amended by Pub. L. 114-255, div. A, title III, §3101(b)(1), Dec. 13, 2016, 130 Stat. 1156.

to assist the development of new treatments for these conditions.”²³ FDA also acknowledged that Congress built on this framework through the passage of the 21st Century Cures Act:

With [21st Century Cures,] great progress has been made towards our shared goal of advancing regulatory science so that we can continue to speed the discovery, development, and delivery of medical products to prevent and cure disease and improve health while sustaining the evidence framework that enables assurance to the public of the safety and effectiveness of medical products.²⁴

As these sources indicate, FDA’s role as contemplated by Congress is to apply FDA’s technical expertise to speed the development and approval of safe and effective medicines—including drugs that treat serious conditions and fill an unmet need. FDA exercises tools for regulatory flexibility in service of this mission. Congress has recognized that it is because of FDA’s expertise that FDA is able to use these tools to promote (and not hamper) the development and approval of safe and effective medicines.

OHA’s proposed section 1115 waiver request would undercut FDA’s role, by substituting the judgement of OHA for that of FDA.²⁵ We think that Congress never would have intended such an outcome, as it would conflict with the statutory framework Congress devised under FDCA and its amendments.

As courts have described, section 1115 allows CMS to exercise its own wisdom to determine whether a state experiment in social services programs and benefits should be allowed: Congress “intended that the Secretary would ‘selectively approve[]’ state projects,” and gave the HHS Secretary “plenary authority to reject State projects and to require States to modify projects to make them more consistent with federal requirements, less likely to harm recipients, and more likely to further the goals of the Social Security Act.”²⁶

Moreover, OHA’s proposed 1115 waiver would violate a core principle of statutory interpretation—that where potential conflicts exist between two federal statutes, one should adopt “the interpretation that preserves the principal purposes of each.”²⁷ If the two laws cannot be reconciled, courts generally give effect to the “later-enacted, more specific statute.”²⁸ Here, OHA’s proposed 1115 waiver would usurp FDA’s authority and contradict the aims of FDCA in creating a cutting-edge legal framework for the exercise of FDA technical expertise.

²³ FDA White Paper, FDA and Accelerating the Development of New Pharmaceutical Therapies, 7-8 (March 23, 2015).

²⁴ 21st Century Cures Act; Making Progress on Shared Goals for patients, FDA Commissioner Dr. Robert M. Califf, FDA Voice, December 13, 2016.

²⁵ Waiver request at 9 (“Massachusetts seeks the ability to use its own rigorous review process, in partnership with the University of Massachusetts Medical School, to determine coverage of new drugs and to guarantee that patients access clinically proven, efficacious drugs.”).

²⁶ Beno v. Shalala, 30 F.3d 1057, 1069 (9th Cir. 1994)(internal citations omitted).

²⁷ See, e.g., SmithKline Beecham Consumer Healthcare, LP v. Watson Pharmaceuticals, Inc., 211 F.3d 21, 27-28 (2d. Cir. 2000).

²⁸ Hawaii v. Trump, 859 F.3d 741, 778 (9th Cir. 2017).

Again, Pfizer appreciates the opportunity to comment on OHA's proposed 1115 waiver renewal and amendment application. If you have questions or need additional information, please contact Tom Brownlie at thomas.brownlie@pfizer.com.

Joyce Rogers

A handwritten signature in cursive script that reads "Joyce Rogers".

Vice President
U.S. Policy and Public Affairs