909 E Street NW, 2nd Floor
Washington D.C. 20004
gene-therapies.org

April 13, 2022

VIA ELECTRONIC DELIVERY

The Honorable Chiquita Brooks-LaSure
Administrator
Centers for Medicare and Medicaid Services
Department of Health and Human Services
7500 Security Boulevard
Baltimore, MD 21244-1850

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

Dear Administrator Brooks-LaSure:

The Institute for Gene Therapies (IGT or "the Institute") is pleased to submit comments to the Centers for Medicare and Medicaid Services (CMS or "the Agency") regarding Oregon Health Authority's (OHA's) Section 1115 waiver extension application.[1] The Institute is concerned about the waiver's proposal to limit access to drugs approved through the FDA's accelerated approval pathway and we urge CMS to reject the proposal.

IGT was launched in February 2020 to advocate for a modernized regulatory and reimbursement framework that encourages the development of transformative gene therapies and promotes patient access. Through a Corporate Advisory Council, Patient Advocacy Advisory Council, and Scientific, Academic & Medical Council, the Institute represents a wide array of gene therapy manufacturers, patient advocacy groups, and scientific, medical, and academic stakeholders seeking to advance the promise of gene therapies. IGT aims to inform the conversation regarding the value of transformative therapies and advocate for policies and practices to ensure patient access to these treatments. A full list of our members is available at https://www.gene-therapies.org/advisory-councils.

I. The Accelerated Approval Pathway and Oregon Health Authority's Section 1115 Waiver

Oregon Health Authority (OHA) submitted a Section 1115 waiver extension application to CMS on February 18, 2022. CMS has opted to treat OHA's application as a new demonstration application based on substantial changes to Oregon's existing 1115 waiver. One such change proposed by OHA would restrict Medicaid beneficiary access to drugs approved under the accelerated approval pathway that OHA deems have "limited or inadequate evidence of clinical efficacy." This proposal represents a fundamental misunderstanding of the

[1] Oregon Health Authority (OHA) 2022-2027 Oregon Health Plan (OHP) 1115 Waiver Application (February 2022), available at: https://www.oregon.gov/oha/HSD/Medicaid-Policy/Documents/2022-2027-Waiver-Application-Final.pdf (last accessed April 12, 2022).

accelerated approval pathway, ignores FDA's precedential authority over drug approval, and overlooks the limited impact of accelerated approval drugs on state Medicaid budgets,[2] including Oregon's.[3] These substantial flaws lead IGT to one plausible conclusion: CMS must reject this proposal just like CMS rejected the 2017 Massachusetts proposal that would have excluded coverage of accelerated approval drugs. In rejecting OHA's request, CMS will preserve the statutory requirement that state Medicaid programs cover all drugs approved by FDA.[4]

Created in 1992, the accelerated approval pathway allows earlier approval of drugs that treat an unmet medical need for serious or life-threatening conditions. Approval is based on a surrogate endpoint, which is a marker (e.g., laboratory measurement, radiographic image, physical sign, or other measure) that is reasonably likely to predict clinical benefit.[5] Using a surrogate endpoint can save valuable years in the drug approval process for patients facing serious or life-threatening illnesses and there is often extensive dialogue between sponsors and FDA prior to an accelerated approval decision. Restricting access to accelerated approval drugs that address this unmet need will exacerbate health inequities, contradicting the objectives outlined in the Executive Order titled, "Advancing Racial Equity and Support for Underserved Communities Through the Federal Government."[6]

Recognizing the value of accelerated approval, in 2012, Congress passed the FDA Safety and Innovation Act, which authorized the FDA to use the pathway to approve drugs treating rare diseases.[7] All drugs approved via the accelerated approval pathway meet the FDA's gold standard for safety and efficacy and are not considered experimental, investigational, or having low evidence – that is, they must meet the same statutory standards for safety and effectiveness as drugs granted traditional approval.[8] In addition, drugs approved via the accelerated approval pathway are considered covered outpatient drugs pursuant to Section 1927 of the Social Security Act (the Act).[9] As CMS noted in your 2018 Notice, "…a drug approved by the [FDA] under its "accelerated approval" pathway…must be covered by state Medicaid programs, if the drug meets the definition of "covered outpatient drug" as found in Section 1927..."[10]

Accelerated approval is used to authorize and provide life-saving treatments to patients who have limited or no treatment options and suffer from serious and/or life-threatening diseases years in advance of the timeframe required under the traditional FDA approval process. IGT recognizes the challenges of executing confirmatory trials. However, there are often valid clinical or other reasons why confirmatory trials may be delayed (e.g.,

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[2] See Thorpe, PhD, Kenneth E. and Holtz-Eakin, PhD, Douglas, "Limiting Medicaid Access to Accelerated Approval Drugs: Costs and Consequences," Am J Manag Care. 2021;27(6):e178-e180. https://doi.org/10.37765/ajmc.2021.88596 (last accessed April 12, 2022).

[3] Thorpe, PhD, Kenneth E., "Debunking Oregon's Cost Argument in Denying Access to Accelerated Approval Drugs," available at: https://www.fightchronicdisease.org/blog/debunking-oregon%E2%80%99s-cost-argument-denying-access-accelerated-approval-drugs (March 15, 2022).

[4] 42 U.S.C. § 1396r–8.

[5] 21 C.F.R. § 601.41.

[6] Executive Order on Advancing Racial Equity and Support for Underserved Communities Through the Federal Government, available at: https://www.whitehouse.gov/briefing-room/presidential-actions/2021/01/20/executive-order-advancing-racial-equity-and-support-for-underserved-communities-through-the-federal-government/ (January 20, 2021).

[7] Food and Drug Administration Safety and Innovation Act of 2012, Pub. L. No. 112-144, 126 Stat. 993-1132 (2012).

[8] Guidance for Industry: Expedited Programs for Serious Conditions – Drugs and Biologicals (May 2014), available at: https://www.fda.gov/media/86377/download (last accessed April 12, 2022).

[9] 42 U.S.C. 1396r–8.

[10] Medicaid Drug Rebate Program Notice, Release No. 185 (June 27, 2018), available at: https://www.medicaid.gov/medicaid-chip-program-information/by-topics/prescription-drugs/downloads/rx-releases/state-releases/state-rel-185.pdf.

identification or enrollment of eligible patients, manufacturing/supply chain constraints, competing for patients with other trials, protocol delays). Additionally, confirmatory trials that fail to verify clinical benefit may be the result of other factors not related to a drug's clinical effect (e.g., selection of primary end point, trial design, statistical issues). Oregon's intent to implement a different "rigorous review process to determine coverage of drugs" through the 1115 waiver oversteps the FDA's authority, as FDA is the sole authority for determining a drug's safety and efficacy.

Moreover, studies have found that accelerated approval drugs account for less than one percent of spending in state Medicaid budgets per year.[11] In Oregon, accelerated approval drugs accounted for 0.4% of Medicaid spending in 2020 and represented only 0.5% of total Medicaid spending growth from 2015-2020.[12] Restricting access to accelerated approval drugs would not result in meaningful savings. Rather, restricted access would exacerbate health inequities and limit patient access to needed treatments. If CMS approves the OHA waiver, CMS will set a harmful precedent for non-coverage of drugs approved through the accelerated approval pathway. While not all gene therapies will be approved via accelerated approval, the pathway is an important tool in ensuring patients can receive these therapies to mitigate disease progression of genetic disorders.

II. Conclusion

The Institute believes that if approved, OHA's proposal will impede patient access to critical gene therapies. IGT urges CMS to reject the OHA 1115 waiver application and preserve Medicaid beneficiary access to all FDA approved drugs. Only the FDA is properly staffed with scientific experts skilled in determining a drug's safety and efficacy. Restricting access to accelerated approval drugs would exacerbate existing health inequities, working against the Biden Administration's priorities, and directly undermine the Executive Order on addressing health equity.

IGT would be pleased to serve as a resource on gene therapy issues during this process and answer any questions regarding these comments.

Sincerely,

John R. Feore, III
Director, Policy and Advocacy
Institute for Gene Therapies

[11] Thorpe, PhD, Kenneth E. and Holtz-Eakin, PhD, Douglas, "Limiting Medicaid Access to Accelerated Approval Drugs: Costs and Consequences," Am J Manag Care. 2021;27(6):e178-e180. https://doi.org/10.37765/ajmc.2021.88596 (last accessed April 12, 2022).

[12] Thorpe, PhD, Kenneth E., "Debunking Oregon's Cost Argument in Denying Access to Accelerated Approval Drugs," available at: https://www.fightchronicdisease.org/blog/debunking-oregon%E2%80%99s-cost-argument-denying-access-accelerated-approval-drugs (March 15, 2022).