Rare Disease Company Coalition

BY ELECTRONIC DELIVERY

April 13, 2022

The Honorable Xavier Becerra
Secretary
Department of Health and Human Services
Hubert H. Humphrey Building
200 Independence Avenue, SW
Washington, DC 20201

Ms. Chiquita Brooks-LaSure
Administrator
Centers for Medicare and Medicaid Services
7500 Security Boulevard
Baltimore, MD 21244

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

Dear Secretary Becerra and Administrator Brooks-LaSure:

The Rare Disease Company Coalition (RDCC) appreciates the opportunity to provide comments on Oregon Health Authority's (OHA) Section 1115 Demonstration Waiver Application to the Centers for Medicare and Medicaid Services (CMS). The RDCC represents life science companies committed to discovering, developing, and delivering rare disease treatments for the patients we serve. As discussed in more detail below, we have serious concerns with the potential impact that waiving certain provisions of §1927 of the Social Security Act (SSA) would have on the discovery and development of new medicines, particularly on treatments and cures for patients with rare diseases who currently have limited or no treatment options.

Rare disease in the United States is defined as a condition that affects fewer than 200,000 people. There are over 7,000 identified rare diseases that impact an estimated 25-30 million Americans.[1] These diseases are often devastating and life-threatening: 80 percent of rare diseases are genetic in origin and 50 percent impact children,[2] with many rare diseases resulting in premature deaths of infants and young children.[3] Coalition members are passionately working every day to meet the needs of rare disease patients with currently limited or no treatment options. Collectively, members invested over $12.4 billion in R&D in 2021; have brought 31 treatments to market to date, the majority of which are first-to-market therapies; and are presently working on more than 200 rare disease development programs, many of which would be first-to-market therapies if approved.[4] The RDCC's goal is to inform policymakers of the unique challenges and promises of rare disease drug discovery, development and manufacturing for small population sizes so that critical innovation for the rare disease community can flourish.

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[1] National Center for Advancing Translational Science and Genetic and Rare Diseases Information Center. *What is a rare disease?* Updated January 26, 2021. Accessed April 2022 at https://rarediseases.info.nih.gov/diseases/pages/31/faqs-about-rare-diseases.
[2] Batshaw ML, Groft SC, Krischer JP. Research into rare diseases of childhood. *JAMA*. 2014; 311(17): 1729-30.
[3] Institute of Medicine (US) Committee on Accelerating Rare Diseases Research and Orphan Product Development; Field MJ, Boat TF, editors. *Rare diseases and orphan products: Accelerating research and development*. Washington (DC): National Academies Press (US); 2010. Available from: https://www.ncbi.nlm.nih.gov/books/NBK56189/ doi: 10.17226/12953
[4] Rare Disease Company Coalition. https://www.rarecoalition.com/.

The RDCC would like to thank the Biden Administration and CMS for their commitment to advance health equity and access in the Medicaid population, a goal we strongly support as the rare disease community is underrepresented and underserved in the health care system. Therefore, we appreciate Oregon's goal to make meaningful progress toward health equity for those enrolled in the Oregon Health Plan (OHP), particularly Oregon's goal of maximizing OHP continuity of coverage. We also appreciate that OHA eliminated the proposal for a closed formulary from their waiver request, as it would have had devastating effects on the rare disease community and worked against their health equity goals. The RDCC is, however, deeply concerned that OHA's request for authority to limit the coverage of drugs approved via the U.S. Food and Drug Administration's (FDA or 'the Agency') Accelerated Approval pathway (AAP) from the state's Medicaid drug formulary would only further exacerbate health inequities. We believe excluding AAP drugs from the formulary based on OHA's arbitrary determination the drug has "limited or inadequate evidence of clinical efficacy" is not in the best interest of the health of Oregonians, particularly those with a rare disease. The proposal would deny Medicaid patients access to important medical advances, would allow OHA to override the FDA, and would curtail innovation in the discovery and development of new treatments. Therefore, we urge the Centers for Medicare and Medicaid Services (CMS) to reject the AAP provision included in the Oregon waiver.

Our specific comments and concerns are in brief and in further detail below:

- Improving Access to Early-Childhood Screening and Diagnostics is Critical to Early Diagnosis and Treatment of Rare Diseases
- Continuous Access to Treatment Will Improve Outcomes and Save Lives
- Oregon's 1115 Demonstration Waiver Would Undermine the Intent of the Accelerated Approval Pathway and FDA's Role as the Sole Arbiter of Determining Safety and Efficacy
- This Approach Would Not Promote the Elimination of Health Disparities Nor the Achievement of Health Equity – Two Stated Goals of Oregon's 1115 Waiver Application
- Oregon's 1115 Demonstration Waiver Would Impact Innovation for Rare Disease Treatments and Cures
- Oregon's 1115 Demonstration Waiver is Not Consistent With Foundational Medicaid Policies
- Oregon's 1115 Demonstration Waiver Would Have a Limited Budgetary Impact

Improving Access to Early-Childhood Screenings and Diagnostics is Critical to Early Diagnosis and Treatment of Rare Diseases

We support Oregon's request to provide continuous enrollment for children through the end of the month in which their 6th birthday falls (Section 3.1, Strategy 1), regardless of when they first enrolled in OHP and regardless of changes in circumstances that would otherwise cause a loss of eligibility. We also generally support the state's request to not seek renewal of their Early and Periodic Screening, Diagnostic, and Treatment (EPSDT) waiver, as the EPSDT program is critical to early diagnosis and treatment for children and adolescents on Medicaid. Rare diseases have a significant impact on young children, and many children with a rare disease will not live to see adulthood. On average, a rare disease patient may wait 4-9 years to receive a correct diagnosis.[5] For many rare diseases, early diagnosis and

[5] Zurynski Y, Deverell M, Dalkeith T, Johnson S, Christodoulou J, Leonard H, et al. Australian children living with rare diseases: Experiences of diagnosis and perceived consequences of diagnostic delays. *Orphanet J Rare Dis*. 2017;12: 68; Isono M, Kokado M, Kato K. Why does it take so long for rare disease patients to get an accurate diagnosis?--A qualitative investigation of patient experiences of hereditary angioedema; Shire. *Rare disease impact report: Insights from patients and the medical community.* Published 2013. Accessed April 2022 at: https://globalgenes.org/wp-content/uploads/2013/04/ShireReport-1.pdf.

treatment can markedly improve outcomes and quality of life.[6] The RDCC supports equitable and early access to lifesaving genetic testing and diagnostics to enable earlier diagnosis and treatment for rare diseases and to achieve better health outcomes for children and their families.

The majority of our coalition member companies are developing or have developed therapies for rare diseases that impact children, and we feel this provision and the EPSDT benefit will meaningfully help improve health care access, reduce churn, and improve health outcomes for Oregonian children. However, children diagnosed with a rare disease or other chronic condition through this provision of the OHP waiver and the EPSDT benefit will also need early and equitable access to the medications that could improve or save their lives. In fact, OHA itself recognizes this need for access expressly stating that "barriers to medically necessary care must be removed" to achieve the goal of eliminating health inequities by 2030.[7] Limiting or excluding access to potentially lifesaving drugs the FDA approved through the AAP would create barriers to that early and equitable access and be counterproductive to OHA's efforts to eliminate health inequities (which is discussed further below).

Continuous Access to Treatment Will Improve Outcomes and Save Lives

The RDCC also generally supports the establishment of two-year continuous OHP enrollment for people ages 6 and older (Section 3.1, Strategy 2), regardless of changes in circumstances that would otherwise cause a loss of eligibility. Preserving continuity of coverage, without the need for frequent eligibility renewals, will help to stabilize coverage for older children and adults by reducing churn, improving continuity of care, and improving access to lifesaving treatments. Continuity of care, including continued access to medications, is critical for the rare disease community to prevent progression of the disease and improve quality of life. In addition, the progressive nature of many rare diseases makes continuity of care especially important.

Oregon's 1115 Demonstration Waiver Would Undermine the Intent of the Accelerated Approval Pathway and FDA's Role as the Sole Arbiter of Determining Safety and Efficacy

The RDCC strongly opposes the OHP's request for the authority to exclude drugs approved through the AAP. If CMS approves OHA's request, low-income Oregonians could be denied access to groundbreaking medications. By law (21 U.S.C. § 356(e)(2)), the FDA must find "substantial evidence of effectiveness" to approve any drug, including drugs approved via the AAP. The AAP is a targeted and robust, science-based pathway established by Congress and the FDA to speed the availability of new therapies to patients with serious conditions with unmet needs,[8] while preserving the FDA's rigorous standards for safety and effectiveness. We believe that OHA's request to subject medicines that are approved via the FDA AAP to additional scrutiny based on their pathway for approval undermines the purpose of the AAP and disregards its benefits to patients with unmet medical needs, including those with rare diseases. The AAP has been credited with significant advances in the treatment of life-threatening diseases where patients have limited or no treatment options. As of December 31, 2021, the FDA has approved nearly

[6] Batshaw ML, Groft SC, Krischer JP. Research into rare diseases of childhood. *JAMA*. 2014; 311(17): 1729-30.
[7] See waiver, pg. 39.
[8] Serious condition is defined as: "…a disease or condition associated with morbidity that has substantial impact on day-to-day functioning. Short-lived and self limiting morbidity will usually not be sufficient, but the morbidity need not be irreversible if it is persistent or recurrent. Whether a disease or condition is serious is a matter of clinical judgment, based on its impact on such factors as survival, day-to-day functioning, or the likelihood that the disease, if left untreated, will progress from a less severe condition to a more serious one." 21 CFR 312.300(b)(1); https://www.fda.gov/media/86377/download.

280 medications through the AAP.[9] Although a significant percentage of the agency's accelerated approvals have been for oncology indications,[10] the AAP holds great promise as a critical pathway for rare diseases as well. Targeting AAP therapies could deprive patients who suffer from certain conditions the important, safe, and effective therapies they need. In many cases, these therapies are the only effective course of treatment for their disease.

The FDA's AAP is a well-established, proven, regulated path forward for certain drugs that rely on the use of surrogate or intermediate clinical endpoints to determine the effectiveness of a therapy. Given the current limited disease knowledge and small populations inherent in rare disease drug development, establishing a traditional clinical endpoint is rarely feasible, making surrogate endpoints imperative to addressing the critical unmet need for these patients. Congress, the FDA, and the scientific community[11] have all recognized the important role of surrogate endpoints as relevant and reliable biomarkers to assess effectiveness in certain circumstances, particularly for slowly progressing, debilitating diseases where verification of clinical benefit may take many years.[12]

Both Congress and the FDA have been clear in affirming that accelerated approval does not diminish or compromise FDA's stringent approval standards. Notably, the FDA has maintained that prescription drugs and biologics approved under the AAP must meet clinically meaningful endpoints and that the benefits of the treatment must outweigh the risks of treatment, finding that "[a]pproval…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."[13] Therefore, the AAP meets the same FDA gold standard for drug safety and efficacy as those drugs approved through the traditional approval pathway.

The FDA has noted that the AAP "ensure[s] that therapies for serious conditions are approved and available to patients as soon as it can be concluded that the therapies' benefits justify their risks."[14] The Agency has also emphasized that the AAP is " intended to provide expedited marketing of drugs for patients suffering from such [serious or life-threatening] illnesses when the drugs provide meaningful therapeutic advantage over existing treatment"[15] and that "it is in the public interest to make promising new treatments available at the earliest possible point in time for use in life-threatening and serious illnesses."[16]

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[9] The U.S. Food and Drug Administration. *CDER drug and biologic accelerated approvals based on a surrogate endpoint (As of December 31, 2021)*. Accessed April 2022. https://www.fda.gov/media/151146/download.

[10] Ibid.

[11] Miyamoto BE, Kakkis ED. The potential investment impact of improved access to accelerated approval on the development of treatments for low prevalence rare diseases. *Orphanet J Rare Dis.* 2011; 6. https://doi.org/10.1186/1750-1172-6-49.

[12] The U.S. Food and Drug Administration. Delivering promising new medicines without sacrificing safety and efficacy. Updated 08.27.19. Accessed April 2022 at https://www.fda.gov/news-events/fda-voices/delivering-promising-new-medicines-without-sacrificing-safety-and-efficacy.

[13] See 57 Fed. Reg. at 58944.

[14] U.S. Food and Drug Administration. *FDA-2013-D-0575: Guidance for industry: Expedited programs for serious conditions – Drugs and biologics*. Updated 06.25.20. Accessed April 2022 at https://www.fda.gov/regulatory-information/search-fda-guidance-documents/expedited-programs-serious-conditions-drugs-and-biologics.

[15] See 57 Fed. Reg. 58942.

[16] See 57 Fed. Reg. at 58944.

Oregon's interest in making determinations that would deny coverage for drugs "with limited or inadequate evidence of clinical efficacy" based on the state's own review process is particularly troublesome, as it would constitute a potentially blunt approach to a historically effective expedited pathway for patients in need and widen health disparities in the state of Oregon (discussed more in depth below). Notably, the Center for Drug Evaluation and Research (CDER) reports that 80 percent of drugs approved through the AAP have been converted to full approval.[17] Similarly, the Center for Biologics Evaluation and Research (CBER) reports that 88 percent of drugs approved through the AAP have been converted to full approval.[18] Both centers report that the typical reasons for why a confirmatory trial may be delayed are directly attributable to other factors – such as the trial is awaiting results from another study, additional time was needed to incorporate feedback from the FDA, or difficulty in enrollment for the trial – and not related to a drug's effect or benefit.[19]

The challenges facing rare and ultra-rare diseases for post-marketing studies are unique, particularly with patient recruitment, ethical considerations, and the slow and variable nature of progression for many rare diseases. Recruitment can be difficult in the rare and ultra-rare space due to the small patient population and the potentially limited number of patients eligible to participate. Randomized controlled trials, which typically require the use of placebos, may present ethical challenges for the rare disease population, as there are not always alternative treatments/standards of care to be included as a placebo. Additionally, it can frequently take many years for clinical outcomes to manifest, due to the complex and variable nature of rare disease progression. Therefore, allowing OHA to exclude an AAP drug because it has not completed a confirmatory post-market study by the date listed in the FDA approval letter does not consider the potential reasons for why the confirmatory trial has not yet been completed or valid reasons for which the FDA works with the sponsor and acknowledges delays, and effectively allows OHA to label the drug ineffective based on their definition of "limited or inadequate evidence of clinical efficacy." This mixed messaging from OHA and the FDA would undermine FDA's scientific rigor and expertise and create confusion and further distrust amongst the public for drugs approved through the AAP.

Finally, the waiver significantly lacks detail in what criteria the state would use in its determinations, who the responsible entity would be for conducting the review, or if an appeal process would be available to beneficiaries who want to appeal coverage decisions made by the state. In addition, we believe it is inappropriate for OHA to substitute its own judgment for determinations related to clinical efficacy of drugs that have been reviewed and approved by the FDA. The FDA remains the gold standard for drug safety and efficacy. Such an approach implies that Oregon is better suited to review drugs than the scientists and disease experts at the FDA. Establishing such a framework undermines the important, independent authority of the FDA as the sole arbiter of safety and efficacy.

<u>This Approach Would Not Promote the Elimination of Health Disparities Nor the Achievement of Health Equity – Two Stated Goals of Oregon's 1115 Waiver Application</u>

OHA's request for authority to exclude coverage for drugs approved via the AAP is in direct conflict with the state's purported goal of the 1115 Demonstration waiver: "to eliminate inequitable access with strategies to extend and stabilize coverage to every eligible child and adult in Oregon." The Health

[17] Reagan-Udall Foundation for the FDA. Accelerated approval program: 30 years on-insights and experiences. Presented March 11, 2022. Accessed April 2022 at https://reaganudall.org/sites/default/files/2022-03/Slide%20deck%20031622.pdf.
[18] Ibid.
[19] Ibid.

Equity Committee, a subcommittee of the Oregon Health Policy Board (OHPB) was tasked with coordinating and developing policy that proactively promotes the elimination of health disparities and the achievement of health equity for all people in Oregon and the state has a goal of "eradicating health inequities by 2030." This is an admirable goal for the state, as the US Census Bureau estimates that 11 percent of the state's population is considered low-income.[20] In addition, the Biden Administration made advancing equity a major commitment of his Presidency by executing two executive orders on his first two days in office, including one specifically on addressing health inequities from the COVID-19 pandemic and beyond through the formation of a COVID-19 Health Equity Task Force.[21,22] This year, the Administration also announced a request for information on "Access to Care and Coverage for People Enrolled in Medicaid and CHIP."[23] Rather than making progress toward Oregon's goal of eradicating health disparities and the Biden Administration's goal of advancing health equity, Oregon's proposed exclusion of AAP treatments would exacerbate health inequities for rare disease patients, especially those who are also part of racial and/or ethnic minority groups. Without coverage of a potential life-altering and lifesaving AA treatment, low-income Oregonians with a rare disease could have worse health outcomes and increased cost of chronic care compared to Oregonians not in the OHP.

Individuals with rare diseases tend to report similar concerns that result from being underserved, such as a long road to diagnosis, limited treatment options, and a need for research to better understand their medical condition.[24] Racial and ethnic minority groups with a rare disease face even more disparities in access to care and are underrepresented in research and clinical trials.[25] These disparities make timely diagnosis and adequate treatment exponentially harder, resulting in poorer health outcomes. Additionally, the Covid-19 pandemic has had a devastating impact on the rare disease community, particularly in communities of color, and has highlighted many of the disparities in our health care system. These patients may also face greater disease burden, earlier age of disease onset, and/or complex sets of comorbidities that limit the safety and effectiveness of older treatment options. We believe the Oregon approach sends a message to patients with high unmet needs that their lives are not worth the investment; we strongly oppose that sentiment. For all these health equity concerns, CMS should deny OHA's request for the authority to exclude drugs approved through the AAP, as it would go against Oregon and the Biden Administration's efforts to reduce health inequities.

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[20] United States Census Bureau. QuickFacts-Oregon. Published July 2021. Accessed at https://www.census.gov/quickfacts/fact/table/OR/PST045221.

[21] The White House. Executive order on advancing racial equity and support for underserved communities through the Federal Government. Published January 20, 2021. Accessed April 2022 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/.

[22] The White House. Executive order on ensuring an equitable pandemic response and recovery. Published January 21, 2021. Accessed April 2022 at https://www.whitehouse.gov/briefing-room/presidential-actions/2021/01/21/executive-order-ensuring-an-equitable-pandemic-response-and-recovery/.

[23] The Department of Health and Human Services. *Biden-Harris administration announces request for information on access to care and coverage for people enrolled in Medicaid and CHIP*. Published February 17, 2022. Accessed April 2022 at https://www.hhs.gov/about/news/2022/02/17/biden-harris-administration-announces-request-for -information-on-access-to-care-and-coverage-for-people-enrolled-in-medicaid-and-chip.html.

[24] National Organization for Rare Disorders. Barriers To Rare Disease Diagnosis, Care and Treatment In The US: A 30-Year Comparative Analysis. Published November 19, 2020. Accessed April 2022 at https://rarediseases.org/wp-content/uploads/2020/11/NRD-2088-Barriers-30-Yr-Survey-Report_FNL-2.pdf.

[25] The Rare Disease Diversity Coalition. *About the Coalition*. Accessed April 2022 at https://www.rarediseasediversity.org/about-the-coalition.

Oregon's 1115 Demonstration Waiver Would Impact Innovation for Rare Disease Treatments and Cures

Rare diseases present specific challenges – such as small population sizes, or slow, irreversible, and variable disease progression – that make it difficult, in some cases, to study using clinical measures as endpoints. The AAP is a critical tool, well-suited to recognize these unique circumstances. For example, the AAP is credited for changing the trajectory of scientific advancement for HIV/AIDS and for oncology.[26] If these drugs are not covered by state Medicaid programs, thereby limiting patient access, manufacturers will have significantly reduced incentive to pursue research and development in rare diseases.

Oregon's approach simply does not align with the intent of the AAP to expedite access to rare disease treatments for patients with serious conditions, especially for diseases that have limited or no treatment options. We are concerned about the impact to these patients and the broader impact on the entire rare disease community, regardless of payor, as we believe the OHA 1115 Demonstration Waiver would potentially significantly reduce incentives for research and development for the treatment of certain rare diseases. Ultimately, this approach would punish patients who are forced to suffer as their disease progresses while continuing to wait for a treatment and further inequities in health for Oregonians who lose/never gain access to these treatments.

Oregon's 1115 Demonstration Waiver is Not Consistent With Foundational Medicaid Policies

OHA's request would violate the longstanding federal open-formulary requirement in which state Medicaid programs must cover all FDA-approved drugs,[27] including those approved under AAP, to ensure low-income Medicaid beneficiaries have access to the prescription drugs they need. The Medicaid Drug Rebate program represents a carefully balanced compromise made by Congress under the Omnibus Reconciliation Act of 1990 to ensure the federal 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.

CMS reiterated this requirement most recently in the National Coverage Decision (NCD) finalized for Aduhelm. In the fact sheet accompanying the NCD, CMS affirms that "Generally, states are required to cover the drug if the manufacturer has in effect a National Drug Rebate Agreement with HHS and when the drug is used for a medically accepted indication, subject to any permissible restrictions or limitations on coverage applied by the state (e.g., prior authorization). Because the manufacturer of Aduhelm has entered into and has in effect a Medicaid drug rebate agreement and because Aduhelm also satisfies the definition of a covered outpatient drug, as set forth at section 1927(k)(2) of the Social Security Act (Act), state Medicaid programs are required to cover the drug when used for a medically accepted indication."[28]

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[26] Siegel RL, Miller KD, Jemal A. *Cancer statistics, 2020*. CA Cancer J Clin. 2020; 70:7-30.

[27] Department of Health and Human Services. *Medicaid drug rebate program notice. Release No. 185. For State technical contacts*. Published June 27, 2018. Accessed April 2022 at https://www.medicaid.gov/medicaid-chip-program-information/by-topics/prescription-drugs/downloads/rx-releases/state-releases/state-rel-185.pdf.

[28] Centers for Medicare and Medicaid Services. Medicare coverage policy for monoclonal antibodies directed against amyloid for the treatment of Alzheimer's Disease. Published April 7, 2022. Accessed April 2022 at https://www.cms.gov/newsroom/fact-sheets/medicare-coverage-policy-monoclonal-antibodies-directed-against-amyloid-treatment-alzheimers-disease.

The Oregon demonstration waiver would disrupt this long-term, successful Medicaid policy that has facilitated access to medicines for beneficiaries in a fiscally responsible way.

Oregon's 1115 Demonstration Waiver Would Have a Limited Budgetary Impact

Historical claims analysis suggests that limiting Medicaid access to AAP drugs would not likely yield significant savings to OHA. From 2007 to 2018, AAP drugs account for less than one percent of overall Medicaid spending while often representing the only treatment options available for beneficiaries.[29] The same analysis continues to conclude that "[l]imiting Medicaid coverage for accelerated approval drugs would have a devastating impact on patients benefiting from these treatments while having a de minimis impact on spending."[30] In addition, one of the authors completed an Oregon specific analysis, which found that in 2020, AAP drugs accounted for only 4 percent of total Medicaid spending in Oregon and just 0.5 percent of total Medicaid spending growth between 2015 and 2020.[31] This percent growth is relative to the 31 percent growth in total Medicaid spending in the state during the same time span. Therefore, excluding or limiting the AAP in OHP would not likely yield significant savings to OHA, but rather restrict access to critical treatments to OHP beneficiaries.

Conclusion

We appreciate the opportunity to provide comments on the OHA's section 1115 Demonstration Waiver Renewal and Amendment application. The RDCC is greatly concerned about the negative impact excluding AAP drugs from the OHP will have on the rare disease community. The proposal will not promote the OHA's purported goal of eliminating disparities nor achieve the Biden Administration's efforts to advance health equity. We ask CMS to weigh the potential impacts the proposed changes could have on the rare disease community, both in Oregon and across the nation, as maintaining access to drugs through the AAP can be a matter of life, quality of life, and hope for the millions of individuals with rare diseases that rely on the Medicaid program for their health care.

Should you have any questions, please feel free to contact me at patroski@kpmgroupdc.com.

Sincerely,

Patroski Lawson
Interim Executive Director
Rare Disease Company Coalition (RDCC)

[29] Thorpe KE, Holtz-Eakin D. Limiting Medicaid access to accelerated approval drugs: Costs and consequences. *Am J Manag Care*. 2021;27(6):e178e180. https://doi.org/10.37765/ajmc.2021.88596
[30] Ibid.
[31] Thorpe, KE. Blog: Debunking Oregon's cost argument in denying access to accelerated approval drugs. Published March 15, 2022. Accessed April 2022 at https://www.fightchronicdisease.org/blog/debunking-oregon%E2%80%99s-cost-argument-denying-access-accelerated-approval-drugs.