Enactment of the EPICrd Act [PFLUGE 193 timestamped July 22, 2024 @ 3:04 pm] would ensure access to medicine for patients diagnosed with rare disorders by improving Medicaid prior authorization for covered outpatient drugs, among other vital policy reforms.

Medical necessity: For a covered outpatient drug prescribed for a rare pediatric disease or a drug that would satisfy an unmet need or improve the standard of care for a rare disease, the bill would establish a streamlined process in Medicaid for prescribers to establish "medical necessity" of the medicine for the individual patient. Once the prescriber establishes medical necessity, the drug would receive an immediate one-year authorization, which would have the effect of prohibiting step therapy and other coverage restrictions.

- ***Rationale:*** Medical necessity should be determined by the prescriber, not the state Medicaid plan. Legislative history of the Medicaid Drug Rebate Program ("MDRP") demonstrates that Congress never intended for states to establish "prior authorization controls that have the effect of preventing competent physicians from prescribing in accordance with their medical judgment."[1] Medical necessity, however, is not defined in statute or regulation, which has given states wide latitude to establish discriminatory coverage policies for prescription drugs.

State prior authorization response: The bill would require state Medicaid plans to make a minimum of three attempts to notify the prescriber, pharmacist, and the patient of the response to a prior authorization request. If such response is a denial, the bill would require the state to provide the prescriber and patient with a subsequent written notification in multiple languages that details the evidentiary basis for the denial, including any coverage criteria relied upon, and instructions for requesting an appeal with an Administrative Law Judge ("ALJ").

- ***Rationale:*** Ensuring prior authorization decisions by a Medicaid plan are appropriately communicated to all relevant parties, and, in the case of a denial, a subsequent written notification in multiple languages to the prescriber and patient detailing the basis of the denial and the appeals process, will allow them to make informed decisions on next steps. Current law requires a decision within 24 hours of the prior authorization request but does not specify to whom such decision is to be provided, the number of attempts the state must make, nor the content of the communication in the event of a denial.[2] These improvements will provide much needed transparency and allow patients and prescribers to meet deadlines for submitting a well-reasoned appeal.

Appeals of denials: The bill would require an initial appeal hearing to a prior authorization denial to be held before an ALJ within 72 hours of a request by the patient or prescriber. The prescriber or beneficiary must request the appeal within 30 days of the denial. Such hearing can occur in person or by video conference or telephone. The ALJ must provide written adjudication within 48 hours of the hearing. Either party may appeal the hearing decision within 60 days to the U.S. District Court for the District of Columbia or a federal district court in which the parties are located.

[1] H.R. REP. NO. 101-881 at 98.

[2] *See* Social Security Act ("SSA") § 1927(d)(5)(A), 42 U.S.C.S. § 1396r-8(d)(5)(A) (LexisNexis 2024).

- ***Rationale:*** A uniform drug appeals process that requires states to provide their Medicaid beneficiaries a hearing before an ALJ would ensure their due process because the hearing decision would be made independent of the bureaucrats that made the initial determination. Such a fair and impartial forum is consistent with the nationwide ALJ program for Medicare.[3] Currently in Medicaid, while some states provide for ALJ hearings on first level appeals, most states do not.[4] Indeed, most first level reviews of a drug prior authorization denial in Medicaid are conducted by hearing officers from within the state Department of Health or a contractor. Such a conflict of interest makes a fair hearing impossible. This provision ensures impartiality at the outset.

Access for stable patients during appeal: The bill would require continued administration or dispensing of a covered outpatient drug for the duration of the appeals process if the prescriber determines the Medicaid beneficiary is currently stable on such drug.

- ***Rationale:*** Establishing a continuity of care protocol to expressly cover Medicaid beneficiaries who are stable on a specified therapy for the duration of the appeals process fills a policy gap in the MDRP. Current law requires a state plan to provide a 72-hour emergency supply of drug during the prior authorization process.[5] States, however, are not obligated to supply this medicine for the duration of the *appeals* process.[6] Providing clarification to require continued administration or dispensing of therapy for the duration of the appeals process in the case of beneficiaries who are stable is a natural extension of the emergency supply provision and will help ensure better patient outcomes.

Publication of coverage criteria: The bill would require state Medicaid plans to develop and publish coverage criteria for a covered outpatient drug approved for a rare disease within 60 days of its approval (or in the case of previously approved drugs, within 180 days of enactment). Any modifications must be published within 45 days.

- ***Rationale:*** Requiring states to generate and publish coverage criteria for rare disease therapies will establish a more equitable environment for Medicaid beneficiaries as they navigate the prior authorization process. It is not uncommon for a state Medicaid plan to cite to an unpublished coverage policy it has generated in denying a Medicaid beneficiary access to therapy, or argue it is unable to cover the medicine because a coverage policy does not exist. This provision would prevent such inequities from continuing.

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[3] *See* 42 C.F.R. § 405.1000 (LexisNexis 2024).

[4] *Compare The Medicaid Recipient Appeal Hearing*, NORTH CAROLINA OFFICE OF APPEALS HEARINGS, https://www.oah.nc.gov/hearings-division/medicaid-recipient-appeals/medicaid-recipient-appeal-hearing *with Provider Disputes and Appeals*, CARE SOURCE GEORGIA, https://www.caresource.com/ga/providers/provider-portal/appeals/medicaid/#clinicalappeal.

[5] *See* SSA § 1927(d)(5)(B), 42 U.S.C.S. § 1396r-8(d)(5)(B) (LexisNexis 2024).

[6] *Id*. (emphasis added).

DUR Board and P&T Committee reviews: In the event a DUR Board, P&T Committee, or similar entity announce a review of a one or more drugs approved for a rare disease and their use in such disease, the bill requires such entity to notify relevant stakeholders and empanel a physician and patient expert (leveraging state Rare Disease Advisory Councils where applicable) as a voting member. Any prior authorization coverage criteria or formulary placement resulting from such review must be based on consultation with nationally recognized physician experts in the rare disease, stakeholder testimony, peer-reviewed medical literature or treatment guidelines, and real-world evidence.

- ***Rationale:*** Medicaid utilization management, whether it is for formulary placement or prior authorization coverage criteria, must reflect the experience and knowledge of clinicians with expertise in the condition and affected patients and caregivers. Under current law, there is no minimum standard of evidence to consider when establishing a Medicaid drug coverage policy. Typically, DUR Boards and P&T Committees exclusively consider cost effectiveness analysis in determining how to restrict access. For example, Kaiser Family Foundation recently published survey data that demonstrate more than two-thirds of state Medicaid plans rely on such studies by the Institute for Clinical and Economic Review ("ICER"), and other third parties.[7] ICER relies on quality adjusted life years ("QALYs") for measuring cost-effectiveness for rare disease therapies.[8] The use of QALYs, however, is widely held to be inappropriate for assessing the value of rare disease therapies.[9] Consideration of the patient and medical expert perspective will help ensure that the nuances of the epidemiology, diagnosis, clinical course, and standard of care for the rare disorder, as well as the clinical trial data, mechanism of action, and real-world evidence for the therapy are well understood by the entity establishing the coverage criteria.

Section 1115 waivers: This bill would expressly exclude restrictive prescription drug coverage policies from the scope of 1115 Medicaid demonstration waiver projects.

- ***Rationale:*** Congressional intervention is necessary to reconcile inconsistent interpretations of the 1115 waiver program, which has resulted in discriminatory drug coverage policies that acutely affect patients with rare diseases. In general, a state may obtain a section 1115 demonstration project waiver to relax its obligations under section 1902 of the Social Security Act ("SSA").[10] The interaction of sections 1115, 1902, and 1927 of the SSA, however, prevents state Medicaid plans from using the waiver to restrict drug coverage if participating in the MDRP. Section 1902(a)(54) of the SSA provides a state with the option to offer a prescription

[7] *See* KAISER FAMILY FOUNDATION, HOW STATE MEDICAID PROGRAMS ARE MANAGING PRESCRIPTION DRUG COSTS: RESULTS FROM A STATE MEDICAID PHARMACY SURVEY FOR STATE FISCAL YEARS 2019 AND 2020 at 12 (April 2020), https://files.kff.org/attachment/How-State-Medicaid-Programs-are-Managing-Prescription-Drug-Costs.pdf.

[8] *See* INST. FOR CLINICAL AND ECON. REV., MODIFICATIONS TO THE ICER VALUE ASSESSMENT FRAMEWORK FOR TREATMENTS FOR ULTRA-RARE DISEASES 7-9, 11-12 (Nov. 2017), https://icer.org/wp-content/uploads/2020/10/ICER-Adaptations-of-Value-Framework-for-Rare-Diseases.pdf.

[9] *See, e.g.*, H.I. Hyry et al, *Limits on Use of Health Economic Assessments for Rare Diseases*, 107(3) QJM: AN INTERNATIONAL JOURNAL OF MEDICINE 241 (Mar. 2014) (criticizing QALYs for the arbitrary cost threshold and insufficient inputs that fail to capture the value of rare disease therapies to the patient).

[10] *See* SSA § 1115, 42 U.S.C.S. § 1315 (LexisNexis 2024).

drug benefit to its beneficiaries by *requiring such state who chooses to do so to comply with section 1927 of the SSA*,[11] which typically requires payment for covered outpatient drugs if the manufacturer has a rebate agreement in place with the Centers of Medicare & Medicaid Services ("CMS") and certain criteria are satisfied. CMS, however, has failed to consistently interpret the interaction of these three sections of the SSA, which could result in barriers to rare disease therapy access. More specifically, after allowing a discriminatory waiver program in Oregon since the inception of the MDRP, CMS denied a request for closed formularies by Massachusetts, but initially approved a similar waiver for Tennessee.[12] CMS has recently reversed course in Oregon and Tennessee,[13] but the uncertainty that states could obtain approval under a different administration to use 1115 waivers to deny access to lifesaving medicine must be stopped by Congress. Legislative precedent exists for closing the loophole that allows Medicaid plans to establish restrictive drug coverage policies through 1115 waivers. Indeed, the House of Representatives previously passed legislation that would have expressly required a state under a section 1115 waiver to continue to meet the requirements of sections 1902(a)(54) and 1927 of the SSA, but the provision was struck when Congress reconciled it with the Senate version.[14]

Categories and classes of rare diseases: For drugs and biologicals indicated for a rare disease or condition, the bill would expressly prohibit qualified health plan offered in the Affordable Care Act ("ACA") Marketplace, an Alternative Benefit Plan, which cover the ACA expanded category of Medicaid

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[11] *See* SSA § 1902(a)(54), 42 U.S.C.S. § 1396a(a)(54) (LexisNexis 2024) (emphasis added).

[12] *Compare* Letter from Donna Shalala, Sec'y, HHS to Barbara Roberts, Governor, Oregon (Mar. 19, 1993) (approving Oregon's waiver to establish a Prioritized Health Services List, which for decades, violated the conditions of its approval by relying on quality adjusted life years to deny FDA-approved therapies for children with rare genetic disorders), *with* Letter from Tim Hill, Acting Dir., Ctr. for Medicaid & CHIP Servs., HHS, to Daniel Tsai, Assist. Sec., MassHealth at 2 (June 27, 2018) (suggesting the Massachusetts' waiver proposal to exclude certain covered outpatient drugs while continuing to collect Medicaid is not permissible under the SSA) *and* Letter from Seema Verma, Admin., CMS, HHS, to Stephen Smith, Director of TennCare, Tennessee Department of Finance and Administration at 6 (Jan. 8, 2021) (conflicting with the Massachusetts analysis in approving Tennessee's request for a section 1115 waiver to deny certain medications where there is at least one drug available per therapeutic class under Essential Health Benefits rules (with the exception of certain protected drug classes), and to exclude certain new drugs from its formulary, with an exceptions process for specialty drugs). HHS initially denied Oregon's waiver for being discriminatory in violation of the Americans with Disabilities Act. *See* Letter from Louis W. Sullivan, M.D., Sec'y, HHS to Barbara Roberts, Governor, Oregon (Aug. 3, 1992), *reprinted in ADA Analyses of the Oregon Health Care Pan*, 9 ISSUES IN L. & MED. 397, 409-412 (1994).

[13] *See* Letter from Chiquita Brooks-LaSure, Administrator, CMS, to Dana Hittle, Interim Medicaid Dir., Oregon Health Authority (Sept. 28, 2022) (extending the section 1115 waiver to Sept. 30, 2027, but phasing out the Prioritized List of Health Services by Jan. 1, 2027 and requesting Oregon withdraw its request for closed formularies and flexibilities to restrict access to accelerated approval drugs); TennCare III Demonstration, Amendment 4 Letter from Tennessee to CMS (August 30, 2022) (agreeing to the CMS request to remove the closed formulary provision from its section 1115 waiver).

[14] *Compare* § 5108(b)(1)(E) of the Omnibus Budget Reconciliation Act of 1993, H.R. 2264, 103rd Cong., (as passed by the House on May 27, 1993) *with* Omnibus Budget Reconciliation Act of 1993, Pub. L. No. 103-66, 107 Stat. 312.

beneficiaries, all non-grandfathered health plans in the individual and small group markets outside the ACA Marketplace, the Basic Health Plan, Medicare, Medicaid, CHIP, TRICARE, and the VA from using the category and class coverage floor described in the United States Pharmacopeia ("USP") Medicare Model Guidelines ("MMG"). It would instead require the NIH Office of Rare Diseases to develop and maintain an appropriate list of rare disease therapy categories and classes.

- ***Rationale:*** The USP MMG fails to provide an adequate drug coverage floor for patients affected with a life-threatening or debilitating, chronic rare diseases. Several payors, including Medicare Part D, Medicare Advantage, and those that must cover essential health benefits (a qualified health plan offered in the ACA Marketplace, an Alternative Benefit Plan, which cover the ACA expanded category of Medicaid beneficiaries, all non-grandfathered health plans in the individual and small group markets outside the ACA Marketplace, and the Basic Health Plan), are permitted to establish prescription drug benefits with restrictive formularies based on categories and classes described in the USP MMG.[15] Traditional Medicaid plans have attempted through section 1115 waivers to rely upon the USP MMG in such coverage restrictions.[16] The USP MMG categories and classes, however, do not appropriately reflect differences among therapies or the rare disorders for which they are indicated. Simply put, the USP MMG undermines science and common sense by creating singular categories and classes that combine multiple drugs with unique mechanisms of action for multiple disorders with varying pathophysiology, clinical manifestations, and rates of progression. For example, the "Genetic or Enzyme or Protein Disorder: Replacement, Modifiers, Treatment" category in USP MMG v9.0 includes *53 branded drugs for 46 unique disorders* without any further separation for therapeutic class based on indication and mechanism of action, or the significant clinical differences characterizing these devastating conditions.[17] Plans could technically comply with minimum coverage requirement for this drug category while potentially *not covering any* FDA-approved therapies for achondroplasia, alpha-1 antitrypsin deficiency, CLN2, Duchenne muscular dystrophy, hypophosphatasia, MPS types I, II, IVA, VI, and VII, phenylketonuria, sickle cell disease, and urea cycle disorders, among others.

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[15] *See* 42 U.S.C.S. § 1395w-104(b)(3) (establishing the USP MMG for developing Medicare Part D formularies); 42 C.F.R. § 423.120(b)(2) (requiring Part D plans to cover a minimum of two drugs in each USP MMG category and class); 42 U.S.C.S. § 1395w-21(a)(1) (providing that individuals enrolled in Medicare Advantage can elect a prescription drug plan, which is governed under Medicare Part D); 45 C.F.R. § 156.122 (requiring plans subject to offering EHBs to cover a minimum of one drug in each USP MMG category and class); Patient Protection and Affordable Care Act; Standards Related to Essential Health Benefits, Actuarial Value, and Accreditation, 78 Fed. Reg. 12834, 12857 (Feb. 25, 2013) (describing those plans subject to the drug coverage requirement in 45 C.F.R. § 156.122).

[16] *Compare* Verma Letter, *supra* note 12, *with* TennCare III Demonstration, *supra* note 13.

[17] *See* USP MEDICARE MODEL GUIDELINES v9.0 25-26 (Sept. 29, 2023), https://go.usp.org/l/323321/2023-09-25/91x9zt/323321/1695931732h74PBnPC/USP_Medicare_Model_Guidelines_v9.0__With_Example_Part_D_Drugs__1.0_.pdf (emphasis added).