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(Original Signature of Member)

118TH CONGRESS
2D SESSION

H. R. _____

To amend title XIX of the Social Security Act to ensure appropriate access to covered outpatient drugs under the Medicaid program.

IN THE HOUSE OF REPRESENTATIVES

Mr. PFLUGER introduced the following bill; which was referred to the Committee on _____

A BILL

To amend title XIX of the Social Security Act to ensure appropriate access to covered outpatient drugs under the Medicaid program.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the ''Ensuring Parity through Individualized Care for Rare Disorders Act of 2024''.

SEC. 2. MEDICAID ACCESS TO COVERED OUTPATIENT DRUGS.

(a) LIMITING THE SCOPE OF MEDICAID WAIVERS.—Section 1115(a)(1) of the Social Security Act (42 U.S.C. 1315(a)(1)) is amended by inserting ''(other than subsection (a)(54) of such section in the case such project would deny, restrict, or otherwise limit access to covered outpatient drugs (as defined in section 1927(k)))'' after ''1902''.

(b) PROHIBITION AGAINST USING THE USP MEDICARE MODEL GUIDELINES IN CERTAIN CIRCUMSTANCES.—

(1) FEDERAL HEALTH CARE PROGRAMS.—Notwithstanding any other provision of law, in carrying out coverage and payment provisions (including provisions relating to the establishment of formularies) for drugs under any Federal health care program (as defined in section 1128B of the Social Security Act (42 U.S.C. 1320a–7b)), to the extent that such provisions rely on a categorization or classification of such drugs, a Federal agency (and any entity carrying out such program on behalf of such agency) shall, with respect to drugs with a medically accepted indication for treatment of a rare disease or condition (as defined in paragraph (5)), rely solely on

the categorizations and classifications of such drugs on the list described in paragraph (4).

(2) MEDICAID AND CHIP WAIVERS.—Notwithstanding any other provision of law, the Secretary of Health and Human Services may not approve any waiver relating to the Medicaid or CHIP program under titles XIX and XXI, respectively, of the Social Security Act (42 U.S.C. 1396 et seq., 1397aa et seq.) to the extent that such waiver would allow for the imposition of coverage or payment restrictions on drugs with a medically accepted indication for treatment of a rare disease or condition based on categorizations and classifications of such drugs other than such categorizations and classifications on the list described in paragraph (4).

(3) ESSENTIAL HEALTH BENEFITS REQUIREMENTS.—In determining whether a group health plan or group or individual health insurance coverage (as such terms are defined in section 2791 of the Public Health Service Act (42 U.S.C. 300gg–91)), benchmark benefit package or benchmark equivalent coverage (as described in paragraphs (1) and (2), respectively, of section 1937(b) of the Social Security Act (42 U.S.C. 1396u–7)), basic health plan (as described in section 1331 of the Patient

Protection and Affordable Care Act (42 U.S.C. 18051)), or any other form of health benefits coverage required to provide the essential health benefits described in section 1302(b) of such Act (42 U.S.C. 18022(b)) provides adequate coverage of prescription drugs, to the extent that such determination relies of the categorization or classification of such drugs, a Federal agency shall, with respect to drugs with an medically accepted indication for treatment of a rare disease or condition, rely solely on the categorizations and classifications of such drugs on the list described in paragraph (4).

(4) ALTERNATIVE BENCHMARK FOR RARE DISEASE THERAPIES.—The Division of Rare Diseases Research Innovation within the National Center for Advancing Translational Science at the National Institutes of Health shall develop and maintain a list of categories and classes of drugs with a medically accepted indication for a rare disease or condition based on therapeutic mechanism of action and disease characteristics, as provided in section 481(b)(3) of the Public Health Service Act.

(5) DEFINITIONS.—In this subsection:

(A) MEDICALLY ACCEPTED INDICATION.—The term ''medically accepted indication'' has

the meaning given such term in section 1860D–2(e)(4) of the Social Security Act (42 U.S.C. 1395w–102(e)(4)).

(B) RARE DISEASE OR CONDITION.—The term ''rare disease or condition'' means a disease or condition that affects fewer than 200,000 individuals in the United States.

(c) IMPROVED APPLICATION OF PRIOR AUTHORIZATION.—Section 1927(d) of the Social Security Act (42 U.S.C. 1396r–8(d)) is amended—

(1) in paragraph (5)—

(A) in the matter preceding subparagraph (A), by striking ''A State plan'' and inserting ''Subject to paragraph (8), a State plan'';

(B) in subparagraph (A), by striking ''by telephone'' and all that follows and inserting ''to the prescribing physician (or other individual authorized to prescribe under State law), pharmacist, and the individual receiving medical assistance under this title by telephone or other telecommunication device within 24 hours of a request for prior authorization, making a minimum of three attempts to confirm acknowledgment of such response by the notified party;'';

(C) by striking subparagraph (B) and inserting the following:

''(B) in the case such response is a denial—

''(i) not later than 1 business day after such response is made, provides by mail to the prescriber of such drug (and to the individual prescribed such drug) a written explanation in English, Spanish, and the 3 other languages most commonly spoken in the zip code where such individual resides (according to the most recent census information), using a template specified by the Secretary and published on the internet website of the Centers for Medicare & Medicaid Services, which details—

''(I) the evidentiary basis for such denial, including any published or unpublished coverage criteria for the covered outpatient drug; and

''(II) clear written instructions, including a specification of any deadlines, for requesting an appeal of such

denial in accordance with subparagraph (E); and

''(ii) provides for a 30-day period beginning on the date of such denial for such prescriber, pharmacist, or individual to appeal such denial;

''(C) except with respect to the drugs on the list referred to in paragraph (2), provides for the dispensing or administration of—

''(i) at least 72-hour supply of a covered outpatient drug in an emergency situation (as defined by the Secretary); and

''(ii) a covered outpatient drug for the duration of the appeals process described in subparagraph (E) if such drug has, in the opinion of the prescriber, controlled or improved the condition of the individual for whom it is being prescribed during the 180-day period before the date of the request for approval of the drug under this paragraph;

''(D) ensures that, with respect to a drug subject to such system with a medically accepted indication (as defined in subsection (k)(6))

for the treatment of a rare disease or condition—

''(i) all coverage criteria for such drug under such system are developed and made available on a public website not later than 60 days after such drug is approved by the Food and Drug Administration (or, in the case of such a drug that is already so approved as of the date of the enactment of this subparagraph, not later than 180 days after such date of enactment); and

''(ii) in the case of a modification of such criteria, such criteria are updated on such website not later that 45 days after such modification; and

''(E) ensures that—

''(i) not later than 72 hours after receipt of an appeal made during the period described in subparagraph (B)(ii), an initial hearing before an administrative law judge is held, which shall be conducted by video conference or other form of telecommunication if requested by such prescriber or individual; and

''(ii) not later than 48 hours after such a hearing, such judge provides a written adjudication to such prescriber and individual.

Not later than 60 days following the date of a hearing described in subparagraph (E), a party to such hearing may file a petition to review the adjudication described in subparagraph (E)(ii) made with respect to such hearing in the United States District Court for the District of Columbia Circuit or the district in which a party is located.''; and

(2) by adding at the end the following new paragraphs:

''(8) THERAPIES FOR CERTAIN RARE DISORDERS.—

''(A) IN GENERAL.—In the case of a qualifying rare disease therapy (as defined in subparagraph (C)) prescribed to an individual on or after January 1, 2025, if the prescribing physician (or other individual authorized to prescribe under State law) making such prescription submits the information described in subparagraph (D) with respect to such therapy, a State shall—

''(i) deem such therapy to be medically necessary for such individual;

''(ii) expeditiously grant approval for such therapy under any program described in paragraph (5) applicable to such therapy; and

''(iii) not apply any other requirement or limitation on the coverage of such therapy (such as step therapy) unless such requirement or limitation is specified in the indication and usage section of the label of such therapy.

''(B) DURATION.—Any authorization under a program described in paragraph (5) for a qualifying rare disease therapy shall be effective for a period of not less than 1 year.

''(C) QUALIFYING RARE DISEASE THERAPY DEFINED.—The term 'qualifying rare disease therapy' means a covered outpatient drug that is prescribed for—

''(i) a medically accepted indication for a rare pediatric disease (as defined in section 529(a)(3) of the Federal Food, Drug, and Cosmetic Act); or

''(ii) an approved or licensed use that received an exclusivity under section 527 of the Federal Food, Drug, and Cosmetic Act and—

''(I) was designated as a breakthrough therapy under section 506(a) of such Act;

''(II) received fast-track approval under section 506(b) of such Act;

''(III) was designated as a regenerative medicine advanced therapy under section 506(g) of such Act; or

''(IV) was designated as a Food and Drug Administration priority for being a significant improvement in the safety or effectiveness of the treatment of such disease or condition compared to available therapies.

''(D) PRESCRIBER SUBMISSIONS.—For purposes of subparagraph (A), the information described in this subparagraph is, with respect to a qualifying rare disease therapy prescribed to an individual—

''(i) the diagnosis code for such individual;

''(ii) evidence, including a diagnostic test result or a description of symptoms, supporting such diagnosis; and

''(iii) an attestation that use or continued use of such therapy by the individual is necessary for an individualized course of treatment that is reasonably likely to—

''(I) prevent the onset of the disease or condition, or episodes, illnesses, injuries, or disabilities related to the disease or condition;

''(II) slow, halt, or reverse disease progression;

''(III) reduce or ameliorate the physical, cognitive, or psychosocial effects of the disease or condition; or

''(IV) allow for the individual to achieve or maintain maximum functional capacity in performing daily activities.

''(9) DUR BOARDS AND P&T COMMITTEES.—

''(A) IN GENERAL.—In the event a DUR board described in subsection (g)(3) or other entity (including a pharmacy and therapeutics

committee) holds a public meeting with respect to coverage or payment policies under a State plan (or waiver of such plan) relating to covered outpatient drugs on or after January 1, 2025, such board or entity shall publish on the internet website of the State Department of Health of such State a meeting agenda 60 days prior to such meeting.

''(B) RARE DISEASE DRUG REVIEWS.—

''(i) NOTIFICATION.—In the event the agenda for a meeting described in subparagraph (A) explicitly indicates an intention to review coverage or payment policies for a covered outpatient drug approved under section 505(b) of the Federal Food, Drug, and Cosmetic Act or licensed under section 351(a) of the Public Health Service Act for a rare disease or condition (as defined in section 2(b)(5) of the Ensuring Parity through Individualized Care for Rare Disorders Act of 2024), such board or entity shall, not later than 5 business days after publishing such agenda, notify—

''(I) national chapters of medical specialty societies and patient advo-

cacy and research organizations with expertise in such disease or condition (as selected from a list that the Division of Rare Diseases Research Innovation within the National Center for Advancing Translational Science at the National Institutes of develops and updates in accordance with section 481(b)(3)(A) of the Public Health Service Act); and

''(II) the manufacturer of such drug.

''(ii) EXPERT CONSULTATION.—Not later than 10 days prior to a meeting described in subparagraph (A) with respect to which notifications are required to be made under clause (i), the DUR board or other entity conducting the review of the drug described in such clause shall consult with at least 3 nationally recognized licensed and actively practicing physician experts in the rare disease or condition for which such drug is approved or licensed and enter into the record of such meeting transcripts of such consultations.

''(iii) INCLUSION OF ADDITIONAL VOTING MEMBERS IN CERTAIN CIRCUMSTANCES.—

''(I) IN GENERAL.—In the case the review of a drug described in clause (i) by a DUR board or other entity, such board or entity shall include as voting members of such board or entity—

''(aa) at least 1 individual diagnosed with a rare disease or condition with respect to which such drug has a medically accepted indication to treat (or caregiver of such an individual); and

''(bb) at least 1 physician with expertise in such rare disease or condition.

''(II) SELECTION.—In selecting an individual described in subclause (I)(aa) or a physician described in subclause (I)(bb), the DUR board or other entity conducting the review of such drug shall—

''(aa) in the case the State offering the State plan (or waiver of such plan) with respect to which such review is being conducted has established a rare disease advisory council, so select such an individual and physician based on the recommendations of such council; and

''(bb) in the case the State offer the State plan (or waiver of such plan) with respect to which such is being conducted has not established such a council, so select such an individual and physician in consultation with national chapters of medical specialty societies described in clause (i).

''(iv) STAKEHOLDER TESTIMONY.—As part of a meeting described in subparagraph (A) with respect to which notifications are required to be made under clause (i), the DUR board or other entity conducting the review of the drug described in

such clause shall allow oral and written testimony from all attendees who are—

''(I) individuals diagnosed with the rare disease or condition for which such drug has a medically accepted indication if such individual—

''(aa) resides within the State offering the State plan (or waiver of such plan) with respect to which such review is being conducted; and

''(bb) has received medical assistance under any State plan (or waiver of such plan) during the 1-year period ending on the date of such meeting (or is receiving such assistance as of such date);

''(II) representatives (including patient research and advocacy organizations) or caregivers (or former caregivers) of an individual described in subclause (I);

''(III) licensed physicians—

''(aa) actively practicing within the State described in subclause (I)(aa); and

''(bb) possessing expertise and knowledge in the rare disease or condition for which such drug has a medically accepted indication; or

''(IV) the manufacturer of such drug.

''(v) MINIMUM BURDEN OF ILLNESS AND STANDARD OF CARE CONSIDERATIONS FOR ESTABLISHING COVERAGE POLICIES.—In the case the review of a drug described in clause (i) by a DUR board or other entity results in such drug being placed on or removed from a formulary used for purposes of this title (or results in a change in placement of such drug on such formulary) or results in application or modification of coverage criteria for such drug under a program described in paragraph (5), the board or entity shall consider, prior to developing or modifying

such formulary placement or coverage criteria—

''(I) the expert consultations described in clause (ii);

''(II) any stakeholder testimony described in clause (iv);

''(III) the most recently published peer-reviewed standard of care or treatment guidelines for the rare disease or condition for which such drug has a medically accepted indication;

''(IV) at least 1 published peer-reviewed medical article that analyzes data sets that have been generated within the 5-year period ending on the date of such application or modification for such drug and other drugs with a medically accepted indication for such rare disease or condition, if available;

''(V) real world data generated from—

''(aa) electronic health records;

"(bb) patient and drug product registries;

"(cc) patient wearable technologies;

"(dd) State and national claims data for such drug and other drugs with a medically accepted indication for such rare disease or condition during the 5-year period ending on the date of such application or modification (separated by diagnosis codes provided in the relevant fiscal year update of the 'International Classification of Diseases, 10th Revision, Clinical Modification' (or a successor publication)); and

"(ee) any other data determined to be relevant by the Secretary for such rare disease or condition.

"(vi) APPEALS.—

"(I) IN GENERAL.—An individual (or their agent), a prescriber, or the manufacturer of a drug shall have

standing to request a hearing before a Departmental Appeals Board administrative law judge at the United States Department of Health and Human Services to appeal any formulary change or application of coverage criteria to a drug described in clause (i) resulting from a meeting described in subparagraph (A) with respect to which notifications are required to be made under such clause.

''(II) PREVIOUSLY PUBLISHED COVERAGE POLICIES.—For formulary placements and prior authorization coverage criteria promulgated prior to enactment of this clause, any entity described in subclause (I) may file an appeal with such an administrative law judge alleging failure by the board or other entity in charge of such placement or development of such criteria to meet the requirements of clause (v) with respect to the development or modification of such placement or criteria.''.

(d) MODIFICATION OF THE DEFINITION OF MEDICALLY ACCEPTED INDICATION.—Section 1927(k)(6) of the Social Security Act (42 U.S.C. 1396r–8(k)(6)) is amended—

(1) by striking ''is supported by one or more'' and inserting the following: ''is supported by—

''(A) one or more'';

(2) by striking ''subsection (g)(1)(B)(i).'' and inserting ''subsection (g)(1)(B)(i); or''; and

(3) by adding at the end the following new subparagraph:

''(B) in the case such use is for the treatment of a rare disease or condition (as defined in section 526(a)(2) of the Federal Food, Drug, and Cosmetic Act)—

''(i) publication in a peer-reviewed journal;

''(ii) inclusion in the most recently developed consensus-based treatment guidelines for managing the disease or condition; or

''(iii) any other statement by a nationally recognized licensed and actively practicing physician expert in such rare disease or condition whose expertise is confirmed

by a medical specialty society involved in the treatment or management of such disease or condition.''.

(e) AUTHORITY TO DETERMINE CATEGORIES AND CLASSES OF DRUGS FOR RARE DISEASES.—Section 481(b) of the Public Health Service Act (42 U.S.C.287a–1(b)) is amended by adding at the end the following new paragraph:

''(3) AUTHORITY TO DETERMINE CATEGORIES AND CLASSES OF DRUGS FOR RARE DISEASES.—In order to promote continued investment in rare disease research and innovation, the Director shall—

''(A) develop and update a list that maps to each rare disease or condition for which the Food and Drug Administration has approved a drug or biological the national chapters of medical specialty societies and patient advocacy and research organizations with expertise in such disease or condition;

''(B) not later than 90 days following the enactment of this paragraph, hold a public coordination meeting featuring representatives from the Center for Drugs and the Center for Biologics at the Food and Drug Administration, the Centers for Medicare & Medicaid Services,

the Department of Defense, the Department of Veterans Affairs, patient advocacy and research organizations, medical societies, and manufacturers to develop a list of categories and classes for rare disease therapies that appropriately reflects the mechanism of action of the drug or biological, and the characteristics of the rare disease or condition; and

''(C) not later than 30 days following the public meeting described in subparagraph (B), publish the initial list of categories and classes of rare disease therapies, while regularly updating the list following new Food and Drug Administration approvals and providing annual updates with a notice and comment period.''.

(f) ENSURING ACCESS TO CERTAIN RARE DISEASE THERAPIES IN CHIP.—

(1) IN GENERAL.—Section 2103 of the Social Security Act (42 U.S.C. 1397cc) is amended—

(A) in subsection (a), by striking ''consistent with paragraphs (5), (6), (7), and (8)'' and inserting ''consistent with paragraphs (5), (6), (7), (8), and (13)''; and

(B) in subsection (c), by adding at the end the following new paragraph:

''(13) LIMITATIONS ON COVERAGE RESTRICTIONS FOR DRUGS PRESCRIBED FOR A RARE PEDIATRIC DISEASE OR CONDITION.—Notwithstanding any other provision of this section, a State child health plan may not, beginning on January 1, 2025, require a prerequisite drug, test (other than a test to confirm the diagnosis) or service, or place any other restriction relating to the use or prescribing of a covered outpatient drug approved under section 505(b) of the Federal Food, Drug, and Cosmetic Act or licensed under section 351(a) of the Public Health Service Act solely for one or more rare pediatric disease (as defined in section 529(a)(3) of the Federal Food, Drug, and Cosmetic Act and prescribed for a medically accepted indication (as defined in section 1927(k)(6)), unless such requirements or limitations are specified in the indication and usage section of the label of such drug.''.

(g) EFFECTIVE DATE.—The amendments made by subsection (c)(1) shall apply beginning January 1, 2025.