EPICrd Act: Line by Line Summary and Rationale

LEGISLATIVE TEXT	SUMMARY	RATIONALE
SECTION 1. SHORT TITLE. This Act may be cited as the "Ensuring Parity through Individualized Care for Rare Disorders (EPICrd) Act of 2022"		
SEC. 2. MEDICAID ACCESS TO COVERED OUTPATIENT DRUGS.		
(a) LIMITING THE SCOPE OF MEDICAID WAIVERS.—		
(1) IN GENERAL. *Section 1115(a)(1) of the Social Security Act (42 U.S.C. 1315(a)(1)) is amended by inserting "with the exception of 1902(a)(54) with respect to a covered outpatient drug (as defined in section 1927(k)(2)) in instances such project would deny, restrict, or otherwise limit access to such drug" after "1902,".*	This provision would expressly exclude restrictive prescription drug coverage policies from the scope of 1115 Medicaid demonstration waiver projects.	**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"). *See* SSA § 1115. Section 1902(a)(54) of the SSA provides a state with the option to offer a prescription drug benefit to its beneficiaries by requiring such state who chooses to do so to comply with section 1927 of the SSA, which typically requires payment for covered outpatient drugs if the manufacturer has a rebate agreement in place with the Centers of Medicare and Medicaid Services ("CMS") and certain criteria are satisfied. CMS, however, has failed to consistently interpret the interaction of these three sections of the SSA. After allowing a discriminatory waiver program in Oregon since the inception of the Medicaid Drug Rebate Program, CMS denied a request for closed formularies by Massachusetts, but approved a similar waiver for Tennessee. Former Rep. Henry Waxman's (D-CA) fear from 30 years ago is coming to fruition – more states are joining Oregon to abuse 1115 waivers by rationing care. For its existing waiver, Oregon Medicaid is requesting an extension and an expansion that would expressly exclude accelerated approval drugs and implement closed formularies like in Tennessee. 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. *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.

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(2) PROHIBITION AGAINST USING THE USP MEDICARE MODEL GUIDELINES FOR DESCRIBING RARE DISEASE THERAPY CATEGORIES AND CLASSES IN GOVERNMENT AND ACA PRESCRIPTION DRUG PROGRAMS. ***(A) PROHIBITION.*** *Chapters 6A and 7 of title 42 of the United States Code, chapter 55 of title 10 of the United States Code, chapter 17 of title 38 of the United States Code, and any implementing regulations shall not use or otherwise reference the United States Pharmacopeia Medicare Model Guidelines list to describe categories and classes of prescriptions drugs for the purpose of coverage and payment policies, including formularies, for drugs indicated for a rare disease or condition.* ***(B) ALTERNATIVE BENCHMARK FOR RARE DISEASE THERAPIES.*** *The Orphan Products Board shall develop and maintain a list of categories and classes of drugs indicated for a rare disease or condition based on therapeutic mechanism of action and disease characteristics, as provided in paragraphs (9) and (10) of section 227(c) of the Public Health Service Act (42 U.S.C. 236(c)), as added by section 2(e) of this Act.*	For drugs and biologicals indicated for a rare disease or condition, this provision 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 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 USP MMG. It would instead require the Orphan Products Board to develop and maintain an appropriate list of rare disease therapy categories and classes.	**The United States Pharmacopeia ("USP") Medicare Model Guidelines ("MMG") fail to provide an adequate drug coverage floor for patients affected with a life-threatening or debilitating, chronic rare diseases.** CMS has approved a section 1115 waiver that allows TennCare (Tennessee's Medicaid program) 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." This EHB rule, however, relies on the USP MMG categories and classes, which do not appropriately reflect differences among therapies or the rare disorders for which they are indicated. *See* 45 C.F.R. § 156.122. 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 v8.0 for benefit years 2020-2022 includes *44 branded drugs for 30 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. *See* USP MMG v8.0 at 27-28 (2019) (on file with the author) (emphasis added). As a result, Tennessee's 1115 waiver will harm patients. Specifically, Tennessee could comply with the requirement it offer a single drug from the "Genetic or Enzyme or Protein Disorder: Replacement, Modifiers, Treatment" category by *only* covering a generic drug approved for mastocytosis, *rather than any* of the FDA-approved therapies for alpha-1 antitrypsin deficiency, amyloidosis (hereditary transthyretin-mediated polyneuropathy), amyloidosis (hereditary transthyretin-mediated cardiomyopathy), amyloidosis (wild type cardiomyopathy), CLN2, cystinosis, Duchenne muscular dystrophy, Fabry disease, Gaucher disease, hereditary orotic aciduria, hypophosphatasia, MPS types I, II, IVA, VI, and VII, phenylketonuria, severe combined immunodeficiency associated with ADA deficiency, sickle cell disease, and urea cycle disorders, among others.

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(b) 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 sentence preceding subparagraph (A),*** *by striking "A State plan" and inserting "Subject to paragraph (8), a State plan";*	This provision would establish in the Medicaid prior authorization statute special consideration for medically necessary qualifying rare disease therapies.	**Conforming text.**
(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;";*	**State Prior Authorization Response**: This provision would specify that in addition to providing a response within 24-hours of the prior authorization request, the State must: • notify the prescriber, pharmacist, and the patient; and • make a minimum of three attempts to confirm the notified party received the response.	**Fleshing out the 24-hour notification requirement to ensure prior authorization decisions are appropriately communicated to all relevant parties will allow them to make informed decisions on next steps (i.e., appeals if necessary).** Section 1927(d)(5)(A) of the SSA requires a decision within 24 hours of the prior authorization request but does not specify to whom such decision is to be provided, nor the number of attempts the State must make. Requiring the State to make three attempts to inform the three most relevant stakeholders (the prescriber, pharmacist, and Medicaid beneficiary) of the prior authorization decision will ensure the decision is immediately communicated so expectations are managed and, if necessary, help the patient meet the deadline to file an appeal.
(C) by striking subparagraph (B) and inserting the following: *"(B) within one business day of the response described in subparagraph (A), if such response is a denial of a covered outpatient drug, provides by mail to the prescribing physician (or other individual authorized to prescribe under State law) and to the individual receiving medical assistance under this title a written response in English, Spanish, and three other languages most commonly spoken in the zip code of such individual (according to the most recent census information) using a template that the Secretary developed and published on the internet website of the Centers for Medicare & Medicaid Services, which details—* *"(i) the evidentiary basis for denial, including any published or unpublished coverage criteria for the covered outpatient drug; and* *"(ii) clear written instruction, including deadlines, for requesting an appeal hearing, as described in subparagraph (E);*	**Detailed Written Response for Denials:** In the event the initial response to the prior authorization request for a covered outpatient drug is a denial, this provision would require a state to provide the physician *and* patient with a subsequent written notification. The notification must be: • provided in English, Spanish, and three other most common spoken language in the zip code of the Medicaid beneficiary; and • based on a template developed by CMS and published on its website, which details— 1. evidentiary basis for the denial, including any coverage criteria relied upon, and 2. instructions for requesting an appeal with an Administrative Law Judge ("ALJ").	**A subsequent written notification of a prior authorization denial would fill an existing policy gap.** Prior authorization notification under the Medicaid Drug Rebate Program is currently limited to 24-hour requirement provided in section 1927(d)(5)(A) of the SSA, which is silent on details of what must be communicated in the event of a denial, including critical information regarding the basis of the denial and the appeals process. Proper communication of prior authorization decisions of covered outpatient drugs is vital to ensure due process for the Medicaid beneficiary. A timely written response in multiple languages detailing the basis of the denial and instructions for appeal crafted by using a CMS template will provide much needed transparency, which will not only help the beneficiary to avoid delays in appealing, but also assist in submitting a well-reasoned appeal.

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"(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 prescribing physician (or other individual authorized to prescribe under State law), controlled or improved the condition of the individual for whom it is being prescribed during the 180 days before the date of the request for approval of the drug under this paragraph;*	**Access for Stable Patients for Duration of Appeals Process:** This provision would modify the emergency supply policy to also 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.	**Expanding the emergency supply provision to include 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.** Section 1927(d)(5)(B) of the SSA requires a 72-hour supply of drug during the prior authorization process for an emergency. States are not obligated to supply this medicine for the duration of the appeals process. 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, especially for rapidly progressing disorders, where disruptions in a treatment regimen could have dire consequences. Such protection is especially necessary because the complete appeals process could take several months or more to conclude.
"(D) in the event of the development of or modification to coverage criteria for a covered outpatient drug by a DUR Board (described in subsection (g)(3)) or similar entity, including a Pharmacy and Therapeutics Committee, publishes and updates such criteria on its website within 45 days of such development or modification; and	**Publication of Coverage Criteria:** This provision would require publication on a State's website of coverage criteria that a DUR Board, P&T Committee, or similar entity has developed for any covered outpatient drug within 45 days of its development or modification.	**Requiring states to publish coverage criteria in instances it is generated or updated for a covered outpatient drug will establish a more equitable environment for Medicaid beneficiaries as they navigate the prior authorization process.** It is not uncommon for a State Medicaid to cite to an unpublished coverage policy it has generated in denying a Medicaid beneficiary access to therapy. This provision would prevent such inequities from continuing
"(E) provides, within 72 hours of an appeal request (such request shall be made within 30 days of the denial described in subparagraph (B)) from the prescribing physician (or other individual authorized to prescribe under State law) or the individual receiving medical assistance under this title, an initial appeal hearing before an Administrative Law Judge, which may be conducted by video conference or other form of telecommunication if requested by such prescriber or individual. Such Administrative Law Judge shall provide written adjudication to the prescriber and such individual within 48 hours of the hearing. Not later than 60 days following the date of the hearing adjudication, either party may file a petition to review the decision in the United States Court of Appeals for the District of Columbia Circuit or the circuit in which the parties are located."	**Initial PA Appeal Before an ALJ:** This provision 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 US Court of Appeals for the District of Columbia or a Federal Circuit in which the parties are located.	**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. *See* 42 C.F.R. 405.1000. Currently in Medicaid, while some states provide for ALJ hearings on first level appeals, most states do not. 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.

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(2) by adding at the end the following new paragraph: ***"(8) THERAPIES FOR CERTAIN RARE DISORDERS.*** ***"(A) IN GENERAL.*** *If the prescribing physician (or other individual authorized to prescribe under State law) provides submissions in accordance with subparagraph (E), a State shall—* *(i) determine that a qualifying rare disease therapy (as defined in subparagraph (D)) is medically necessary; and* *(ii) expeditiously grant approval for such therapy pursuant to paragraph (5) in accordance with subparagraph (B).*	**Medically Necessary Qualifying Rare Disease Therapies:** In the event a prescribing physician submits to the state Medicaid plan the diagnosis code, evidence supporting the diagnosis (a description of symptoms or a diagnostic test), and an attestation that the use or continued use of a "qualifying rare disease therapy" is reasonably likely to be effective for the individual patient, this provision would require the State to determine such drug is "medically necessary." In doing so, the States would be required to grant immediate authorization.	**Medical necessity should be determined by the prescriber, not the State Medicaid plan.** State Medicaid plans are permitted to use prior authorization as a "safeguard against unnecessary utilization [to ensure] that payments are consistent with efficiency, economy, and quality of care." *See* H.R. REP. NO. 101-881 at 98. Congress, however, *"[did] not intend that states establish or implement prior authorization controls that have the effect of preventing competent physicians from prescribing in accordance with their medical judgment."* *Id*. (emphasis added). Indeed, "[such a result] would defeat the intent of [creating the MDRP]." *Id*. Adhering to this congressional intent in its implementation, CMS has stated, "[it] would not permit a state to use prior authorization program to deny covered outpatient drugs when medical necessity is shown." *See* Medicaid Program; Payment for Covered Outpatient Drugs under Drug Rebate Agreements with Manufacturers, 60 Fed. Reg. 48442, 48455 (Sept. 19, 1995). Medical necessity, however, is not defined in statute or regulation, which has given states wide latitude in establishing discriminatory coverage policies. *See, e.g.*, Sarepta Therapeutics, Inc. v. Ark. Dep't of Human Servs., No. 60CV-18-8359 (Cir. Ct., Pulaski Co., Ark, 6th Div., Jan. 2, 2020) (holding that in claiming Exondys 51 was *not* medically necessary for Duchenne muscular dystrophy, Arkansas Medicaid illegally applied state and Federal laws that allow prior authorization for drugs to make a "threshold decision") *aff'd* Ark. Dep't of Human Servs. v. Sarepta Therapeutics, Inc., 2021 Ark. App. 330 at 5, 8 (concluding not only that Arkansas Medicaid "impermissibly substituted its judgment about the efficacy of the drug for that of FDA and the patient's prescribing physician," but also that it is "the FDA's job, not that of the Arkansas Medicaid agency, to evaluate the clinical data to determine whether a drug meets efficacy and safety standards."). For high value and transformative therapies that are satisfying unmet need or improving the standard of care for rare diseases, there should be a clear, streamlined pathway for the prescriber to establish medical necessity, and thus ensure payment in Medicaid.

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"(B) APPLICATION OF COVERAGE CRITERIA. *In the case of a medically necessary qualifying rare disease therapy, as defined in subparagraph (D), a prior authorization program described in paragraph (5) shall not require a prerequisite drug, test (other than a test to confirm the diagnosis), or service (such as emergency room intervention), or place any other restriction relating to the use or prescribing of such covered outpatient drug, unless such requirements or limitations are specified in the 'Indication and Usage' section of its label.*	**Prohibition Against Step Therapy and Other Coverage Restrictions for Medically Necessary Qualifying Rare Disease Therapies:** For a medically necessary qualifying rare disease therapy receiving such immediate authorization, this provision would expressly prohibit a step therapy protocol or any other restrictive coverage criteria, such as limiting the covered population to the clinical trial population, forced emergency room intervention, or unnecessary testing (other than confirmatory diagnostic tests), unless any restrictions or limitations are specified in the "Indications and Usage" section of the drug's label.	**A prohibition against restrictive coverage criteria in Medicaid would ensure patients can access their therapy as prescribed without delay.** Timely access to therapy is especially critical in rapidly progressing, fatal pediatric disorders, such as adrenoleukodystrophy, Batten disease, cystic fibrosis, Hunter syndrome, metachromatic leukodystrophy, Sanfilippo syndrome, and spinal muscular atrophy type 1, conditions that have a limited treatment window, such as achondroplasia, or diseases with life-threatening episodes, such as sickle cell disease and hereditary angioedema. The U.S. Department of Health and Human Services has previously stated that Medicaid coverage criteria designed to limit access to therapies for such chronic, disabling genetic disorders are "based in substantial part on the premise that the value of the life of a person with a disability is less than the value of the life of a person without a disability…which is *inconsistent with the [Americans with Disabilities Act].*" *See* Letter from Louis W. Sullivan, M.D., Sec'y, U.S. Dep't of Health & Hum. Servs. ("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) (emphasis added). This provision would expressly prevent Medicaid from denying the poor and disabled, including children with complex rare genetic disorders, medically necessary therapies and from making presumptions about their quality of life in justifying such action. Without congressional intervention, such rationing of care "[will continue and] could decimate the Medicaid program." *See Oregon Medicaid Rationing Experiment, Hearing Before the Subcomm. on Health & the Env't of the H. Comm. on Energy & Com.,* 102nd Cong. 94 (1991) (statement of Rep. Henry Waxman, Chairman, Subcomm. on Health & the Env't of the H. Comm. on Energy & Com.)
"(C) DURATION. *Authorization and reauthorization described in paragraph (5) of a covered outpatient drug described in clauses (i) and (ii) of subparagraph (D) shall be for a minimum of 12 months.*	**Authorization Period:** Provides for a minimum of 12-month authorization and reauthorization periods for rare pediatric indicated drugs, and drugs with orphan exclusive approval that were designated as breakthrough, RMAT, fast track, or priority review.	**Authorizations and reauthorizations for these rare disease therapies should be for a reasonable period to alleviate the administrative burden on physicians and ensure no disruptions to a patient's treatment regimen**.

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"(D) QUALIFYING RARE DISEASE THERAPY. *A qualifying rare disease therapy is a covered outpatient drug (as defined in subsection (k)(3)) that is prescribed for its approved or licensed use in a rare disease or condition (as defined in section 526(a)(2)(A) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bb(a)(2)(A)) and—*	**Definition of Qualifying Rare Disease Therapy:** This provision would define a qualifying rare disease therapy as a drug prescribed for its FDA approved rare disease indication **and**—	**Providing that the first condition to meet the definition of a "qualifying rare disease therapy is that it the therapy be prescribed for its FDA-approved indication limits the scope of the protected class.** By balancing state autonomy with the recognition of the high FDA approval standard for demonstrating safety and efficacy in a specific population, this initial qualifying condition underscores that Medicaid should not be permitted to undermine the FDA approval process with a restrictive coverage policy. Indeed, state Medicaid programs cannot justify denials of innovative medicines targeting conditions that FDA has determined to be safe and effective for its prescribed indication, which the prescriber determines to be medically necessary for the individual. Although some courts have made this point with respect to sweeping medical necessity thresholds in prior authorization coverage criteria and CMS has articulated this point with respect to accelerated approval, too much ambiguity remains, which requires legislation.
"(i) such approval under section 505(b) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(b)) or licensure under section 351(a) of the Public Health Service Act (42 U.S.C. 262(a)) is for a rare pediatric disease (as defined in section 529(a)(3) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360ff(a)(3));	Such indication is for a rare pediatric disease; **or**	**Protecting medicines indicated for rare pediatric diseases is consistent with longstanding congressional intent.** For example, in recent years, Congress has enacted multiple policies to create more drug development incentives (the Rare Pediatric Disease Priority Review Voucher program) and remove potential market barriers (special category of minimum Medicaid rebate percentage for drugs exclusively indicated for a rare pediatric disease). *See* 21 U.S.C. § 360ff; SSA § 1927(c)(1)(B)(iii)(II)(bb). Ensuring that a medicine prescribed for its FDA-approved rare pediatric indication can be accessed by children enrolled in Medicaid appropriately reflects this well-established policy priority.

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"(ii) such approval under section 505(b) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(b)) or licensure under section 351(a) of the Public Health Service Act (42 U.S.C. 262(a)) received exclusivity under section 527 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360cc) and such drug is further designated for such disease or condition— *"(I) as a breakthrough therapy under section 506(a) of such Act (21 U.S.C. 356(a));* *"(II) for fast track under section 506(b) of such Act (21 U.S.C. 356(b));* *"(III) as a regenerative medicine advanced therapy under section 506(g) of such Act (21 U.S.C. 356(g)); or* *"(IV) as a Food and Drug Administration priority application for being a significant improvement in the safety or effectiveness of the treatment of such disease or condition compared to available therapy; or*	Such indication received orphan exclusive approval and received breakthrough, fast track, RMAT, **or** priority review designation; **or**	**By linking the criteria for a "qualifying rare disease therapy" that is eligible for an automatic determination of medical necessity to existing Food and Drug Administration standards for measuring improved standard of care or the satisfaction of unmet need, the policy would ensure patient access to high value rare disease therapies.** More specifically, a drug approved with orphan exclusivity for the rare disease demonstrates that it is either the first brand featuring a particular molecule approved by FDA for the disease, or subsequent brand featuring the same molecule if it demonstrates it is safer, more effective, or provides a major contribution to patient care. *See* 21 U.S.C. § 360cc (codified at 21 C.F.R. § 316.34). By requiring an additional FDA designation under this provision, it would limit the streamlined medical necessity pathway to transformative orphan drugs: • For "breakthrough" designation, FDA has determined that preliminary evidence suggests that the drug would provide a "substantial improvement over available therapy" for a "serious or life-threatening disease or condition;" *See* 21 U.S.C. § 356(a). • For "fast track" designation, FDA has determined that the drug would satisfy "unmet medical need" for a "serious or life-threatening disease or condition;" *See* 21 U.S.C. § 356(b). • For "RMAT" designation, FDA has determined that the drug would satisfy "unmet medical need" in "treating, modifying, reversing, or curing" a "serious or life-threatening disease or condition;" *See* 21 U.S.C. § 356(g). • For "priority review" designation, FDA has determined that the drug would provide "significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention when compared to standard applications" for a "serious condition." *See* FDA, GUIDANCE FOR INDUSTRY: EXPEDITED PROGRAMS FOR SERIOUS CONDITIONS – DRUGS AND BIOLOGICS 24-25 (2014).

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"(iii) is a single course transformative therapy (as defined in subsection (k)(12)) sold under an outcomes-based agreement (as defined in subsection (k)(13)).	Such covered outpatient drug is a rare disease gene therapy sold under an outcomes-based agreement.	**Rare disease gene therapies sold under outcomes-based agreement should be considered qualifying rare disease therapies that are eligible for the streamlined medical necessity pathway because of the safeguard that Medicaid will only pay the full price if the individual patient achieves outcomes or measures pre-defined in the agreement.** In other words, payment for a drug sold under an outcomes-based agreement is based on its efficacy and durability in an individual patient. By holding the manufacturer "accountable" for the performance of the drug such agreements promote patient access by serving as an "alternative" to traditional utilization management tools employed by payers. Thus, including such therapies as part of this protected class eligible for a streamlined medical necessity determination is appropriate.

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"(E) PRESCRIBER SUBMISSIONS. *As provided in subparagraph (A), the prescribing physician (or other individual authorized to prescribe under State law) shall submit—* *"(i) the diagnosis code for the individual for whom the covered outpatient drug is prescribed;* *"(ii) evidence, including a diagnostic test result or a description of symptoms, supporting the diagnosis; and* *"(iii) in the case of a covered outpatient drug that is—* *"(I) described in clauses (i) and (ii) of subparagraph (D), an attestation that its use or continued use by the patient is necessary for an individualized course of treatment that is reasonably likely to:* *"(aa) prevent the onset of the disease or condition, or episodes, illnesses, injuries, or disabilities related to the disease or condition;* *"(bb) slow, halt, or reverse disease progression;* *"(cc) reduce or ameliorate the physical, cognitive, or psychosocial effects of the disease or condition; or* *"(dd) allow for the individual to achieve or maintain maximum functional capacity in performing daily activities; or* *"(II) described in subparagraph (D)(iii), an attestation that the administration of such therapy is reasonably likely to allow the patient to meet the pre-defined outcomes or measures specified in such agreement."*	**Streamlined Prior Authorization Submission:** In seeking to establish the medical necessity of a qualifying rare disease therapy, this provision requires the prescriber to provide the diagnosis code, supporting evidence of the diagnosis, and a simple attestation. • For a covered outpatient drug prescribed for its indicated rare pediatric disease, or for an indication that received orphan exclusive approval and designation as breakthrough, RMAT, fast track, or priority review, such attestation to demonstrate medical necessity must state that the prescriber believes the drug is reasonably likely to be effective for the patient for whom it is prescribed based on certain criteria. • For a rare disease gene therapy sold under an OBA and prescribed for its approved use, such attestation to demonstrate medical necessity must state that prescriber believes the drug is reasonably likely to allow the patient to meet the outcomes or measures specified in the OBA.	**Establishing a streamlined pathway for individualized determination of medical necessity by a prescriber for a covered outpatient drug based on a diagnosis and a simple attestation will ensure better outcomes through individualized patient care**. Under current law, states have complete autonomy with respect to prescriber submissions to support a medical necessity argument for a covered outpatient drug. For example, Apple Health (Washington state's Medicaid program) undermines physician judgement by rating the evidence submitted by prescribers under a prior authorization program, valuing certain evidence more than other evidence, while still requiring the least costly alternative when determining drug coverage. *See* WASH. ADMIN. CODE § 182-501-0165(6) (2022). In application, such policy sets an unreasonable evidentiary threshold that put an FDA-approved drug for Duchenne muscular dystrophy out of reach for several children. *See* Sarepta Therapeutics Inc. v. Wash. State Health Care Auth., 497 P.3d 454, 459-460 (Wash. Ct. App. 2021) (describing the rationale for EXONDYS 51 initially receiving a "D" rating from Apple Health). Such an outcome is not unique to Washington state as it is well-established throughout the country that states ignore physician judgment and deny patient access, even in cases where death is imminent. At a recent Senate hearing, several physicians and researchers agreed that prior authorization is used to "delay and ration care" that patients require, including evidence-based medicine. *See Mental Health and Substance Use Disorders: Responding to the Growing Crisis: Hearing Before the S. Comm. on Health, Education, Labor, and Pensions*, 117th Cong. (2022) (statements of Sen. Marshall, Member, S. Comm. on HELP, Michelle Dunham, MD, BU School of Medicine, Mitch Prinstein, PhD., CSO, APA, Sara Goldsby, MSW, Dir., S.C. Dep't of Alcohol & Drug Abuse Servs., Jennifer Lockman, PhD, CEO, Centerstone Research Inst.). They further agreed that a "streamlined" prior authorization submission process would improve patient outcomes and reduce health care provider burden. *Id*.

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"(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, it shall publish on the internet website of its State Department of Health a meeting agenda 60 days prior to such meeting.*	**Meeting Agenda:** This provision would require State DUR Boards and P&T Committees to publish an agenda 60 days prior to a public meeting.	**Timely publication of a meeting agenda will ensure appropriate stakeholder participation in a drug or therapeutic class review by a DUR Board, P&T Committee, or other entity.** Although these entities are tasked with determining coverage criteria to ensure medically necessary utilization of prescription drugs, there is no uniform standard for publishing notification or relevant details of public meetings. Requiring 60 days advance notice of an agenda is reasonable and allows for public discourse.
"(B) RARE DISEASE DRUG REVIEWS.— ***"(i) NOTIFICATION.*** *In the event the agenda for the meeting described in subparagraph (A) explicitly indicates an intention to review a covered outpatient drug that the Food and Drug Administration has approved under section 505(b) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(b)) or licensed under section 351(a) of the Public Health Service Act (42 U.S.C. 262(a)) for a rare disease or condition (as defined in section 526(a)(2)(A) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bb(a)(2)(A)) and its use in such disease or condition, such board or entity shall, within five business days of publishing such agenda, notify—* *"(I) national chapters of medical specialty societies and patient advocacy and research organizations with expertise in the disease or condition (selected from a list that the Orphan Products Board develops and updates in accordance with section 227(c)(8) of the Public Health Service Act (42 U.S.C. 236(c)(8))) for publication by the Centers for Medicare & Medicaid Services on its internet website; and* *"(II) the manufacturer of such drug.*	**Rare Disease Drug Review – Stakeholder Notification:** If the DUR Board or P&T Committee public meeting intends to review a drug approved for a rare disease or condition and its use in that disease or condition, this provision would require the board or committee to notify, within five days of publishing the agenda, the national chapters of the specialty societies and patient advocacy groups with expertise in the disease or condition (selected by a list generated by the Orphan Products Board and published on the CMS website) as well as the manufacturer of the drug.	**Because many rare genetic disorders are characterized by low prevalence, a heterogeneous clinical course in terms of age of onset, progression, and severity, and lack of FDA approved therapies, states should be required to provide timely direct notification to the affected patient and provider organizations, and the manufacturer of any drug or therapeutic class review by a DUR Board, P&T Committee, or other entity.** For example, Sanfilippo syndrome type A is a fatal and debilitating lysosomal storage disorder that is typically characterized by progressive central nervous system dysfunction, neurocognitive decline, and severe behavioral abnormalities before death in one's teens or early twenties. Several phenotypes exist, however, demonstrating "heterogeneity in the onset and rate of cognitive decline." Although there are several medicines in clinical development, there are currently no FDA-approved therapies for Sanfilippo syndrome type A, which affects approximately 700 children in the U.S. Once FDA approves a medicine, the complexities associated with the condition requires collaboration among experts when establishing coverage criteria. Timely notification of a drug or class review is critical to ensure relevant stakeholders have time to prepare and participate. Such participation will help ensure that the nuances of the epidemiology, diagnosis, clinical course, and standard of care for the 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.

LEGISLATIVE TEXT	SUMMARY	RATIONALE
"(ii) EXPERT CONSULTATION. *Not later than 10 days prior to the meeting described in this subparagraph, the entity conducting the review shall consult with a minimum of three nationally recognized licensed and actively practicing physician experts in the rare disease or condition, and enter into the meeting record transcripts of such consultations.*	**Rare Disease Drug Review – Clinician Key Opinion Leader Consultation:** Within 10 days of the public DUR Board or P&T Committee meeting, this provision would require the board or committee to consult with a minimum of three nationally recognized physician experts in the rare disease under review and submit transcripts of the consultations into the meeting record.	**Requiring DUR Board or P&T committee consultation would ensure all nuances of the rare disease for which the drug under review is indicated are properly considered when generating coverage criteria.** Without context provided by a medical expert, literature reviews and compendia are an insufficient substitute for the clinical experience with respect to the burden of illness and the most current standard of care for rare disorders. For example, it stands to reason that obtaining direct medical expert opinion when developing coverage criteria would be more efficient for the states than hearing their expert testimony during appeals hearings that are a result from coverage criteria that do not reflect the standard of care.
"(iii) STAKEHOLDER TESTIMONY. *As part of the public meeting described in this subparagraph, such board or entity shall allow oral and the submission of written testimony from all attendees who are –* *"(I) a patient diagnosed with the rare disease or condition for which the covered outpatient drug is under review if such individual—* *"(aa) resides within the State; and* *"(bb) has received medical assistance under this title within the previous 12 months or is currently receiving such assistance;* *"(II) a representative (including an individual from a patient research and advocacy organizations) or caregiver (or former caregiver) of a patient described in subclause (I);* *"(III) a licensed physician—* *"(aa) actively practicing within the State; and* *"(bb) possessing expertise and knowledge in the specific rare disease or condition for which such covered outpatient drug is approved or licensed; and* *"(IV) the manufacturer of such drug.*	**Rare Disease Drug Review – Stakeholder Testimony:** This provision would require the DUR Board or P&T Committee to receive live or written testimony from local Medicaid patients and their caregivers or representatives affected by the rare disease, local physicians with expertise in the disease, and the manufacturer of the drug.	**Much like requiring medical expert consultation, requiring a DUR Board or P&T committee to allow oral or the submission of written local stakeholder testimony when holding a public meeting to review a rare disease therapy will ensure the local standard of care for the rare disease and the local Medicaid beneficiary voice is presented.** Indeed, participation in the Medicaid drug review process by community-based organizations is vital for positively influencing coverage criteria. Establishing a uniform process for receiving testimony would help prevent an exacerbation of healthcare disparities for underserved and less known rare disease patient populations by ensuring stakeholders can engage decisionmakers.

LEGISLATIVE TEXT	SUMMARY	RATIONALE
"(iv) MINIMUM BURDEN OF ILLNESS AND STANDARD OF CARE CONSIDERATIONS FOR ESTABLISHING COVERAGE POLICIES. *In the event such review of a covered outpatient drug results in formulary placement described in paragraph (4) or prior authorization coverage criteria described in paragraph (5)(D), the board or entity shall consider prior to developing or modifying such formulary placement or prior authorization coverage criteria—* *"(I) the expert consultation described in clause (ii);* *"(II) the stakeholder testimony described in clause (iii);* *"(III) the most recently published peer-reviewed standard of care or treatment guidelines for the rare disease or condition;* *"(IV) a minimum of one published peer-reviewed medical article that analyzes data sets that have been generated within the five previous years for the covered outpatient drug and other drugs approved for the same 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 the covered outpatient drug and other drugs approved for the same rare disease or condition from the previous five calendar years 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 the rare disease or condition under review.*	**Rare Disease Drug Review – Minimum Evidence Threshold for Generating a Coverage Policy:** If the DUR Board or P&T Committee review of a rare disease therapy results in a formulary placement or prior authorization coverage criteria for the therapy, this provision would require the board or committee, prior to developing or modifying the formulary placement or prior authorization coverage criteria, to consider the medical expert consultation, the testimony of the manufacturer and the local patients, caregivers, and providers, the most recently published standard of care or treatment guidelines for the rare disease or condition, a peer-reviewed journal article years analyzing data generated within the previous five years for the entire class of drugs approved for the rare disease, and real world data.	**Medicaid utilization management, whether it is for formulary placement or prior authorization coverage criteria, should reflect not only the most recent treatment guidelines, but also disease burden as articulated by both treating clinicians with expertise and afflicted patients and caregivers or as demonstrated by appropriate claims data and other real-world evidence.** Under current law, there is no minimum standard of sources 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. ICER relies on quality adjusted life years ("QALYs") for measuring cost-effectiveness for rare disease therapies. The use of QALYs, however, is widely held to be inappropriate for assessing the value of rare disease therapies. *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). Stephanie Bozarth, who is a parent of a child with Morquio syndrome type A, has cautioned against coverage policies that "discriminate against children with disabilities." In a blog posted on *Disabled World* in July 2018, Bozarth criticized the recent trend of payers using QALYs in assessing whether a patient is worth the treatment costs, "When I watch my daughter walking rather than riding in a wheelchair and enjoying after-school activities like drama or coding club rather than being exhausted and isolated because of pain and exhaustion, the answer is very clear to me that she is worth it. She is living her best life because she has access to a treatment that is genuinely improving her quality of life." Without a statutory directive, states will ignore the patient perspective, continuing exclusive reliance on QALYs and other metrics that discriminate against those with chronic disabling conditions.

LEGISLATIVE TEXT	SUMMARY	RATIONALE
(v) APPEALS. ***(I) IN GENERAL.*** *Manufacturers, medical specialty societies, and patient advocacy and research organizations shall standing to file an appeal with an Administrative Law Judge regarding formulary placement and prior authorization coverage criteria resulting from the review described in this subparagraph.* ***(II) PREVIOUSLY PUBLISHED COVERAGE POLICIES.*** *For formulary placements and prior authorization coverage criteria promulgated prior to enactment of the Ensuring Patients with Rare Disorders Receive Individualized Care Act of 2022, manufacturers, medical specialty societies, and patient advocacy and research organizations may file an appeal with an Administrative Law Judge alleging failure by the board or other entity to meet the threshold described in clause (iv).*	**Rare Disease Drug Review – Stakeholder Appeals of Formulary Placement or Prior Authorization Coverage Criteria:** This provision provides standing for manufacturers, specialty societies, and patient organizations to appeal drug formulary placement and drug prior authorization coverage criteria with an ALJ. It would further allow such appeals to occur for previously published formularies and coverage criteria in the event the DUR Board or P&T Committee failed to satisfy the newly established minimum threshold for evidence.	**Providing standing to all relevant stakeholders for filing a formal appeal challenging any new or previous formulary placements or prior authorization coverage criteria will help ensure state Medicaid plans are complying with the newly established process for DUR Board and P&T Committee reviews of rare disease therapies.**

LEGISLATIVE TEXT	SUMMARY	RATIONALE
(c) DEFINITIONS.—Section 1927(k) of the Social Security Act (42 U.S.C. § 1396r-8(k)) shall be amended—		
(1) by striking paragraph (6), and inserting: ***"(6) MEDICALLY ACCEPTED INDICATION.—****The term "medically accepted indication" means any use for a covered outpatient drug which is—* *"(A) approved under section 505 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355), or licensed under section 351 of the Public Health Service Act (42 U.S.C. 262); or* *"(B) supported by—* *"(I) one or more citations included or approved for inclusion in any of the compendia described in subsection (g)(1)(B)(i); or* *"(II) with respect to a rare disease and condition (defined in section 526(a)(2) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bb(a)(2))), publication in a peer-reviewed journal within the previous five years."*	This provision updates the definition of "medically accepted indication" and expands it by also including off-label use of a drug for a rare disease or condition if such use is published in a peer-reviewed journal within the previous five years.	**Strengthening the ability of prescribers to rely on the most recent peer-reviewed medical literature when prescribing drugs for an off-label use is necessary because such literature may not be immediately reflected.** When offering a prescription drug benefit, a State must pay for a "covered outpatient drug," including physician-administered drugs, administered or dispensed for a use that FDA has approved or that is *"medically accepted," according to DRUGDEX or the American Hospital Formulary Service Drug Information*, if the manufacturer of such drug has executed a Medicaid National Drug Rebate Agreement with CMS and such drug is "medically necessary" for the beneficiary. *See* SSA § 1902(a)(54) (requiring a state that chooses to provide a prescription drug benefit to its Medicaid beneficiaries to comply with section 1927 of the SSA); SSA § 1927(a)(1) (requiring Medicaid payment for a "covered outpatient drug" if its manufacturer has "entered into" and has "in effect" a Medicaid Drug Rebate agreement); SSA § 1927(a)(7)(A) (codified at 42 C.F.R. § 447.520(a)(1)) (clarifying the scope of a "covered outpatient drug" includes physician-administered drugs); SSA § 1927(k)(3) (codified at 42 C.F.R. § 447.502) (limiting a "covered outpatient drug" to a drug administered or dispensed for its FDA-approved use or medically accepted off-label use); SSA § 1927(d)(1)(A) (permitting prior authorization for any "covered outpatient drug"); H.R. REP. NO. 101-881 at 98 (1990) (clarifying that, notwithstanding prior authorization programs, Congress intends the Medicaid Drug Rebate Program to ensure Medicaid beneficiary access to medically necessary prescription drugs) (emphasis added). Based on this requirement, CMS expressly prohibits prior authorization programs from denying payment for compendia-listed off-label uses of covered outpatient drugs. Unfortunately, these two compendia do not provide for a formal review process for adding off label uses for rare disease therapies, so by limiting it, there is no guarantee patients will have the benefit of the most recent peer-reviewed medical literature that supports off-label use of a medicine for a rare disease.

LEGISLATIVE TEXT	SUMMARY	RATIONALE
(2) by adding at the end the following paragraphs: ***"(12) Single Course Transformative Therapy.—****The term 'single course transformative therapy' means a treatment that consists of the administration of a covered outpatient drug that—* *"(A) is a form of gene therapy, as defined by the Commissioner of Food and Drugs, that is—* *"(i) designated under section 526 of the Federal Food, Drug, and Cosmetics Act (21 U.S.C. 360bb); and* *"(ii) licensed under subsection (a) or (k) of section 351 of the Public Health Service Act for a serious or life-threatening rare disease or condition;* *"(B) if administered in accordance with the "Indications and Usage" section of its label, is expected to result in—* *"(i) the cure of such disease or condition;* *"(ii) a reduction in the symptoms of such disease or condition to the extent that it is expected to—* *"(I) extend life expectancy for those individuals with such disease or condition;* *"(II) prevent, eliminate, or halt progression of comorbidities related to such disease or condition in such individuals; or* *"(III) allow such individuals to achieve or maintain maximum functional capacity in performing daily activities; or* *"(iii) prevention or elimination of episodes, illnesses, injuries, or disabilities related to such disease or condition; and* *"(C) is expected to achieve a result described in subparagraph (B), which may be achieved over an extended period of time, following a single prescribed course of treatment.*	This provision would define "single course transformative therapy" as a – • gene therapy approved for a serious or life-threatening rare disease, which • following a single prescribed course of treatment and if administered according to its label, is expected to over an extended period of time: 1. provide a cure 2. reduce symptoms to • extend life expectancy, • prevent, eliminate, or halt progression of related comorbidities; or • maximize daily function; or 3. prevent or eliminate episodes, illnesses, injuries, or disabilities related to the disease.	**Defining "single course transformative therapy" in such a manner balances the need to limit the scope of the types of cell and gene therapies that would be eligible for the streamlined medical necessity pathway if sold under an outcomes-based agreement, while ensuring the policy is inclusive of all possible disease modifying outcomes relevant to patients.** Beyond cures, significantly affecting the clinical course and prevention of episodes associated with the condition within a single treatment regimen are meaningful outcome measure that would provide high value to the patient and the health care system. These innovative medicines are designed to transform the life of a patient following a single prescribed course of treatment (often a single administration). For example, by transferring a functioning factor VIII gene to an individual with severe hemophilia A, a single infusion of gene therapy should result in that person expressing normal levels of factor VIII protein. Normal levels of factor VIII will ensure normal blood clotting, which would eliminate the need for regular infusions of maintenance therapy, which not only improves patient quality of life, but also is expected to *save* several millions of dollars per patient in lifetime treatment costs alone.
"(13) Outcomes-Based Agreement. *The term 'outcomes-based agreement' means a written contract between a manufacturer and purchaser in which the aggregate price over the course of the contract of the covered outpatient drug is based on the achievement of pre-defined outcomes or measures and adjusted accordingly."*	This provision would define an OBA as a written contract between a manufacturer and a payer in which payment is based on patient outcomes after using the medicine.	**Defining an "outcomes-based agreement" as a contract that conditions full payment for a prescription drug on its performance is consistent with the policy objective of manufacturer accountability.** Indeed, tying payment to "how well that drug performs in real-world patients" ensures the health care system is paying for value, which is critically important in managing costs of cell and gene therapies.

LEGISLATIVE TEXT	SUMMARY	RATIONALE
(d) CONFORMING AMENDMENT.—Section 227(c) of the Public Health Service Act (42 U.S.C. 236(c)) is amended—		
(1) in paragraph (6), by striking "and"; ***(2) in paragraph (7), by striking the period at the end and inserting "; and"; and*** ***(3) by adding at the end the following new paragraphs:*** *"(8) 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;* *"(9) hold a public coordination meeting to be held 90 days following the enactment of the Ensuring Patients with Rare Disorders Receive Individualized Care Act of 2022 featuring representatives from the Center for Drugs and the Center for Biologics at the Food and Drug Administration, the Centers for Medicare and 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* *"(10) publish the initial list of categories and classes of rare disease therapies 30 days following the public meeting described in paragraph (9), while regularly updating the list following new Food and Drug Administration approvals and providing annual updates with a notice and comment period."*	**Duties for Orphan Products Board:** This provision would require the Orphan Products Board, which sits in the Public Health Service and has been largely dormant for the previous 25 years, to develop and update list that maps the list of FDA-approved orphan drugs with provider and patient organizations that possess expertise in the rare disease. This conforming amendment would help give effect to the newly established rare disease drug review notification requirement. Additionally, this provision would require, within 90 days of enactment of the EPIC Act, the Orphan Products Board to convene a public meeting to develop an alternative to the USP MMG categories and classes for rare disease therapies. It would further require the Orphan Products Board to publish and regularly update, including providing for an annual notice and comment period.	**Because Congress established the Orphan Products Board to facilitate coordination among Federal agencies, patient organizations, and manufacturers (in addition to many other duties), this policy is an opportunity to revive this dormant entity.** Although the FDA Office of Orphan Product Development has assumed many of the duties of the Orphan Products Board, this mapping exercise and the needed coordination with CMS is outside of its mission. Reviving the Orphan Products Board to lead this initiative is appropriate and necessary. Additionally, the Orphan Products Board would further satisfy its mission as a facilitator among Federal agencies by convening the public meeting to develop and maintain the alternative to the USP MMG for rare disease therapy categories and classes.
(e) EFFECTIVE DATE..—		
The amendments made by this section shall apply to drugs prescribed on or after the date that is 90 days after the date of the enactment of this Act.	The provisions in section 2 (section 1115 waiver exclusion of drugs, improvements to prior authorization, streamlined medical necessity, procedures for DUR Board and P&T Committee drug review, and clarifying the existing Medicaid rebate for pediatric and hemophilia drugs) would go into effect within 90 days of enactment of the legislation.	**Providing three months for these policies to become effective will balance the immediate patient and provider need, with allowing CMS and state Medicaid plans reasonable time to comply.**

LEGISLATIVE TEXT	SUMMARY	RATIONALE
SEC. 3. ENSURING ACCESS TO CERTAIN RARE DISEASE THERAPIES IN CHIP		
(a) IN GENERAL.—Section 2103 of the Social Security Act (42 U.S.C. 1397cc) is amended—		
(1) in subsection (a), by striking ''consistent with paragraphs (5), (6), and (7)'' and inserting ''consistent with paragraphs (5), (6), (7), and (9)''; and ***(2) in subsection (c), by adding at the end the following new paragraph:*** ***''(9) 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 shall not require a prerequisite drug, test (other than a test to confirm the diagnosis) or service (such as emergency room intervention), 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 (21 U.S.C. 355(b)) or licensed under section 351(a) of the Public Health Service Act (42 U.S.C. 262(a)) solely for one or more rare pediatric disease (as defined in section 529(a)(3) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360ff(a)(3)) and prescribed for its approved or licensed use approved or licensed use, unless such requirements or limitations are specified in the 'Indication and Usage' section of its label."*	This provision would prohibit the Children's Health Insurance Program ("CHIP") from permitting step therapy and any other coverage restrictions (e.g., limiting coverage to the clinical trial population, forced ER intervention, or requiring unnecessary diagnostics) from being used to establish coverage criteria as part of prior authorization for drugs exclusively approved for one or more rare pediatric disease when prescribed for their FDA-approved use.	**Improving patient access to medicine in CHIP in a similar fashion as section 2 of this bill would provide in Medicaid will ensure parity between the two programs in protecting children with rare genetic disorders.** Two states operate CHIP as a separate program, eight states operate CHIP as a Medicaid expansion program, and 40 states operate CHIP as a combination of the two, according to the Medicaid and CHIP Payment and Access Commission. Thus, it is important to align these patient protections in both titles XIX and XXI of the SSA.
(b) EFFECTIVE DATE—		
The amendments made by this section shall apply to drugs prescribed on or after the date that is 90 days after the date of the enactment of this Act.	This provision that would limit the CHIP coverage restriction prohibition would go into effect 90 days after enactment of the bill.	**Providing three months following enactment for these policies to become effective will balance the immediate patient and provider need, with allowing state Medicaid plans reasonable time to comply in the same time frame as called for in section 2.**

LEGISLATIVE TEXT	SUMMARY	RATIONALE
SEC. 4. MEDICAID OUT-OF-STATE PROVIDER DEMONSTRATION PROJECT FOR ADMINISTRATION OF CERTAIN COVERED OUTPATIENT DRUGS AND CERTAIN DRUGS ADMINISTERED AS PART OF INPATIENT HOSPITAL SERVICES		
(a) IN GENERAL.—		
The Secretary of Health and Human Services (in this section referred to as the "Secretary") shall establish a 3-year project (in this section referred to as the "demonstration project") to test enrollment of and payment to out-of-state providers dispensing or administering certain covered outpatient drugs or administering certain drugs as part of inpatient hospital services in the Medicaid program under title XIX of the Social Security Act.	**Establishing an Out-of-State Provider Demo for Drug Administration:** With respect to an out-of-state provider dispensing or administering a rare disease therapy in either the inpatient or outpatient setting for a rare disorder to a child with medically complex conditions, this demonstration would establish a three-year demonstration project that would require a state to not only immediately recognize as a participating provider within the state the out-of-state provider that attests to possessing expertise in the disease or condition, but also pay no less than the drug and administration reimbursement rates that such provider would be entitled to under its state plan.	**By establishing this demonstration project, CMS can test the impact streamlined provider enrollment and adequate reimbursement will have on access by out-of-state Medicaid beneficiaries who require physician administration of therapy.** Currently, heath care providers who specialize in treating rare genetic disorders are discouraged from administering therapies to Medicaid beneficiaries who travel from a different state because of burdensome provider enrollment processes and potentially lower reimbursement rates. Section 1902(a)(16) of the SSA requires a state to pay providers in another state for items and services rendered to an enrollee temporarily absent from that state. Of most relevance to medically complex children and others with rare, genetic conditions, the implementing regulations specify that this reimbursement must occur if the state "determines that the needed medical services…are more readily available in the other state," among other conditions. *See* 42 C.F.R. § 431.52. For reimbursement purposes, section 1902(kk)(7)(A) of the SSA requires physicians to be enrolled as a participating provider, so an out-of-state provider is required to enroll as a participating provider in the Medicaid program in the state of the beneficiary served in order to receive payment. Such out-of-state providers are subject to the payment rates of the state where the Medicaid enrollee resides. Because of the complexity of the condition and low prevalence, patients diagnosed with rare, genetic disorders, such as Batten disease, typically must travel out of state to receive their medical care, often at a disease-specific center of excellence. *See, e.g.*, Batten Disease Support & Research Ass'n, Centers of Excellence, (listing Massachusetts General Hospital, Nationwide Children's Hospital, Texas Children's Hospital, and University of Rochester Medical Center as those having a Batten Disease Center of Excellence in the U.S.) (last visited Feb. 4, 2022). Provider burden and reimbursement challenge are an impediment to patient access that must be resolved.

LEGISLATIVE TEXT	SUMMARY	RATIONALE
(b) DURATION AND SCOPE.—		
The demonstration project conducted under this section shall operate during a period of fiscal years 2023 through 2025 and be limited to medical assistance (as defined in section 1905(a) of the Social Security Act (42 U.S.C. 1396d(a))) for eligible drugs administered or dispensed to eligible individuals by an out-of-state provider enrolled as a participating provider in the State of the beneficiary pursuant to subsection (c).	This subsection would specify the demonstration project will last between FY 2023 and FY 2025.	**A three-year demonstration should be an adequate period to test the success of streamlined provider enrollment and adequate reimbursement.**
(c) PROVIDER ENROLLMENT.—		
A State shall immediately recognize an out-of-state provider as a participating provider described in section 1902(kk)(7)(A) of the Social Security Act (42 U.S.C. 1396a(kk)(7)(A)) if, through a web-based portal developed by the Secretary for this demonstration project, such out-of-state provider— *(1) attests to possessing expertise in the disease or condition for which the eligible drug is being dispensed or administered; and* *(2) provides submissions in accordance with section 1927(d)(8)(E) of the Social Security Act, as added by section 2(b)(2) of this Act.*	**National Medicaid Enrollment Portal:** This provision would require CMS to manage a national enrollment website portal for providers from which states would be required to immediately recognize an out of state provider as a participating provider within the state if the provider attests to possessing expertise in the disease or condition for which the qualifying rare disease therapy is provided and submits details on the diagnosis code and an attestation that it would reasonably likely to be effective in the individual	**Streamlined enrollment as an out-of-state Medicaid provider would alleviate significant provider burden and improve patient outcomes.** Notwithstanding CMS guidance that encourages automatic reciprocity by allowing state Medicaid plans to "rely on the results of screening performed by Medicare contractors, other State Medicaid agencies or other CHIP programs," most states require their own, often duplicative or more burdensome process. For example, physicians at Boston Children's Hospital have provided several examples of absurd enrollment processes required by other states to treat their Medicaid patients, which has had a direct negative impact on patient care. A national portal with a streamlined enrollment process will remove a growing barrier to Medicaid patient access.

LEGISLATIVE TEXT	SUMMARY	RATIONALE
(d) PAYMENT.—		
(1) PAYMENT TO AN OUT-OF-STATE PROVIDER ENROLLED AS A PARTICIPATING PROVIDER.—*Payment under a State plan to an out-of-state provider enrolled as a participating provider in such State pursuant to subsection (c) for an eligible drug and costs associated with dispensing or administering such drug shall be no less than the payment rates for such drug and associated costs under the State plan of such provider's State during the quarter in which the drug was dispensed or administered.*	For the purpose of this demonstration project, this provision would require the state Medicaid plan to pay no less than the payment rates for the drug and administration costs than the rates in the provider's state.	**Paying no less than the drug and administration reimbursement rates that the out-of-state Medicaid provider would be entitled to under its state plan will remove the second barrier to administering therapy to Medicaid beneficiaries from other states.** Lower payment rates force out-of-state providers to bill the Medicaid beneficiary or absorb the costs as uncompensated care, which is unsustainable in the long run, especially as more therapeutic interventions are approved for conditions that currently lack an FDA-approved therapy. For example, once the FDA approves a gene therapy for recessive dystrophic epidermolysis bullosa, it may initially only be available for administration at hospitals with expertise in the condition, such as Cincinnati Children's Hospital, University of Minnesota Masonic Children's Hospital, Children's Hospital of Philadelphia, and Lucile Packard Children's Hospital Stanford. If a child on Arizona Medicaid receives the gene therapy at Stanford, Arizona may pay less for the medicine and its administration than MediCal, which would cause Stanford to absorb the cost differential as uncompensated care.
(2) ENHANCED FEDERAL MEDICAL ASSISTANCE PERCENTAGE.—*The Federal medical assistance percentage (as defined in section 1905(b) of the Social Security Act (42 U.S.C. 1396d(b))) applicable to the payments described in paragraph (1) shall be increased by 20 percentage points, but in no case shall exceed 90 percent.*	For the purpose of this demonstration project, this provision would mitigate any burden on a state Medicaid plan required to pay higher reimbursement rates for the drug and its administration by providing a higher federal match, capped at 90 percent.	**Providing an enhanced FMAP for drug and administration costs under this demonstration project will protect states who would be required to pay more than their current rates for such items and services.**

LEGISLATIVE TEXT	SUMMARY	RATIONALE
(e) DEFINITIONS.—		
(1) ELIGIBLE DRUG.–*The term "eligible drug" means—* *(A) a drug that is—* *(i) a qualifying rare disease therapy as described in section 1927(d)(8)(D) of the Social Security Act (42 U.S.C. 1396r-8(d)(8)(D)); or* *(ii) administered as part of inpatient hospital services but would otherwise meet such definition of a qualifying rare disease therapy; and* *(B) such drug is more readily available in a State other than the State of the individual receiving medical assistance under title XIX due to its complexity or the rarity or complexity of the disease or condition for which it is prescribed; or* *(C) although such drug is available in the State of the individual receiving medical assistance under title XIX—* *(i) the distance for the eligible individual to travel for the administration of such drug within such State—* *(I) is greater than 50 miles from the primary residence of such individual; and* *(II) exceeds the distance to travel from such residence for the administration of such drug in a neighboring State; or* *(ii) the site of service administering such drug within the state is not recognized as a center of excellence for the disease or condition for which the drug is indicated by the patient advocacy and research organization representing such disease or condition, but the out-of-state site of service is recognized as such a center.*	This provision specifies that drugs eligible for inclusion in the demonstration project are "qualifying rare disease therapies" (including those that are inpatient administered) in certain circumstances. Such drug would either be required to be • more readily available in a different State due to its complexity or the complexity of the rare disease or condition; **or** • if available in the state of the individual, the out-of-state provider must be— ○ a center of excellence; **or** ○ a shorter distance if the in-state provider is more than 50 miles from the primary residence of the Medicaid beneficiary.	**This project not only would help ensure access for certain Medicaid beneficiaries to disease specific centers of excellence and experts in the rare disease, but also give such patients some additional flexibilities in receiving treatment from boarder providers who are experts in closer proximity.**
(2) ELIGIBLE INDIVIDUAL.–*The term "eligible individual" means an individual who is—* *(A) a "child with medically complex conditions" (as defined in section 1945A(i)(1) of the Social Security Act (42 U.S.C. 1396w-4a(i)(1)); or* *(B) a "qualified severely impaired individual" (as defined in section 1905(q) of the Social Security Act (42 U.S.C. 1396d(q))).*	This provision specifies that Medicaid beneficiaries eligible for the demonstration project are— • children with medically complex conditions; or • qualified severely impaired individuals.	**By limiting the scope of the project to Medicaid beneficiaries under 21 years of age diagnosed with one or more multi-system chronic conditions that reduces cognition or physical function, a life-limiting illness, or a rare pediatric condition and other blind or disabled Medicaid beneficiaries receiving supplemental security income, the project would capture the Medicaid beneficiaries most likely to require to out-of-state providers for therapeutic intervention.**

LEGISLATIVE TEXT	SUMMARY	RATIONALE
(f) REPORT.—		
Within 180 days of the conclusion of the demonstration project, the Secretary shall submit to Congress a report– *(1) evaluating the impact of the demonstration project on eligible individuals accessing eligible drugs from out-of-state providers in a timely fashion;* *(2) assessing whether the demonstration project reduced the burden for out-of-state providers enrolling as a participating provider and eliminated any barriers for out-of-state providers administering or dispensing eligible drugs to eligible individuals;* *(3) analyzing the financial impact the enhanced FMAP had on States paying the out-of-state provider's rates;* *(4) providing statistics on—* *(A) medical specialists participating in the demonstration project;* *(B) the qualification of eligible individuals participating in the demonstration project;* *(C) eligible drugs dispensed or administered in the demonstration project, including diagnosis codes associated with such drugs; and* *(D) the amount of State and Federal reimbursement for such drugs; and* *(5) recommending whether Congress should continue the demonstration project indefinitely.*	This provision requires a report to Congress within six months of the conclusion of the demonstration project. The report must evaluate patient access to care, reduction in provider burden, and costs to State and Federal governments, as well as recommend whether to continue the program permanently.	**Such a report to Congress should capture relevant data to provide the evidence necessary to support continuing the program for sick and disabled Medicaid beneficiaries who require out-of-state providers.**

LEGISLATIVE TEXT	SUMMARY	RATIONALE
SEC. 5. MEDICAID PAYMENT FOR HOME INFUSION, DME, MEDICAL SUPPLIES, MEDICAL FOODS, AND TELEHEALTH FOR CERTAIN INDIVIDUALS WITH SPECIAL HEALTH CARE NEEDS		
(a) IN GENERAL. Section 1902 of the Social Security Act (42 U.S.C. 1396a) shall be amended as follows:		
(1) in subsection (a)— ***(A) in paragraph (86), by striking "and";*** ***(B) in paragraph (87), by striking the period at the end and inserting ";"; and*** ***(C) by adding at the end the following:*** *"(88) provide, in accordance with (tt), payment for medically necessary home infusion therapy for certain eligible individuals;* *"(89) provide, in accordance with (uu), payment for medically necessary durable medical equipment for certain eligible individuals;* *"(90) provide, in accordance with (vv), payment for medically necessary medical supplies for certain children;* *"(91) provide, in accordance with subsection (ww), for payment for medically necessary food and the medical equipment and supplies necessary to administer such food;" and* *"(92) provide, in accordance with subsection (xx), for payment for telehealth services provided to certain eligible individuals;";*	**Mandatory Payment of Home Infusion Therapy, DME, Medical Supplies, and Medical Food:** This provision would require state Medicaid payment of: • medically necessary home infusion therapy for eligible individuals; • medically necessary durable medical equipment for eligible individuals; • medically necessary medical supplies for eligible individuals; • medically necessary medical food for eligible individuals; and • telehealth services for eligible individuals.	**Medicaid benefits for home infusion therapy, durable medical equipment, medical supplies, medical foods, and telehealth should be mandatory for beneficiaries with debilitating and life-threatening chronic conditions.**

LEGISLATIVE TEXT	SUMMARY	RATIONALE
(2) by adding at the end the following: ***"(tt) MEDICALLY NECESSARY HOME INFUSION THERAPY FOR CERTAIN ELIGIBLE INDIVIDUALS.—*** ***"(1) IN GENERAL.—****In order to meet the requirements of subsection (a)(88), the State plan shall provide payment for home infusion therapy (as defined in paragraph (2)) that a prescribing physician (or other individual authorized to prescribe under State law) attests is medically necessary for the eligible individual (as defined in paragraph (3)) receiving medical assistance under this title.* ***"(2) HOME INFUSION THERAPY.—****For the purpose of this subsection, the term 'home infusion therapy' means—* *"(A) the covered outpatient drug (as defined in section 1927(k)(2) (42 U.S.C. § 1396r-8(k)(2)));* *"(B) supplies and equipment required for infusion (including infusion pump, intravenous pole, intravenous tubing, intravenous catheters, syringes, needles, and intravenous start and dressing change kits); and* *"(C) services, including drug administration and patient education, training, and monitoring, provided by a trained infusion nurse.* ***"(3) ELIGIBLE INDIVIDUAL.–****For the purpose of this subsection, the term "eligible individual" means an individual who is—* *"(A) a 'child with medically complex conditions' (as defined in section 1945A(i)(1) (42 U.S.C. 1396w-4a(i)(1)));* *"(B) a 'qualified severely impaired individual' (as defined in section 1905(q) (42 U.S.C. 1396d(q))); or* *"(C) eligible for 'home and community-based services' under section 1915(i) (42 U.S.C. 1396n(i)).*	This provision would require Medicaid payment for home infusion therapy for eligible individuals, deeming it medically necessary following an attestation to its necessity by the prescriber. Home infusion therapy expressly comprises not only the covered outpatient drug, but also infusion pump, intravenous pole, intravenous tubing, intravenous catheters, syringes, needles, and intravenous start and dressing change kits, as well as services provided by a trained infusion nurse, including drug administration and patient education, training, and monitoring. Medicaid beneficiaries eligible for this benefit are: • children with a medically complex conditions; • qualified severely impaired individuals; or • individuals who qualify for HCBS.	**Requiring payment without delay for not only the drug, but also the required supplies, equipment, and necessary nursing services will ensure home infusion therapy is a viable option for Medicaid patients severe, disabling disorders**. Medicaid coverage of home infusion therapy is inconsistent throughout the country. Delays due to prior authorization coverage criteria, limitations on nursing services, and restrictions on certain supplies and equipment are access barriers that negatively affect patient outcomes. A uniform standard for home infusion therapy that establishes a minimum coverage standard for items and services will guarantee Medicaid beneficiaries the ability to use this preferred and more cost-effective site of service.

LEGISLATIVE TEXT	SUMMARY	RATIONALE
"(uu) MEDICALLY NECESSARY DURABLE MEDICAL EQUIPMENT FOR CERTAIN ELIGIBLE INDIVIDUALS.— ***"(1) IN GENERAL.—****In order to meet the requirements of subsection (a)(89), the State plan shall provide payment for durable medical equipment (as defined in section 1861(n) (42 U.S.C. 1395x(n)) and including any other adaptive equipment and medical furniture not otherwise described) that a prescribing physician (or other individual authorized to prescribe under State law) attests is medically necessary for an eligible individual (as defined in paragraph (2)) receiving medical assistance under this title.* ***"(2) ELIGIBLE INDIVIDUAL.–****For the purpose of this subsection, the term "eligible individual" means an individual who is—* *"(A) a 'child with medically complex conditions' (as defined in section 1945A(i)(1) (42 U.S.C. 1396w-4a(i)(1)));* *"(B) a 'qualified severely impaired individual' (as defined in section 1905(q) (42 U.S.C. 1396d(q))); or* *"(C) eligible for 'home and community-based services' under section 1915(i) (42 U.S.C. 1396n(i)).*	This provision would require Medicaid payment for DME for children with medically complex conditions, a qualified severely impaired individuals, and individuals eligible for HCBS deeming such DME as medically necessary following at attestation to its necessity by the prescriber. With respect to defining DME, it would • rely on the definition found in title XVIII, which "includes iron lungs, oxygen tents, hospital beds, and wheelchairs (which may include a power-operated vehicle that may be appropriately used as a wheelchair, but only where the use of such a vehicle is determined to be necessary on the basis of the individual's medical and physical condition and the vehicle meets such safety requirements as the Secretary may prescribe) used in the patient's home (including an institution used as his home [ ]), whether furnished on a rental basis or purchased, ….and eye tracking and gaze interaction accessories for speech generating devices furnished to individuals with a demonstrated medical need for such accessories;" and • expand the title XVIII definition to also include "any other adaptive equipment or medical furniture not otherwise described" in that definition.	**Restricting access to DME, including adaptive equipment, for individuals with disabilities is discriminatory, making independent living impossible.** Because coverage of DME is an optional Medicaid benefit, some states are denying and delaying patient access. For example, Connecticut Medicaid is denying severely disabled individuals with spinal muscular atrophy and Duchenne muscular dystrophy access to adaptive equipment in the form of a robotic arm "to assist with basic activities of daily living, such as eating and meal preparation, and basic health and safety-related activities, such as answering a phone, opening a door, or adjusting paralyzed legs." This provision would ensure disabled Medicaid beneficiaries can access wheelchairs, robotic arms, and other medically necessary DME without delay.

LEGISLATIVE TEXT	SUMMARY	RATIONALE
"(vv) MEDICALLY NECESSARY MEDICAL SUPPLIES FOR CERTAIN ELIGIBLE INDIVIDUALS.— ***"(1) IN GENERAL.—****In order to meet the requirements of subsection (a)(90), the State plan shall provide payment for medical supplies (as defined in paragraph (2)) that a prescribing physician (or other individual authorized to prescribe under State law) attests is medically necessary for an eligible individual (as defined in paragraph (3)) receiving medical assistance under this title for wound care and pain management.* ***"(2) MEDICAL SUPPLIES.—****For the purpose of this subsection, medical supplies include over-the-counter antihistamine, acetaminophen, nonsteroidal anti-inflammatory drugs, antiseptic, zinc oxide, and antibiotic ointment, as well as bandages, gauze, and dressings.* ***"(3) ELIGIBLE INDIVIDUAL.–****For the purpose of this subsection, the term "eligible individual" means an individual who is—* *"(A) a 'child with medically complex conditions' (as defined in section 1945A(i)(1) (42 U.S.C. 1396w-4a(i)(1)));* *"(B) a 'qualified severely impaired individual' (as defined in section 1905(q) (42 U.S.C. 1396d(q))); or* *"(C) eligible for 'home and community-based services' under section 1915(i) (42 U.S.C. 1396n(i)).*	This provision would require Medicaid payment for certain medical supplies for wound care and pain management for children with medically complex conditions, deeming such supplies as medically necessary following at attestation to its necessity by the prescriber. Medical supplies included in this coverage requirement include OTC antihistamine, acetaminophen, NSAIDs, antiseptics, zinc oxide, and antibiotic ointment, as well as bandages, gauze, and dressings.	**Requiring Medicaid payment for wound care and pain management for children with medically complex conditions like epidermolysis bullosa ("EB") is critical.** Only six of 50 states have Medicaid programs that cover the bandages that people with EB desperately need. Paying for them out-of-pocket is unaffordable. EB is a rare, debilitating, and potentially fatal connective tissue disorder that manifests through severe blisters on the skin, eyes, throat, and internal organs. Friction from wearing shoes, brushing teeth and scratching an itch can cause large, painful blisters or erosions that are prone to life threatening infections from bacteria like MRSA. Children with EB are often called "butterfly children"' because their skin so fragile. "Typically, even a mild localized trauma can cause skin to fall off, leading to open sores that do not heal, which cause indescribable pain and agony." The lack of Medicaid coverage of bandages for the EB community recently caused Brett Kopelan and his family to "uproot their entire lives" to move to Colorado because New York Medicaid refused to pay. The Kopelans fortunately had "the resources and the means to move to Colorado, where Medicaid will cover [Rafi's] supplies. But many others are not so fortunate." Even more importantly, people, especially those with EB, should not be required to abandon family, friends, and support systems to relocate for the sole purpose of better Medicaid coverage.

LEGISLATIVE TEXT	SUMMARY	RATIONALE
"(ww) MEDICALLY NECESSARY FOOD.— ***"(1) IN GENERAL.—****In order to meet the requirements of subsection (a)(91), the State plan shall provide payment for medically necessary food (and the medical equipment and supplies necessary to administer such food), including a low protein modified food product or an amino acid preparation product, that is—* *"(A) furnished pursuant to the prescription, order, or recommendation (as applicable) of a prescribing physician (or other individual authorized to prescribe under State law) for the dietary management of a covered disease or condition;* *"(B) a specially formulated and processed product (as opposed to a naturally occurring foodstuff used in its natural state) for the partial or exclusive feeding of an individual by means of oral intake or enteral feeding by tube;* *"(C) intended for the dietary management of an individual who, because of therapeutic or chronic medical needs, has limited or impaired capacity to ingest, digest, absorb, or metabolize ordinary foodstuffs or certain nutrients, or who has other special medically determined nutrient requirements, the dietary management of which cannot be achieved by the modification of the normal diet alone;* *"(D) intended to be used under medical supervision, which may include in a home setting; and* *"(E) intended only for an individual receiving active and ongoing medical supervision under which the individual requires medical care on a recurring basis for, among other things, instructions on the use of the food.*	This provision would require Medicaid payment for prescribed food medically necessary for the safe and effective dietary management of a Medicaid beneficiary diagnosed with certain digestive, absorption, and inherited metabolic disorders and conditions.	**Requiring Medicaid payment for medically necessary foods will ensure those individuals with medical conditions requiring a special diet can avoid adverse health consequences.** For example, the American College of Medical Genetics and Genomics Practice Guidelines for phenylketonuria ("PKU") state that strict adherence to a phenylalanine-restricted diet, which precludes eating protein such as beef, pork, poultry, fish, eggs, and cheese replacing those foods with low protein medical food and medical formula, is necessary for the preservation of optimal intellect and psychological health in PKU patients. Without access to medical foods for dietary management, PKU patients are at risk for toxic levels of phenylalanine, which can cause severe intellectual disability and other neurological problems, such as executive function impairment, memory loss, anxiety, depression, and phobias. In addition to PKU, rare disorders that rely on medical foods as part of their treatment regimen include: • cystic fibrosis; • eosinophilic esophagitis; • food protein induced enterocolitis syndrome; and • short bowel syndrome.

LEGISLATIVE TEXT	SUMMARY	RATIONALE
"(2) LIMITATION.—*For the purpose of this subsection, medically necessary food shall not include food—* *"(A) taken as part of an overall diet designed to reduce the risk of a disease or medical condition or as weight-loss products, even if the food is recommended by a physician or other health care professional;* *"(B) marketed as gluten-free for the management of celiac disease or non-celiac gluten sensitivity; or* *"(C) marketed for the management of diabetes.*	This provision would expressly exclude weight loss food and supplements, gluten-free food items, and food intended for diabetes.	**Expressly excluding certain foods will help ensure state Medicaid plans can safeguard against unnecessary utilization.**
"(3) COVERED DISEASE OR CONDITION.—*In this subsection, the term 'covered disease or condition' means—* *"(A) inborn errors of metabolism;* *"(B) medical conditions of malabsorption;* *"(C) pathologies of the alimentary tract or the gastrointestinal tract;* *"(D) a neurological or physiological condition; and* *"(E) such other diseases or conditions the Secretary determines appropriate.*	This provision limits the types of conditions for which medical foods is appropriate to inborn errors of metabolism, malabsorption disorders, gastrointestinal disorders affecting the alimentary tract, and neurological or physiological conditions.	**Expressly limiting the conditions eligible for mandatory Medicaid payment of medical foods will help ensure state Medicaid plans can safeguard against unnecessary utilization.**

LEGISLATIVE TEXT	SUMMARY	RATIONALE
"(xx) TELEHEALTH FOR CERTAIN ELIGIBLE INDIVIDUALS. ***"(1) IN GENERAL.—****In order to meet the requirements of subsection (a)(92), the State plan shall provide payment for all telehealth services (and an originating site facility fee) provided by an eligible provider (as defined in paragraph (3)) to an eligible individual (as defined in paragraph (2)) receiving medical assistance under this title and who accesses such telehealth services from any location.*	This provision would require Medicaid payment for telehealth services (and an originating site fee) provided to certain Medicaid beneficiaries accessing such services at any location.	**Establishing a telehealth benefit for certain beneficiaries regardless of geography and without restrictions on sites of service will improve access to care and outcomes.** According to CMS, "states are encouraged to facilitate clinically appropriate care within the Medicaid program using telehealth technology to deliver services covered by the state." CMS further explains that states that choose to offer the telehealth benefit, however, have full autonomy regarding the "types of services to cover; where in the state it can be utilized; how it is implemented; what types of practitioners or providers may deliver services via telehealth, as long as such practitioners or providers are 'recognized' and qualified according to Medicaid federal and state statute and regulation; and reimbursement rates." For example, Apple Health (Washington Medicaid) reimburses for telemedicine visits, but the patient must be live at an originating site and participating during encounters. Because this provision would allow the patient to remain at home while receiving telehealth services, it would overcome restrictive state laws, supporting the objectives of the rare disease patient community, especially during the recent COVID-19 pandemic.

LEGISLATIVE TEXT	SUMMARY	RATIONALE
"(2) ELIGIBLE INDIVIDUAL. *For the purpose of this subsection, an eligible individual is an individual who is—* *"(A) a "child with medically complex conditions" (as defined in section 1945A(i)(1) of the Social Security Act (42 U.S.C. 1396w-4a(i)(1)));* *"(B) a "qualified severely impaired individual" (as defined in section 1905(q) of the Act (42 U.S.C. 1396d(q)));* *"(C) eligible for "home and community-based services" under section 1915(i) of the Act; or* *"(D) otherwise eligible for medical assistance under this title and diagnosed with a rare metabolic disorder."*	This provision would specify that Medicaid beneficiaries eligible for this mandatory benefit is limited to: • children with medially complex conditions; • qualified severely impaired individuals; • eligible for HCBS; and • those diagnosed with rare metabolic disorders. Rare metabolic disorders would include those with PKU, AADC deficiency, maple syrup urine disease, propionic acidemia, long-chain fatty acid oxidation disorders, primary carnitine deficiency, Zellweger syndrome, X-linked adrenoleukodystrophy, cystinuria, cystinosis, ornithine transcarbamylase deficiency, von Gierke disease, Pompe disease, glucose transporter protein type 1 deficiency, GM1 gangliosidosis, Sandhoff disease, Tay-Sachs disease, Fabry disease, Gaucher disease, Krabbe disease, Niemann-Pick disease, metachromatic leukodystrophy, Batten disease, Wolman's disease, Hurler syndrome, Scheie syndrome, Hurler-Scheie syndrome, Hunter syndrome, Sanfilippo syndrome, Morquio syndrome, Maroteaux-Lamy syndrome, Sly syndrome, mucolipidosis, homozygous familial hypercholesterolemia, Barth syndrome, Smith-Lemli-Opitz syndrome, Wilson's disease, hypophosphatemia, hypophosphatasia, cystic fibrosis, amyloidosis, and alpha1 antitrypsin deficiency, among many other disorders.	**Telehealth would significantly improve the rare disease patient experience.** According to the National Organization for Rare Disorders ("NORD"), "[f]or many rare diseases, there are only a handful of specialists nationwide, or even worldwide, who have expertise in that condition. As a result, patients often are forced to travel long distances to access their treating providers." NORD conducted a survey that revealed "Thirty-nine percent of patients travel at least 60 miles to receive medical care. The burden of travel is so great that 17 percent have moved (or are considering relocation) to be closer to treatment to manage their rare diseases over the long-term." For example, peer-reviewed medical literature recommends telehealth as an option for bringing lost to follow-up PKU patients back to life-long disease management and treatment adherence. "Frequent outpatient visits for diet maintenance, biochemical controls, and dietary evaluations make PKU difficult to manage for both patients and parents, resulting in loss of follow-up, especially in adult patients." Alternatively, one recent study during the COVID-19 pandemic illustrated the success of telehealth for approximately 100 PKU patients. Patients would draw a blood sample at home to measure phenylalanine levels, transport it to the lab for analysis, and submit journal details of diet, weight, and any therapeutic intervention electronically. The provider would meet with patient and assess the data, modifying diet and medicine as necessary. Telehealth resulted in better phenylalanine control for PKU patients and should be used for monitoring and follow-up, according to the study.

LEGISLATIVE TEXT	SUMMARY	RATIONALE
*"(3) **ELIGIBLE PROVIDER.—*** *"**(A) IN GENERAL.—**For the purpose of this subsection, an eligible provider who shall receive payment for telehealth services is a–* *"(i) physician;* *"(ii) psychologist;* *"(iii) neuropsychologist;* *"(iv) genetic counselor;* *"(v) social worker;* *"(vi) case manager;* *"(vii) dietitian;* *"(viii) behavioral therapist;* *"(ix) speech therapist;* *"(x) audiologist;* *"(xi) physical therapist; or* *"(xii) occupational therapist.* *"**(B) PEER-TO-PEER CONSULTS.—*** *"**(i) IN GENERAL.—**In the event of a peer-to-peer consult using telehealth, both the originating provider and distant provider shall receive payment for such services.* *"**(ii) ORIGINATING PROVIDER.—**For the purpose of clause (i), the originating provider is an eligible provider in the same location as the eligible individual during the consult.* *"**(iii) DISTANT PROVIDER.—**For the purpose of clause (i), the distant provider is an eligible provider with whom the originating provider is consulting.* *"**(C) OUT-OF-STATE PROVIDERS.—*** *"**(i) IN GENERAL.—**An out-of-state provider is an eligible provider if such provider satisfies the requirements of subsection (kk)(7)(A), which shall be expedited for the purpose of this subsection.* *"**(ii) STREAMLINED ENROLLMENT.—**With respect to subsection (kk)(7)(A), an out-of-state provider shall be deemed enrolled as a participating provider in the State of the eligible individual upon the submission by such provider of an attestation of expertise in the disease or condition with which such individual is diagnosed.*	The provision would specify an eligible provider includes a full range of specialists, including those typically part of the team of multi-discipline care management specialists who practice at clinics or centers of excellence dedicated to metabolic or connective tissue disorders. The provision would further provide for peer-to-peer consultations that allow two providers to receive payment when using telehealth to facilitate. This provision would also allow out-of-state providers enrolled as a participating provider in the state of the beneficiary to receive payment for telehealth services as an eligible provider. Enrollment for such provider can be achieved through a simple attestation of expertise in the disease or condition affecting the Medicaid beneficiary.	**Many rare diseases require multi-discipline care teams, some of which are located out-of-state.** For example, PKU requires regular monitoring and intervention at the metabolic clinic from the multidiscipline team of specialists, including physician, dietitian, genetic counselor, social worker, case manager, behavioral therapist, and a psychologist or neuropsychologist, who are needed to manage this complex condition. Hunter syndrome requires physicians, audiologists, speech therapists, physical therapists, occupational therapists, and behavioral therapists. Additionally, peer-to-peer consults allow for efficient care coordination and are often a necessity in rare diseases, especially multi-system disorders like cystinosis and Friedreich's ataxia. With respect to leveraging telehealth for care coordination that is necessary in managing such disorders, Colorado Medicaid provides payment for all providers in a peer-to-peer telehealth consult, and also covers speech therapists, physical therapists, occupational therapists, and behavioral therapists providing telehealth. Out-of-state providers is an issue that acutely affects rare diseases because of the limited number of specialists and capacity issues (especially for adult PKU patients accessing metabolic clinics). Some Medicaid programs have placed restrictions on providers located out-of-state, requiring them to have some sort of an in-state presence, while other states have explicitly allowed out-of-state providers as long as they are licensed in the state and enroll with the Medicaid program. This provision would remove several identified barriers for provider reimbursement for telehealth services.

LEGISLATIVE TEXT	SUMMARY	RATIONALE
"(yy) PREVIOUSLY OPTIONAL HOME HEALTH SERVICES.— *With respect to home health services described in subsection (a)(10)(D), the State plan shall provide payment for physical therapy, occupational therapy, or speech pathology and audiology services provided by a home health agency or by a facility licensed by the State to provide medical rehabilitation services if a prescribing physician (or other individual authorized to prescribe under State law) attests such a service is medically necessary for the individual."*	**Mandatory Coverage of Physical Therapy, Occupational Therapy and Audiology Services, Speech Therapy:** This provision would require payment for PT, OT, speech therapy, and audiology services under the Medicaid home health benefit. Such services are currently optional under the benefit.	**PT, OT, speech therapy, and audiology services are a necessary element of the management of many rare disorders and should be covered under the Medicaid home health benefit.** For example, children diagnosed with SYNGAP1-related intellectual disability, which is an ultra-rare neurodevelopmental disorder, rely on these vital services.
(b) EFFECTIVE DATE.—		
The amendments made by this section shall apply to drugs prescribed on or after the date that is 90 days after the date of the enactment of this Act.	This provision that would require Medicaid payment for home infusion therapy, durable medical equipment, medical supplies, medical foods, and PT, OT, speech therapy, and audiology services would go into effect 90 days after enactment of the bill.	**Providing three months following enactment for these policies to become effective will balance the immediate patient and provider need, with allowing state Medicaid plans reasonable time to comply in the same time frame as called for in sections 2 and 3.**
SEC. 6. MEDICAID ACCESS TO CARE DEMONSTRATION		
(a) IN GENERAL.—		
The Secretary of Health and Human Services (in this section referred to as the "Secretary") shall establish a two-year project (in this section referred to as the "Medicaid Access to Care Demonstration Project") beginning calendar year 2023 to evaluate the impact on patient outcomes of requiring Medicaid payment for a care coordination program involving a program navigator.	This provision would establish the Medicaid Access to Care Demonstration Project – a two-year demonstration project that begins on January 1, 2023 – to test Medicaid payment for a program navigator for certain patients.	**Because not all states currently provide for Medicaid program navigators, a demonstration project will help demonstrate the vital role such entities can play in ensuring beneficiaries have access to the services and supports best suited for their individual needs.** The Affordable Care Act established the navigator concept to aid consumers in selecting among Federally-facilitated Marketplace plans in participating states. *See* Patient Protection and Affordable Care Act § 1311(i), Pub. L. No. 111-148, 124 Stat. 119, 180-181 (codified at 45 C.F.R. § 155.210). States are permitted to require ACA Navigators to assist with Medicaid and CHIP eligibility and enrollment, entitling such services to a Federal match. *See* 76 Fed. Reg. 41866, 41878 (July 15, 2011). Establishing a demonstration project to evaluate the impact of requiring states to pay for navigators is an opportunity to maximize care coordination and coverage for patients with complex medical conditions.

LEGISLATIVE TEXT	SUMMARY	RATIONALE
(b) SCOPE.—		
The Medicaid Access to Care Demonstration Project shall include a minimum of ten States (including a minimum of two States in which at least 65 percent of the counties in the State are counties that have 6 or less residents per square mile, as determined by the Secretary) that do not currently provide payment for a navigator program to assist with beneficiary eligibility and enrollment to ensure the selection of coverage and benefits that best meet the individual needs of the beneficiary.	This provision would require the demonstration to include a minimum of ten states (including at least two rural states) that do not currently pay for a navigator.	**Ten states (including two rural states) should provide an adequate sample size to test the demonstration project.**
(c) NAVIGATOR.—		
***(1) IN GENERAL.** A State participating in the Medicaid Access to Care Demonstration Project shall establish a Medicaid navigator program within its Department of Health or by contract with an eligible entity.*	For the purpose of the demonstration project, this provision would require participating states to establish a Medicaid navigator on their own or by contract with a third party	**Ensuring states have flexibilities in selecting an entity to serve as a program navigator will allow the participating state to potentially maximize existing relationships, such as expanding the scope of work for its ACA Marketplace Navigator.**
(2) ELIGIBLE ENTITY. ***(A) IN GENERAL.** For the purpose of this Medicaid Access to Care Demonstration Project, an eligible entity is an entity with experience assisting consumers in—* *(i) navigating the local health care system, including Medicaid;* *(ii) understanding health care coverage options; and* *(iii) appealing denials and otherwise resolving delays in accessing health care services in Medicaid.* ***(B) CONSUMERS WITH SPECIAL NEEDS.** The eligible entity shall also have demonstrated experience serving consumers with special needs, including, but not limited to consumers with—* *(i) limited-English language proficiency;* *(ii) low literacy rates;* *(iii) disabilities; and* *(iv) multiple health conditions, including behavioral health issues.*	For the purpose of the demonstration project, this provision would require a state that opts to select third party to serve as a contract Medicaid navigator must have general experience in assisting consumers in health insurance enrollment and appeals. Additionally, such contract navigator must have specific experience serving consumers with special needs, including limited English language proficiency, low literary rates, disabilities, and multiple health conditions, including behavioral health.	**Medicaid beneficiaries with special needs will be the ones relying most on such a program so it is critical a contract navigator has appropriate experience serving these populations.**

LEGISLATIVE TEXT	SUMMARY	RATIONALE
(3) CONSUMER ASSISTANCE SERVICES. *The navigator shall assist in—* *(A) determining individual eligibility for medical assistance under title XIX of the Social Security Act;* *(B) Medicaid enrollment to ensure those eligible for medical assistance under title XIX of the Act are receiving all benefits to which they are entitled and most beneficial for their individual needs;* *(C) coordinating with local school systems with respect to medically necessary services, equipment, and furniture for children receiving medical assistance under title XIX of the Act;* *(D) coordinating care with social workers, including those with experience in neurogenetics, for children with medically complex conditions receiving medical assistance under title XIX of the Act; and* *(E) coordinating with a prescribing physician (or other individual authorized to prescribe under State law) to file an appeal for a coverage denial for medically necessary items and services, including covered outpatient drugs.*	For the purpose of this demonstration project, the navigator must assist with Medicaid eligibility determination, enrollment, and coordination with schools and social workers. The navigator must also provide support by coordinating with the prescriber in appealing denials of items and services.	**Beyond basic eligibility and enrollment assistance for Medicaid beneficiaries, it is especially important for children with complex conditions for the navigator to coordinate with school systems and social workers and assist with coverage appeals.**
(d) REPORT.—		
Not later than 180 days following the conclusion of the Medicaid Access to Care Demonstration Project, the Secretary shall submit to Congress a report– *(1) evaluating the impact that the navigator program had on Medicaid beneficiary access to care; and* *(2) recommending whether Congress should require all 50 States to provide payment for a Medicaid navigator.*	This provision would require a report to Congress examining the impact of the demonstration on patient access and outcomes for Medicaid beneficiaries. The report would also recommend whether all states should be required to establish navigator programs.	**Such a report to Congress would capture evidence necessary to support the need and benefit of each state requiring Medicaid navigators.**

LEGISLATIVE TEXT	SUMMARY	RATIONALE
SEC. 7. MATERNAL AND CHILD HEALTH		
(a) STATE MCHB BLOCK GRANTS— Section 501(a)(1) o f the Social Security Act (42 U.S.C. 701(a)(1)) is amended—		
(1) in subparagraph (C), by striking "and"; ***(2) in subparagraph (D), by striking ";";*** ***(3) inserting at end the following:*** *"(E) for the purpose of reducing morbidity and mortality in newborns and children at risk for heritable disorders that are not adopted for inclusion on a state newborn screening panel, to establish state-wide genetic screening programs for—* *"(1) epilepsy;* *"(2) autism spectrum disorders;* *"(3) hyperphenylalaninemia (for individuals born prior to January 1, 1980);* *"(4) skeletal dysplasia; and* *"(5) neuromuscular disorders; and*	**State-Wide Genetic Screening Program:** This provision would allow states to use MCHB grants to establish state-wide genetic screening programs to capture heritable disorders for which the state does not currently screen as part of its newborn screening program. It would focus on the genetic causes of epilepsy, autism spectrum disorders, skeletal dysplasia, and neuromuscular disorders. It would also be used to identify adults who could have undiagnosed PKU (all 50 states did not test for PKU until 1980).	**Clarifying MCHB state grants to provide for these genetic screening programs would maximize the potential of genetic testing in identifying heritable disorders that require early intervention but are not currently on state newborn screening panels.** Several rapidly progressing heritable disorders are not screened by states because they are not on the Health Resources and Services Administration's Recommended Uniform Screening Panel. These disorders are initially misdiagnosed due to clinical presentation. For example, late infantile neuronal ceroid lipofuscinosis type 2 ("CLN2 disease" – a form of Batten disease), Sanfilippo syndrome, Morquio A syndrome, and Duchenne muscular dystrophy are sometimes initially diagnosed as epilepsy, autism spectrum disorder, skeletal dysplasia, and neuromuscular disorders, respectively. The delay to treatment for patients with these heritable disorders could be reduced if states could use MCHB grants to establish genetic screening programs. Because such conditions present with life threatening symptoms in childhood, some of which that are rapidly progressing, early diagnosis and onset of treatment is vital. Gene panels for epilepsy, autism spectrum disorders, skeletal dysplasia, and neuromuscular disorders can fill a huge gap in disease management. Similarly, a hyperphenylalaninemia gene panel could end the diagnostic odyssey for individuals who are diagnosed with depression, anxiety, agoraphobia, or intellectual disability and born after January 1, 1980 (when *all* states formally screened for PKU).

LEGISLATIVE TEXT	SUMMARY	RATIONALE
"(F) to promote long-term follow-up for individuals diagnosed through newborn screening with a metabolic disorder that affects cognition through a public health campaign that develops training materials and leverages traditional and digital media to educate the public on— *"(1) the necessity of lifelong disease management by a multi-discipline team of specialists at a metabolic clinic;* *"(2) the opportunity to use telehealth for metabolic clinic visits; and* *"(3) the most recent standard of care, including the availability of and importance of adherence to therapeutic interventions, to prevent progressive cognitive impairment and other neurological and psychological manifestations.*	**State-Wide Metabolic Disorder Education:** This provision would help address the public health issue of patients who are diagnosed through newborn screening with a metabolic disorder that can result in mental health difficulties and cognitive impairment without proper disease management, but who are "lost to follow-up." It would allow states to use MCHB grants to promote life-long disease management through specialists at metabolic clinics.	**State programs to establish a public health campaign to bring patients with metabolic disorders back to clinic could help patients diagnosed with PKU, who are at risk for mental health difficulties and cognitive impairment without proper disease management.** PKU is a rare, inherited metabolic disorder that is characterized by the inability of the body to process the essential amino acid phenylalanine, which is toxic at high levels causing severe intellectual disability and other neurological problems, such as executive function impairment, memory loss, anxiety, depression, and phobias. According to published peer-reviewed medical literature, more than 50 percent of adults diagnosed with PKU are reported as "lost to follow up," which means they have had no contact with a metabolic clinic over the course of the previous two years. The American College of Medical Genetics and Genomics ("ACMG") state that "initiation of treatment for PKU should be undertaken as early as possible, preferably within the first week of life," and must continue on an individualized basis for the duration of life to prevent a "variety of adverse neurocognitive and psychiatric outcomes." According to ACMG, regular monitoring and intervention at a metabolic clinic from a multidiscipline team of specialists, which include a physician, dietitian, genetic counselor, social worker, case manager, behavioral therapist, and a psychologist or neuropsychologist, is a critical component of successful management of this complex condition.

LEGISLATIVE TEXT	SUMMARY	RATIONALE
(b) CLARIFYING THE SCOPE OF THE SPECIAL PROJECTS OF REGIONAL AND NATIONAL SIGNIFICANCE—Section 501(a)(2) of the Social Security Act (42 U.S.C. 701(a)(2)) is amended to read as follows:		
"(2) for the purpose of enabling the Secretary (through grants, contracts, or otherwise) to provide for special projects of regional and national significance, research, and training with respect to maternal and child health and children with special health care needs (including early intervention training and services development), including— *"(A) genetic disease testing, counseling, and information development dissemination programs, including efforts to promote genetic screening for heritable disorders that are not on the Recommended Uniform Screening Panel for newborn screening;* *"(B) grants relating to hemophilia (without regard to age), including funding for comprehensive hemophilia diagnostic and treatment centers;* *"(C) screening of newborns for cystic fibrosis, metabolic disorders (including lysosomal storage disorders and peroxisomal disorders), muscle disorders, primary immunodeficiency diseases, sickle cell disease, and other genetic disorders and follow-up services for such disorders; and* *"(D) a Rare Metabolic Disorder Surveillance program to ensure that patients diagnosed through newborn screening with rare metabolic disorders, especially women with phenylketonuria who are pregnant or planning to have children, receive appropriate primary and specialty care throughout their lifetime."*	**Genetic Screening Promotion for Non-RUSP Conditions:** This provision expressly clarifies that Special Projects of Regional and National Significance ("SPRANS") Program funds may be used for grants to promote genetic screening for non-RUSP conditions. **Newborn Screening Technical Correction:** This provision expressly clarifies that SPRANS funds may be used for newborn screening of cystic fibrosis, lysosomal storage disorders, metabolic disorders, muscle disorders, peroxisomal disorders, and primary immunodeficiency diseases. The statute currently only mentions sickle cell disease. **Rare Metabolic Disorder Surveillance Program:** This provision would direct SPRANS funds to establish a Rare Metabolic Disorder Surveillance Program for individuals diagnosed with metabolic disorders, such as women diagnosed with PKU and who are pregnant or planning to have children.	**Clarifying that SPRANS funds can be used to promote genetic screening for non-RUSP conditions, the screening of newborns for debilitating genetic disorders that are fatal if untreated, and to conduct metabolic disorder surveillance will help ensure better patient outcomes.** The SPRANS program is a set aside program under the Maternal Child and Health Services Block Grant Program that provides competitive grants for research and training programs and services related to maternal and child health and children with special health care needs. SPRANS funds may be used for genetic disease testing, counseling, and information development and dissemination programs; for grants relating to hemophilia including hemophilia treatment centers; and for the screening of newborns for sickle cell disease and other genetic disorders and for related follow-up services. *See* SSA § 501(a)(2). It is well established that there are significant gaps in United States newborn screening with respect to adding conditions to the RUSP, state adoption of RUSP conditions, and long-term follow-up and supports. Long-term follow up is especially vital to mitigate risk of maternal PKU syndrome. Because of the threat maternal PKU syndrome has on fetal development, a Rare Metabolic Disorder Surveillance Program will promote the mission of SPRANS funds. This is also true of clarifying that states can use such funds for state newborn screening of additional medically complex conditions and promote genetic screening for non-RUSP conditions, most of which are rapidly progressing.