Clarifying Amendment to Section 1927 of the Social Security Act (Medicaid Payment for Covered Outpatient Drugs)

Purpose: To clarify the Medicaid drug coverage provision in section 1927 of the Social Security Act ("SSA") to ensure patients with a rare disease are able to access medically necessary covered outpatient drugs when prescribed for their Food and Drug Administration ("FDA") approved use.

Issue: Budget constraints are causing states to exploit existing federal law to discriminate against Medicaid patients with debilitating and potentially fatal rare diseases by rationing access to therapies prescribed to treat their conditions. Specifically, some Medicaid plans are implementing prior authorization programs that compel alternative drugs (including off-label uses), unnecessary diagnostic tests, or other medical services as prerequisites to a rare disease therapy that a treating physician has deemed medically necessary.[1] States also have obtained Centers for Medicare & Medicaid Services ("CMS") waivers to deny access to medically necessary rare disease therapies. Because these restrictions and denials are targeting rare disease therapies, the neediest Americans who have already endured the physical and emotional toll of the multi-year odyssey from the onset of symptoms to an accurate diagnosis are disproportionately harmed. Moreover, with children representing more than half of the 30 million Americans that suffer from a rare disease,[2] this discrimination could result in a pediatric public health crisis.

Current Law: Prescription drug coverage is an optional benefit in traditional Medicaid. If providing this benefit, states must cover a medically necessary covered outpatient drug if the manufacturer of such drug has executed a Medicaid Drug Rebate agreement with CMS.[3] Medically necessary covered outpatient drugs include those prescribed for uses approved by the FDA or supported in compendia specified in section 1927 of the SSA.[4] Although states may use prior authorization programs to manage utilization of covered outpatient drugs,[5] such programs

[1] This practice is often referred to as a "step therapy" or "fail first" protocol.

[2] *See, e.g.*, S. Res. 368, 113th Cong. (2014) (enacted) (providing the latest rare disease statistics in designating February 28, 2014 as "Rare Disease Day" in the United States).

[3] *See* 42 U.S.C.S. §1396a(a)(54) (LexisNexis 2014) (requiring a state that chooses to provide a prescription drug benefit to its Medicaid beneficiaries to comply with section 1927 of the SSA); *id*. at §1396r-8(a)(1) (LexisNexis 2014) (requiring Medicaid payment for a covered outpatient drug if its manufacturer has "entered into" and has "in effect" a rebate agreement).

[4] CMS expressly prohibits prior authorization programs from denying payment for compendia-listed off-label uses of covered outpatient drugs. *See* CMS State Medicaid Drug Rebate Program Release for State Medicaid Directors #141 (May 4, 2006). Because of this prohibition, logic dictates that states are likewise forbidden from using prior authorization to deny payment for a covered outpatient drug that a provider has prescribed for its FDA approved use. Because a state Medicaid must cover both categories of drugs without exception, one must recognize them as "medically necessary."

[5] *See* 42 U.S.C.S. §1396r-8(d)(1) (LexisNexis 2014) (describing prior authorization as a permissible restriction that states may place on covered outpatient drugs).

shall not deny Medicaid beneficiaries access to such drugs that are medically necessary.[6] A state may obtain a section 1115 demonstration project waiver to relax its obligations under section 1902 of the SSA,[7] but such waivers do not allow a state to circumvent the requirements and limitations found in section 1927 of the SSA, which controls how such drugs are covered.[8]

Policy Rationale: States are skirting their obligation under Federal Medicaid law to cover medically necessary therapies. Federal legislation that would both prevent CMS from enabling states to ration rare disease therapies through Medicaid waiver and clarify the congressional intent of prior authorization programs in Medicaid is critical in the context of rare disease therapies. Such an amendment would:

- **Provide needed clarification to existing Federal law to ensure health care equity for Medicaid beneficiaries with rare diseases:** States increasingly are targeting rare disease therapies for coverage restrictions. In one state, a prior authorization program for a prophylaxis regimen of a medically necessary therapy that FDA has approved to replace the deficient plasma protein that causes hereditary angioedema ("HAE") requires the patient to not only endure a minimum of 12 emergency room visits over six months to treat life-threatening episodes (which by itself is egregious), but also fail prophylaxis regimens on two different classes of alternative drugs.[9] Because of these prerequisites, Medicaid beneficiaries with HAE are unable to access a proven therapeutic regimen. Such a result increases the risk of poor health outcomes and associated costs. Strengthening the statutory text to better reflect the congressional intent regarding Medicaid coverage of prescription drugs will prevent states from construing Federal law in a manner that completely undermines the drug benefit for patients with rare diseases.

- **Improve health outcomes for Medicaid beneficiaries with rare diseases, which will reduce the economic burden on Medicaid and other federal safety net programs:** Restrictive Medicaid drug coverage policies are disrupting the physician-patient joint

[6] *See* H.R. Rep. No. 881, 101st Cong., 2d Sess. 96-98 (1990) (stating the clear congressional intent in support of this proposition); Medicaid Program; Payment for Covered Outpatient Drugs Under Drug Rebate Agreements with Manufacturers, 60 Fed. Reg. 48442, 48454-48455 (Sept. 19, 1995) (expanding upon the underlying policy rationale of the congressional intent by suggesting that using step therapy as prior authorization criteria to put drugs out of reach for patients poses a risk for poor health outcomes, which would result in increased costs to the Medicaid program). Despite this analysis, CMS never promulgated a final rule. Such regulations could have provided the clarity necessary to ensure patient access to medically necessary covered outpatient drugs.

[7] *See* 42 U.S.C.S. § 1315 (LexisNexis 2014).

[8] *Id.* at §1396a(a)(54) (requiring a state that chooses to provide a prescription drug benefit to its beneficiaries to comply with section 1927 of the SSA).

[9] *See, e.g.*, ARK. DIV. OF MEDICAL SERVS., ARK. MEDICAID PRESCRIPTION DRUG PROGRAM PRIOR AUTHORIZATION CRITERIA, https://www.medicaid.state.ar.us/Download/provider/pharm/PACriteria.pdf (follow "C1 Esterase inhibitor (Cinryze)" hyperlink on page 3) (last updated Dec. 23, 2013). Medicaid patients with cystic fibrosis, cystinosis, Gaucher disease, and the various types and subtypes of mucopolysaccharidoses ("MPS") are experiencing similar barriers to accessing their treatment.

determination of rare disease treatment regimens.[10] For patients unable to access a therapy that a physician has concluded is best suited for their unique clinical needs, poor health outcomes are a significant risk. CMS predicted that using prior authorization for the sole purpose of restricting or denying a patient access to a drug "could result in [Medicaid beneficiaries] being treated with alternat[ive] therapies that may not be in their best interest…[, which] could result in increased program costs if other medical services, such as inpatient hospital services, are necessary because a drug therapy is made less accessible under the State Medicaid program."[11] Consistent with this rationale, it is reasonable to suppose that access without delay to therapy as prescribed will allow individuals to advance further academically and professionally because of fewer health-related obstacles. Such advancement will reduce the long-term burden on Medicaid, Supplemental Security Income, and Social Security Disability Insurance.

- **Preserve the continuity of care for Medicaid beneficiaries with a rare disease:** Some states are forcing Medicaid patients with a rare disease to discontinue using covered outpatient drugs that have proven effective in controlling or improving their individual condition. Ironically, these states are taking from the patient precisely what the federal government had intended to provide with the enactment of the Orphan Drug Act ("ODA") and the subsequent establishment of policies and programs with an emphasis on rare diseases.[12] Even more confounding is that patients, their families, and their caregivers have overcome considerable obstacles to receive a correct diagnosis,[13] yet in cases where the FDA has approved a therapy for the rare disease, states are refusing to recognize that it is medically necessary for the patient to continue this therapeutic regimen that has

[10] In developing a treatment regimen, prescribing physicians consider patient preference and tolerability, the characteristics of the disease being treated, the individual patient's treatment history, disease severity, age, gender, and comorbidities, as well as contraindications, warnings, precautions, and the overall safety and efficacy profile of all available treatment options.

[11] 60 Fed. Reg. at 48454.

[12] *See* Orphan Drug Act, Pub. L. No. 97-414, 96 Stat. 2049 (1983) (codified as amended at 21 U.S.C.S. §§ 360aa-360ff; 26 U.S.C.S. § 45C (LexisNexis 2014)); H.R. REP. NO. 97-840, pt. 1, at 8 (1982) (providing the opportunity for manufacturers to obtain market exclusivity, tax credits, and clinical development grants as incentives to "encourage orphan drug development"). The federal government has expended significant resources in addition to the ODA incentives to drive the development and commercialization of rare disease therapies by awarding development grant opportunities through the National Institutes of Health and providing funding through the Centers for Disease Control and Prevention to develop newborn screening programs.

[13] For most rare diseases when family history is unknown or unavailable, a correct diagnosis often will not occur for an average of five years from the onset of symptoms. *See, e.g.*, Patti A. Engel et al., *Physician and Patient Perceptions Regarding Physician Training in Rare Diseases: The Need for Stronger Educational Incentives for Physicians*, 1(2) J. OF RARE DISORDERS 9 (Dec. 2013). This diagnostic odyssey often requires several visits to the ER and physician office, hospitalizations, and surgical interventions. *Id*. at 14 (reporting that a patient with a rare disease will see an average of seven physicians prior to diagnosis). Once properly diagnosed, most patients with a rare disease have few, if any, therapeutic options. For those patients diagnosed with one of the approximately 200 rare diseases with an FDA approved therapy, the treatment regimen often requires regular intervention for the duration of their lives.

controlled or improved their condition. The odyssey of the rare disease patient is already characterized by physical and emotional challenges[14] – rationing access to treatment exacerbates them. Despite presenting with symptoms and swelling episodes since the age of six, doctors did not diagnose a patient with severe HAE until she was 30-years-old due to a lack of familiarity with the disease and no family history (she was adopted). Although a therapy for HAE has been available in Europe since the 1980s, it was not available in the U.S. This therapy has successfully controlled her condition since she began receiving treatment in 2006 as part of the clinical trial, but when enrolling in Medicaid in 2011 and again in 2013, the state required her to discontinue her FDA approved treatment regimen to first satisfy a step therapy protocol. Many rare diseases are progressive in nature and some, such as HAE, can be fatal from a single swelling attack. Therefore, continuity of care and preventing delays in accessing proven therapeutic regimens is essential to slowing disease progression and preventing severe debilitation or death for some patients.

- **Promote the value of an FDA label:** States are ignoring the value of an FDA label by forcing Medicaid beneficiaries to use alternative drugs "off-label" or choosing to only treat their symptoms and episodes when there is an FDA approved treatment for the underlying rare disease. Drug development is incredibly expensive, with a low probability of success.[15] For rare diseases, the small patient populations magnify this risk, but the ODA allows innovators to mitigate it.[16] Coverage policies, however, are requiring children with HAE to use off-label drugs with documented severe, hazardous pharmacological side effects, rather than allow them to use the FDA approved treatment that replaces the deficient plasma protein that causes the disease.[17] Similarly, instead of covering an FDA approved therapy that replaces the deficient enzyme in children with

[14] *See, e.g.*, SHIRE, RARE DISEASE IMPACT REPORT: INSIGHTS FROM PATIENTS AND THE MEDICAL COMMUNITY 8 (April 2013) (revealing that between 72 percent and 89 percent of rare disease patients and caregivers surveyed have reported feelings of depression, anxiety, and stress).

[15] *See, e.g.*, INSTITUTE OF MEDICINE OF THE NATIONAL ACADEMIES, RARE DISEASES AND ORPHAN PRODUCTS: ACCELERATING RESEARCH AND DEVELOPMENT 147-148 (2010) (estimating that even with a 10 percent chance of success manufacturers willingly make an investment of as much as $1 billion and 14 years to develop and commercialize a drug).

[16] *See, e.g.*, 26 U.S.C.S. § 45C (allowing innovators to claim a tax credit to offset 50% of its qualifying clinical testing expenses for an orphan designated drug).

[17] *See, e.g.*, ARK. DIV. OF MEDICAL SERVS., *supra* note 9. Severe risks associated with androgen use include liver damage and heart damage. Evidence demonstrates that androgens may interfere with a child's bone maturation and sexual maturation. For women, androgen use is relatively contraindicated due to the significant risk of amenorrhea, irreversible virilization, including hirsutism, clitoral hypertrophy, and voice deepening, early onset of osteoporosis, weight gain, alopecia, acne, and, in pregnant women, fetal virilization and other teratogenic complications. *See* AMERICAN SOCIETY OF HEALTH-SYSTEM PHARMACISTS, AMERICAN HOSPITAL FORMULARY SYSTEM DRUG INFORMATION § 68:08 (2012); TRUVEN HEALTH ANALYTICS, DRUGDEX INFORMATION SYSTEM, DRUGDEX ® EVALUATIONS: DANAZOL (2013).

Hunter Syndrome, another state relies on a section 1115 waiver[18] to only cover episodic and symptomatic interventions, such as surgeries, hospitalizations, diagnostic imaging, physical therapy, antibiotics, and a nebulizer. This devaluing of the FDA approval process and the ODA incentives is not receiving adequate attention. Ironically, the media and Congress are preoccupied with social media campaigns by patients seeking early access to developmental stage drugs that may lack safety and efficacy data.

- **Offer hope to millions of Americans affected by one of the nearly 7,000 life threatening and debilitating rare diseases that lack an FDA approved treatment:** The persistence of restrictive and often insurmountable coverage policies that place rare disease therapies out of reach for the patient will impede continued innovation in patient-centric, individualized treatments for rare diseases. More than 30 years ago, Congress enacted the ODA to provide incentives for drug manufacturers to make the investment to bring therapies to market for rare diseases. Those incentives comprise seven years of market exclusivity, a tax credit for 50% clinical testing expenditures in a taxable year, and federal grants to offset clinical development costs.[19] Because of these policies, FDA has approved more than 400 rare disease therapies since the enactment of the ODA, while drug manufacturers had only obtained marketing approval for 34 rare disease therapies prior to the ODA.[20] The enactment of clarifying legislation that would ensure access to rare disease therapies would align with the ODA and more than 30 years of rare disease policy to further stimulate innovation in patient-centric, individualized treatments for rare diseases, as well as advance the public policy goal of encouraging FDA approved treatments that benefit pediatric populations.

Description of the amendment: This legislation will clarify that states shall <u>not</u>:

- compel prerequisite drugs, tests, or other services as part of the prior authorization of a covered outpatient drug that is prescribed for a rare disease or condition that is an FDA approved use of such drug; or

- use section 1115 Medicaid demonstration project waivers to deny, restrict, or otherwise limit access to a covered outpatient drug that is prescribed for a rare disease or condition that is an FDA approved use of such drug.

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[18] *See* Letter from Cindy Mann, Dir., CMS, to Susan Hoffman, Acting Dir., Oregon Health Authority (June 27, 2014) (extending Oregon's section 1115 waiver through June 30, 2016). CMS originally granted the waiver allowing the Prioritized List of Health Services in 1993. It only has recently begun excluding rare disease therapies, including treatments for lysosomal storage disorders. Oregon has inappropriately targeted the various types and subtypes of MPS and Gaucher disease, which are debilitating and often fatal, by placing FDA approved therapies for those diseases as low priority treatments based on efficacy claims. FDA, however, has already evaluated the safety and efficacy of these therapies for their approval.

[19] *See* 21 U.S.C.S. §§ 360aa-360ff; 26 U.S.C.S. § 45C (LexisNexis 2014)).

[20] *Compare* FDA., U.S. DEP'T OF HEALTH & HUMAN SERVS., ORPHAN DRUG PRODUCT DESIGNATION DATABASE, http://www.accessdata.fda.gov/scripts/opdlisting/oopd *with* H.R. REP. NO. 97-840, pt. 1, at 7 (1982).

Amendment language: The amendment is as follows:

(a) Section 1927(d)(5) of the Social Security Act (42 U.S.C. 1396r–8(d)(5)) is amended as follows:

(1) By redesignating paragraphs (A) and (B), as subparagraphs (i) and (ii), respectively.

(2) By inserting "(A) In general." after "Requirements of prior authorization programs.".

(3) By adding at the end the following new paragraph:

"(B) Limitation. Following a diagnosis of a rare disease or condition, as defined in section 526(a)(2) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bb(a)(2)), a prior authorization program described in subparagraph (A) shall not require a prerequisite drug, test, or service, including emergency room intervention, if the covered outpatient drug described in subparagraph (A) is prescribed for such rare disease or condition and such use is approved under section 505(b) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(b)) or under 351(a) of the Public Health Service Act (42 U.S.C. 262(a))."

(b) Section 1927(d) of the Social Security Act (42 U.S.C. 1396r–8(d)) is amended by adding at the end the following new paragraph:

"(8) Use of section 1115 waivers. Notwithstanding any other provision of law, the Secretary shall not allow a State through a waiver under section 1115 (42 U.S.C. 1315) to deny, restrict, or otherwise limit access to a covered outpatient drug if such drug is prescribed for a rare disease or condition, as defined in section 526(a)(2) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bb(a)(2)), and such use is approved under section 505(b) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(b)) or under 351(a) of the Public Health Service Act (42 U.S.C. 262(a))."