

March 17, 2015

The Honorable Fred Upton  
Chairman  
Committee on Energy & Commerce  
United States House of Representatives  
Washington, DC 20515

The Honorable Diana DeGette  
Member  
Committee on Energy & Commerce  
United States House of Representatives  
Washington, DC 20515

**Re: Access to 21<sup>st</sup> Century Cures for Individuals with Rare Diseases**

Dear Chairman Upton and Representative DeGette:

Many rare disease patients enrolled in Medicaid cannot access lifesaving and life improving Food and Drug Administration (“FDA”) approved therapies as a result of barriers designed to make it impossible for rare disease patients to benefit from medical innovation. As organizations committed to advancing patient access to innovative technologies that can diagnose, treat, and potentially cure rare diseases, we applaud your leadership in providing a collaborative forum for stakeholders to shape the *21<sup>st</sup> Century Cures* initiative. Although the rare disease community has previously shared a wide range of perspectives on innovation and pressing public health needs, we are united in asking Congress to use *21<sup>st</sup> Century Cures* to prohibit Medicaid plans from rationing access to rare disease therapies. States are claiming conflicting federal statutes give them authority to use step therapy protocols as part of prior authorization to deny access to medically necessary therapies. Despite recognizing the significant risk that the forced use of alternative treatments poses to patient health outcomes and the resulting increased costs to the health care system, the Centers for Medicare & Medicaid Services (“CMS”) has not intervened. CMS also in some instances has enabled rationing through Medicaid waivers. Simply put, congressional intervention through legislation is needed to expand the “path” to *21<sup>st</sup> Century Cures* to ensure **“access” to *21<sup>st</sup> Century Cures***.

We are proposing a solution that is based on our understanding of the direct correlation between innovation and access, which has been gleaned from decades of our efforts advocating for new and improved technologies for the nearly 7,000 identified rare diseases and conditions. Before restricted access to rare disease therapies becomes a public health crisis for not only millions of Medicaid beneficiaries, but also every individual and family member coping with a rare disease, we urge Congress to address this issue through *21<sup>st</sup> Century Cures*. More specifically, Congress must consider enacting legislation that will prohibit discriminatory utilization control practices by state Medicaid plans – “epic” legislation that would ensure:

- **Equity** for individuals with rare diseases and conditions;
- **Physician** and patient treatment determination;
- **Innovation** of and access to individualized treatment; and
- **Continuity** of care.

Scientific advancements have led to the development of modern therapies that are not only satisfying unmet medical need, but also significantly improving the standard of care. Curative technologies like gene therapy platforms are finally within reach. Science has evolved to allow physicians to prescribe treatment regimens that are specific to the unique clinical needs of the individual patient. Coverage policies for rare disease therapies, however, are not keeping pace with this innovation. For individuals affected by debilitating and life threatening rare diseases, this disconnect is creating a serious disparity in care. The *21<sup>st</sup> Century Cures* initiative is an opportunity for necessary coverage reform measures to complement policies designed to spur innovation into treatments and cures for rare diseases. Without such measures, patient benefit from new technologies will be limited.

Medicaid plans currently are limiting the ability of patients with a rare disease to access medically necessary FDA approved therapies for their condition. Increasingly common are policies requiring alternative drugs (including off-label uses), unnecessary diagnostic tests, or other medical services as prerequisites to a therapy that a treating physician has deemed medically necessary. These conditions to access a therapy generally are not based on the most recently published peer-reviewed treatment guidelines for the rare disease (if one even exists), but often instead are assembled through a cursory and selective review of medical literature that is initiated to support the restrictive coverage policy. The resulting policy might favor older, antiquated treatment standards, or fail to recognize the unique value of all available treatments. Certainly, these arbitrary, rigid coverage policies preclude patients from benefiting from the prescribing physician's recommendation, which is based on patient preference and tolerability, the characteristics of the disease being treated, the individual patient's treatment history, disease severity, age, gender, and comorbidities, and contraindications, warnings, precautions, and the overall adverse event and efficacy profile of all available treatment options. For example, Medicaid programs in Alaska, Arkansas, Georgia, and Minnesota have established prior authorization programs that use step therapy to restrict access to certain FDA-approved therapies for cystic fibrosis ("CF"), cystinosis, Gaucher disease, and hereditary angioedema ("HAE").<sup>1</sup>

Until recently, Arkansas was restricting access to a more recently approved medically necessary therapy that restores function of the deficient protein that causes CF in patients with specific gene mutations. Before Arkansas Medicaid would cover this therapy for a 14-year old CF patient from Walnut Ridge, Arkansas, it required her to use an inflatable vest three times each day to loosen mucus in her lungs and to fail a 12-month regimen of older, alternative drugs that only treat symptoms associated with CF. This policy clearly is in conflict with the standard of care and not in the best interest of the patient.

CF patients are not the only rare disease patient population that has experienced discrimination in Arkansas. Arkansas also has implemented an extraordinarily restrictive step therapy protocol for a prophylaxis regimen of a medically necessary therapy that replaces the deficient plasma protein that causes HAE. A Rogers, Arkansas mother and her two teenage children suffer from severe HAE and have been stabilized on this therapy since its clinical trial in 2006. Counter to the standard of care, each time the family "churns" onto Medicaid, Arkansas forces all three patients to discontinue the therapy. Instead, they must first endure a minimum of

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<sup>1</sup> This trend will continue. For example, California recently enacted legislation that will result in its Medicaid program more frequently implementing utilization management policies for rare disease therapies.

12 emergency room visits over six months to treat life-threatening episodes and also fail prophylaxis regimens on two different classes of alternative drugs. These alternative drugs are not all approved for adults with HAE and none are approved for use in children. Moreover, such off-label use is not supported in medical literature, and one of these alternative drugs also is relatively contraindicated in women.

Congress never intended for states to use prior authorization programs to deny Medicaid beneficiaries access to medically necessary covered outpatient drugs.<sup>2</sup> CMS has acknowledged that using step therapy to put such drugs out of reach for the patient poses a risk for poor health outcomes, which would result in increased costs to the Medicaid program.<sup>3</sup> By failing to mitigate or eliminate this threat, CMS missed an opportunity to provide necessary clarity for acceptable prior authorization programs for covered outpatient drugs. As a consequence, several states argue that they have authority to use step therapy to deny access to medically necessary drugs under existing federal law.

Of equal concern is the section 1115 waiver that permits Oregon Medicaid to establish a Prioritized List of Health Services. Oregon only covers items and services that it has ranked at a high enough level on this list. Therapies for lysosomal storage disorders, such as Gaucher disease and the various types and subtypes of mucopolysaccharidoses (“MPS”), are given low priority, which results in denials. For example, Oregon has used the list to repeatedly deny an 11-year old Hunter Syndrome (MPS II) patient from Tenmile in Douglas County, Oregon access to a medically necessary enzyme replacement therapy. Rather than cover this therapy that replaces the deficient enzyme causing the underlying debilitating and often fatal disease, Oregon has chosen to only cover episodic and symptomatic interventions, such as surgeries, hospitalizations, diagnostic imaging, physical therapy, antibiotics, and a nebulizer – again, in conflict with the standard of care and not in the best interest of the patient.

These examples demonstrate the devastating impact that recent and ongoing abuses of the prior authorization process and section 1115 waivers are having on Medicaid patients with rare diseases. Congress designed Medicaid to provide a critical safety net to millions of Americans with limited resources or disabilities, yet policies that prevent access to medically necessary rare disease therapies are limiting the utility of the program for a certain subset of beneficiary.

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. Our legislative proposal (attached) would restore health care equity for Medicaid patients with a rare disease by expressly prohibiting Medicaid plans from using coverage prerequisites for therapies that FDA has approved for the rare disease and preventing CMS from granting waivers for Medicaid demonstration projects that deny coverage of such rare disease therapies. Such a policy would complement the discovery, development, and delivery policy objectives of *21<sup>st</sup> Century Cures*. Moreover, it is consistent

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<sup>2</sup> See H.R. Rep. No. 881, 101<sup>st</sup> Cong., 2d Sess. 96-98 (1990).

<sup>3</sup> See Medicaid Program; Payment for Covered Outpatient Drugs Under Drug Rebate Agreements with Manufacturers, 60 Fed. Reg. 48442, 48454 (Sept. 19, 1995). CMS has not finalized the proposed rule.

with the innovation goals of the Orphan Drug Act and more than 30 years of subsequent rare disease policy.

The *21<sup>st</sup> Century Cures* initiative provides a unique opportunity for Congress to remove the gap between the science of cures and the ability for the patient to access lifesaving and life improving therapies. For rare disease patients, who often spend years without a proper diagnosis and have few, if any, treatment options, it is absolutely critical to ensure access to proven, valued therapeutic regimens that a physician has prescribed based on the unique clinical needs of the individual patient. Legislation that will eliminate barriers to accessing rare disease therapies in Medicaid will allow our nation to continue its forward progress in developing innovative therapies and individualized treatment approaches that benefit the public health. Our organizations are determined to work with you to enact “epic” legislation that will protect and promote patient access to innovative treatments and cures and that recognizes the equal importance of both a “path” to and “access” to *21<sup>st</sup> Century Cures*. Thank you for considering our views.

Sincerely,



Barbara Wedehase, MSW, CGC  
Executive Director  
National MPS Society



Stephanie Bozarth, MSW  
President, Board of Directors  
National MPS Society

5p- Society/ National Cri du Chat Syndrome Support Organization  
A Kids' Brain Tumor Cure Foundation (The Pediatric Low Grade Astrocytoma Foundation)  
Adrenal Insufficiency United  
Adult Congenital Heart Association  
Adult Polyglucosan Body Disease Research Foundation  
Alagille Syndrome Alliance  
ALD Connect  
Alpha-1 Foundation  
Alström Angels  
American Autoimmune Related Diseases Association  
American Behcet's Disease Association  
American Partnership For Eosinophilic Disorders  
Amyloidosis Support Groups  
Aplastic Anemia & MDS International Foundation  
Association for Glycogen Storage Disease  
Avery's Angels Gastroschisis Foundation  
Batten Disease Support and Research Association  
Beautiful You MRKH Foundation, Inc.  
Bridge the Gap – SYNGAP Education and Research Foundation

CADASIL Together We Have Hope  
Caregiver Action Network  
Caring for Carcinoid Foundation  
Celiac Support Association  
CCHS Family Network  
Charcot-Marie-Tooth Association  
Children's PKU Network  
Children's Tumor Foundation  
Chordoma Foundation  
Chronic Granulomatous Disease Association  
Coalition for Pulmonary Fibrosis  
Congenital Hyperinsulinism International  
Cooley's Anemia Foundation  
Crohn's & Colitis Foundation of America  
Cure AHC  
cureCADASIL/ CADASIL Association Inc.  
CureDuchenne  
CurePSP  
Cutaneous Lymphoma Foundation  
Cystinosis Foundation  
Cystinosis Research Network  
debra of America  
Dempster Family Foundation  
Dravet Syndrome Foundation  
Dupuytren Foundation  
EB Research Partnership, Inc.  
EveryLife Foundation for Rare Diseases  
Fabry Support & Information Group  
Fibromuscular Dysplasia Society of America  
Foundation for Ichthyosis & Related Skin Types, Inc.  
Friedreich's Ataxia Research Alliance  
GBS/CIDP Foundation International  
Genetic Alliance  
Global Genes  
Global Hydranencephaly Foundation  
Gwendolyn Strong Foundation  
Hemophilia Federation of America  
Hereditary Neuropathy Foundation  
HHT Foundation International, Inc.  
Histiocytosis Association  
Hope for Hypothalamic Hamartomas  
Immune Deficiency Foundation  
International Cystinuria Foundation  
International FOP Association  
International Myeloma Foundation  
International Pemphigus & Pemphigoid Foundation

ISMARD – The International Advocate for Glycoprotein Storage Diseases  
Jeffrey Modell Foundation  
Jonah’s Just Begun  
Joubert Syndrome & Related Disorders Foundation  
Lennox-Gastaut Syndrome Foundation  
Let Them Be Little X2 Foundation  
Lipodystrophy United  
Little Miss Hannah Foundation  
Lymphangiomatosis & Gorham's Disease Alliance  
MLD Foundation  
Moebius Syndrome Foundation  
Myotonic Dystrophy Foundation  
National Adrenal Diseases Foundation  
National Down Syndrome Society  
National Eosinophilia-Myalgia Syndrome Network  
National Fragile X Foundation  
National Gaucher Foundation, Inc.  
National Hemophilia Foundation  
National Organization for Rare Disorders  
National PKU Alliance  
National Tay-Sachs & Allied Diseases Association  
National Urea Cycle Disorders Foundation  
NBIA Disorders Association  
New York State Rare Disease Alliance  
NGLY1 Foundation  
Noah’s Hope  
Organic Acidemia Association  
Organizations for Rare Diseases India  
Oxalosis & Hyperoxaluria Foundation  
Parent Project Muscular Dystrophy  
Patient Services, Inc.  
PCDH19 Alliance  
Pediatric Hydrocephalus Foundation  
Phelan-McDermid Syndrome Foundation  
Pitt Hopkins Research Foundation  
Platelet Disorder Support Association  
Prader-Willi Syndrome Association (USA)  
Pulmonary Fibrosis Foundation  
Rare Disease United Foundation  
RARE Science  
RASopathies Network USA  
Relapsing Polychondritis Awareness and Support Foundation, Inc.  
Sanfilippo Foundation for Children  
Saving Case & Friends  
SCAD Alliance  
Scleroderma Foundation, Inc.

Scleroderma Research Foundation  
Share & Care Cockayne Syndrome Network, Inc.  
Short Bowel Syndrome Foundation, Inc.  
The Epidermolysis Bullosa Medical Research Foundation  
The Erythromelalgia Association  
The FPIES Foundation  
The LAM Foundation  
The Marfan Foundation  
The Mastocytosis Society, Inc.  
The United Mitochondrial Disease Foundation  
The XLH Network, Inc.  
Tuberous Sclerosis Alliance  
Turner Syndrome Society of the United States  
United Leukodystrophy Foundation  
United Pompe Foundation  
U.S. Hereditary Angioedema Association  
Usher Syndrome Coalition  
Vasculitis Foundation  
Vestibular Disorders Association  
VHL Alliance  
We Are R.A.R.E., Inc.  
Williams Syndrome Association  
Wilson Disease Association

Enclosure

## 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.<sup>1</sup> 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,<sup>2</sup> 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.<sup>3</sup> 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.<sup>4</sup> Although states may use prior authorization programs to manage utilization of covered outpatient drugs,<sup>5</sup> such programs

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<sup>1</sup> This practice is often referred to as a “step therapy” or “fail first” protocol.

<sup>2</sup> See, e.g., S. Res. 368, 113<sup>th</sup> Cong. (2014) (enacted) (providing the latest rare disease statistics in designating February 28, 2014 as “Rare Disease Day” in the United States).

<sup>3</sup> 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).

<sup>4</sup> 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.”

<sup>5</sup> 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.<sup>6</sup> A state may obtain a section 1115 demonstration project waiver to relax its obligations under section 1902 of the SSA,<sup>7</sup> 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.<sup>8</sup>

**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.<sup>9</sup> 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

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<sup>6</sup> See H.R. Rep. No. 881, 101<sup>st</sup> 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.

<sup>7</sup> See 42 U.S.C.S. § 1315 (LexisNexis 2014).

<sup>8</sup> *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).

<sup>9</sup> 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.<sup>10</sup> 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.”<sup>11</sup> 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.<sup>12</sup> Even more confounding is that patients, their families, and their caregivers have overcome considerable obstacles to receive a correct diagnosis,<sup>13</sup> 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

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<sup>10</sup> 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.

<sup>11</sup> 60 Fed. Reg. at 48454.

<sup>12</sup> 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.

<sup>13</sup> 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<sup>14</sup> – 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 forcing Medicaid beneficiaries to use alternative drugs “off-label” and limiting coverage to the treatment of symptoms and episodes – this is happening *even when there is an FDA approved therapy available to treat the underlying rare disease*. This devaluing of the rigorous FDA drug approval process and resulting FDA label is deeply troubling for patients and their clinicians and puts future development of rare disease therapies at risk. Drug development is incredibly costly, with a low probability of success.<sup>15</sup> For rare diseases, the small patient populations magnify this risk, but the ODA allows innovators to mitigate it.<sup>16</sup> There are, however, coverage policies that require 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.<sup>17</sup> Similarly, instead of covering an FDA approved therapy that replaces the

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<sup>14</sup> 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).

<sup>15</sup> 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).

<sup>16</sup> 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).

<sup>17</sup> 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).

deficient enzyme in children with Hunter Syndrome, another state relies on a section 1115 waiver<sup>18</sup> to only cover episodic and symptomatic interventions, such as surgeries, hospitalizations, diagnostic imaging, physical therapy, antibiotics, and a nebulizer. In approving a therapy for a specific disease or condition, the scientific experts in the review division at FDA analyze considerable data that demonstrate the safety and efficacy of the drug in the intended patient population. As patient organizations committed to ensuring our patients have access to treatments proven safe and effective by FDA, we believe that it is not the role of a Medicaid plan to refute this analysis or establish its own criteria for what it deems safe and effective for the sole purpose of denying or rationing access to a rare disease therapy.

- **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.<sup>19</sup> 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.<sup>20</sup> 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.

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<sup>18</sup> 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.

<sup>19</sup> See 21 U.S.C.S. §§ 360aa-360ff; 26 U.S.C.S. § 45C (LexisNexis 2014)).

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

**Description of the amendment:** This legislation will clarify that states shall not:

- 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.

**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)).”