February 26, 2015

The Honorable Fred Upton The Honorable Diana DeGette

Chairman Member

Committee on Energy & Commerce Committee on Energy & Commerce

United States House of Representatives United States House of Representatives

Washington, DC 20515 Washington, DC 20515

**Re: Access to 21st 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 *21st 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 *21st 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 *21st Century Cures*, to ensure **“access” to *21st 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 *21st 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:

* **E**quity for individuals with rare diseases and conditions;
* **P**hysician and patient treatment determination;
* **I**nnovation of and access to individualized treatment; and
* **C**ontinuity 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 *21st 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”).[[1]](#footnote-1)

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 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.[[2]](#footnote-2) 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.[[3]](#footnote-3) 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 *21st Century Cures*. Moreover, it is consistent with the innovation goals of the Orphan Drug Act and more than 30 years of subsequent rare disease policy.

The *21st 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 that recognizes the equal importance of both a “path” to and “access” to *21st Century Cures*. Thank you for considering our views.

Sincerely,



Barbara Wedehase, MSW, CGC

Executive Director

National MPS Society

Enclosures

1. 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. [↑](#footnote-ref-1)
2. *See* H.R. Rep. No. 881, 101st Cong., 2d Sess. 96-98 (1990). [↑](#footnote-ref-2)
3. *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. [↑](#footnote-ref-3)