



December 16, 2019

Submitted Electronically to: cures2@mail.house

The Honorable Diana DeGette
United States House of Representatives
2111 Rayburn House Office Building
Washington, DC 20515

The Honorable Fred Upton
United States House of Representatives
2183 Rayburn House Office Building
Washington, DC 20515

RE: Haystack Project input on Cures 2.0 legislation to modernize coverage and access to life-savings cures in the United States and across the globe.

Dear Representatives DeGette and Upton:

Haystack Project appreciates the opportunity to provide input as Congress considers legislation to build upon the success of the 21st Century Cures Act by facilitating a modernized system of developing new cures combined with a health care system that delivers coverage and access.

Haystack Project is a 501(c)(3) non-profit organization enabling rare and ultra-rare disease patient advocacy organizations to coordinate and focus efforts that highlight and address systemic reimbursement obstacles to patient access. Our core mission is to evolve health care payment and delivery systems with an eye toward spurring innovation and quality in care toward effective, accessible treatment options for all Americans. We strive to amplify the patient and caregiver voice in these disease states where unmet need is high and treatment inadequacies can be catastrophic.

The Rare Cancer Policy Coalition (RCPC) is a Haystack Project initiative that brings together rare cancer patient organizations. RCPC gives participants a platform for focusing specifically on systemic reimbursement barriers and emerging landscape changes that impact new product development and treatment access for rare cancer patients. It is the only rare cancer coalition developed just to focus attention on reimbursement, access and value issues across the rare cancer community. Working within the Haystack Project enables RCPC participants and rare and ultra-rare patient advocates to leverage synergies and common goals to optimize advocacy in disease states where unmet need is high and treatment inadequacies can be catastrophic.

While countless lives have been improved, or saved by new therapies enabled by Congress' set of incentives for orphan drugs, 95% of the 7,000 rare diseases identified to date have no FDA-approved treatment option.

- Approximately 50% of the people affected by rare diseases are children;
- 30% of children affected by a rare disease will not live to see their 5th birthday; and
- Approximately half of identified rare diseases do not have a disease-specific advocacy network or organization supporting research and development.

Despite dramatically increased availability of novel treatment options, many patients with rare diseases still face hurdles accessing lifesaving and life-improving FDA-approved therapies.

These hurdles are often related to reimbursement structures such as inadequate bundled payment rates, high cost-sharing and/or payer coverage delays and restrictions on what may be the only treatment available to reduce a patient's disease burden. Exceedingly small populations, long diagnostic journeys, and a limited natural history knowledge base for many rare diseases can also make the development and regulatory processes particularly challenging. Moreover, as reform initiatives increasingly focus on reducing treatment costs, we see an emerging threat that the policies favoring patient access to new treatments such as accelerated approvals, use of surrogate endpoints, and single-arm trials will be nullified by emerging payer requirements for robust data demonstrating long-term "value." We are similarly concerned that drug-pricing reforms will all but close the narrow window for commercial viability of ultra-rare disease treatments. Our sincere hope is that a greater understanding of our experiences will enable pragmatic solutions to existing problems and guide future health system refinements that take our unique needs into account.

The patient advocacy organizations within Haystack Project have identified policy refinements that could even the playing field with that of patients with more common conditions. We welcome the opportunity to discuss these concepts as you move toward devising Cures 2.0 legislation.

Modernizing how new cures and medical products are covered.

- Direct CMS to create an "Extremely Low Claim Volume in Bundled Settings List ("ELCV")" that gets distributed to Medicare participating hospitals through a Program Memorandum. This should remind hospitals that Medicare conditions of participation are at risk if the patients with low-prevalence conditions report inadequate care and include a 1-800 number for patients and providers to be able to report care issues.
 - The payment bundles CMS uses, particularly in the inpatient setting, aggregate "similar" conditions and set an average payment. Extremely rare conditions are grouped in broad categories that can contain hundreds of diseases with different standards of care;

- When an orphan drug is developed to address a very low prevalence condition, inpatient payment levels are uniformly and intractably deficient, and patients have significant access hurdles.
- Require CMS to (i) extend its prohibition on copayment accumulator programs to treatments for extremely low prevalence conditions regardless of generic entry **AND** (2) allow copay assistance for Medicare beneficiaries living with conditions of extremely low prevalence.
 - In very low prevalence conditions, pricing of any generics that come to market remains very high, with little “savings” realized by encouraging generic use;
 - Today, many generic entrants in these disease states decline to offer copay assistance, leaving patients with profound access issues not experienced by other patients;
 - Generics may not offer the same therapeutic benefits to patients. Tubular sclerosis patients (very young children) have experienced significant breakthrough seizures when switched to generic treatment.
- Expand statutory definition of medically accepted use to include compendia-listed uses (and peer-reviewed articles) of treatments for extremely low prevalence conditions.
 - The Medicare statute deems certain uses of anti-cancer treatments as “medically accepted” uses, effectively requiring Medicare to cover anti-cancer treatments not only for their on-label indications, but for off-label indications if those indications were listed in a compendia or there were two or more peer-reviewed articles supporting an off-label use;
 - The circumstances that drove that legislation for oncology all those years ago, is very much the circumstance that patients with ultra-rare conditions find themselves in today. With over 7,000 rare conditions identified, most without treatments today, off-label use is often patients’ only hope.
- Require payers to cover FDA indications per approved label rather than limit coverage to inclusion/exclusion criteria or assume that the FDA boilerplate language on elderly populations, or other special populations, is a statement that the approved drug is somehow “experimental” in these patients.
- Require payers to eliminate any utilization management provisions that require extremely low prevalence condition patients to step through an off-label treatment when a FDA approved/on-label or compendia-listed treatment option exists.
- Congress should close up the incentive for MA plans to use the national coverage process to shift costs of new treatments costing over \$100K. This use of CMS’ process is

costly, burdensome, injects uncertainties that constrict access, and is not what the National Coverage Determination process was designed to accomplish.

- Establish a rare and ultra-rare disease Ombudsman within HHS to ensure that patients are not subject to barriers in accessing meaningful, quality coverage for their unique healthcare needs. This would include access to FDA-approved treatments using the same level of medical necessity inquiry that is applied to commonly-encountered conditions.

Harnessing data to empower patients and improve their health.

- Require a GAO study of how the European system reviews ultra-rare applications and its applicability in the US -- Specifically, how the EU allows submission of updated data during the review, including from open label extension studies for patients who remain/continue on drug or cross-over from a control arm after clinical trial data has been gathered and submitted.
- For any very low prevalence orphan applications, require FDA to consult with patients/patient organizations in devising any REMS elements that require patient action/participation.

Additional initiatives that would improve access for individuals with rare and ultra-rare conditions.

- Establish a uniform mechanism for providers to qualify for accepting Medicaid payments from other state programs. Patients with conditions best addressed by sub-specialists should be able to obtain treatment without incurring additional costs.
- Direct CMS to develop quality measures that capture the long diagnostic journey for patients with rare diseases to reinforce what should go without saying – sick patients deserve to know what is wrong and what, if anything, is available to treat them.
- In conditions of extremely low prevalence, especially with a first treatment to market, payers must not be allowed to delay access as a standard operating procedure. Medicare and Medicaid often delay access to new drugs until formulary decisions are made, and several commercial payers (CVS Caremark/United) now systematically block coverage of new drugs so that they can review and determine coverage. If medical necessity documentation is needed, forms should be uniform, decisions should be made quickly, and there should be a minimal burden on providers.
- Consider a 340B covered drug exemption for drugs and biologics treating very low prevalence conditions. Manufacturers of these products can have extremely high, and highly variable 340B exposure that can threaten commercial viability and deter future investment in innovation.
- Require a rare or ultra-rare expert in the science of small population studies at Advisory Committee meetings when the application under review is for a low prevalence condition.

- Require annual report to Congress that sets out, by division, how many rare applications were reviewed, Agency actions, and the prevalence #s for that rare condition (this could be pulled from sponsor submission on orphan designation request.)
- Require a review division to include Rare Disease Program staff in their review team when reviewing a first drug/biologic or a first disease modifying agent for a particular indication associated with an orphan condition with very low prevalence. This same Rare disease program staff support should be extended to support review division decisions beyond just approval to REMS, post market commitments, etc.,.

Once again, we appreciate the opportunity to provide input as you consider next steps towards Cures 2.0 legislation. We are encouraged by your stakeholder-inclusive approach and hope to have the opportunity to discuss the unique circumstances patients with very rare conditions face in finding and accessing effective treatment options.

As the voice of rare and ultra-rare disease stakeholders that care deeply about learning and educating about reimbursement and patient access issues, we look forward to working with you on these proposals, which have been gathered directly from patient groups that participate with Haystack Project. We hope to discuss these in more detail with you in person and will reach out to schedule a meeting, but if in the meantime, you have any questions or would like to discuss our comments and recommendations, please contact Saira Sultan at 202-360-9985.

Respectfully submitted,

Saira Sultan, J.D.
Haystack Project, Inc.





Rare Trait Hope Fund



Fighting for Kaiden Foundation

