Access to Rare Indications Act

**Legislation.** The Access to Rare Indications Act (H.R. 8467 in the last Congress) is designed to ensure that individuals with very rare conditions have the intended benefit of health coverage that individuals with more common conditions, and even those with more prevalent orphan diseases, have today — access to health care meeting the standard of care to treat or manage their medical condition(s). For these patients:

- There is often no FDA-approved option addressing their rare condition, and disease symptoms are frequently addressed with off-label use of existing treatments and evolving standards of care derived from a limited set of clinicians and researchers with highly specific expertise;
- Disease course is often progressive and frequently involves increasing disease burden and loss of function and/or death;
- When new treatment options offer reduction in disease burden, slowed progression, or cure, timely access is essential to relieve disease burden and can even be determinative of long-term prognosis.

**Problem.** Existing laws, regulations, and payer practices designed to balance patient access to medically necessary care with fiscal conservatism have enabled most U.S. patients to receive the medical care they need without undue burden on their providers. For individuals with very rare disorders, however, these mechanisms can:

- drive insurmountable access burdens for the vast majority of patients unable to receive care from the handful of specialists focused on their condition;
- present unnecessarily poor health outcomes through delays in access to new treatment options;
- subject patients to step therapy protocols that expose them to drugs that could be both ineffective and harmful; and
- significantly deter, or even prohibit coverage of off-label treatment that are not only the standard of care, but are the only available treatment(s) for their condition.

**Solution.** The Access to Rare Indications Act was narrowly tailored to address the specific hurdles these patients face by:

- appending the definition of medically accepted indication to include sources in which medically accepted indications for very rare diseases can be found. Off-label uses of existing products in very rare conditions are generally not included in the various compendia and other sources outlined in Medicare Part D, Medicaid, and the private health insurance market;
  - for **Medicare Part D** patients, this is needed to remove an unfair statutory prohibition on coverage of off-label uses that are not listed in the limited set of compendia. Today, if Part D plans look to clinical guidelines and standards of care published in peer reviewed literature for very rare conditions not addressed in compendia, the Plan would be violating the statute and could face program integrity scrutiny and compliance investigations.
  - In **Medicaid** and **private health plans**, clearly identifying the relevant sources for the standard of care in very rare diseases would permit patients to avoid potentially harmful step protocols and access denials, and would reduce the already-high burden their clinicians face;
- creating a mechanism through which individuals with very rare conditions can access an expedited appeals mechanism to receive coverage for new treatments that have not been reviewed for formulary inclusion or off-label uses that are not included within formulary.

This is not a coverage mandate, and applies only to individuals with these conditions, and not broadly to all uses of the drugs or biologicals.

**Example 1.** Pemphigus is a rare group of blistering autoimmune diseases that affect the skin and mucous membranes. The mainstay of treatment is corticosteroids that also suppress the normal function of the immune system. While not usually fatal, patients with uncontrolled/untreated pemphigus can die from opportunistic infections. Several studies completed before 2007 noted clear benefits of Rituximab in a subset of patients with refractory disease and severe adverse effects from long-term steroid therapy. Patients who may have found relief from Rituximab were unable to access it through Medicare or other insurers for over a decade until FDA approved Rituxan for pemphigus in 2018, and it is now considered a first-line therapy.

**Example 2.** Tuberous sclerosis complex. The most severe cases of denials based on prior authorizations were for children between the ages of 2 and 9 (outside of label). The treatment was labeled for 1 month to 2 years of age for infantile spasms and ages 10+ for complex partial seizures. The patient group finally approached Lundbeck in Spring 2018 about applying for label change to FDA using global guidance for extrapolation of complex partial seizures. The group supported the collection of supplemental information from 200 patient chart reviews and in 2019 submitted with Lundbeck to the FDA. FDA approved the label change in January 2020, but the impact on these children and their families was needless suffering that is hard to accept.

**Example 3.** Cutaneous lymphoma -- Off-Label use of interferon, topical steroids and other drugs to treat skin conditions is frequent and quite helpful in managing the disease. The increase in prices for these drugs or lack of access to them because an insurer will not cover it due to being off-label, creates limitations for effective treatment for some patients as there may be no alternatives suitable to managing their form of the disease. Additionally, these treatments are often used in combination with other FDA approved treatments making the FDA approved treatment more effective or supporting the effectiveness of the FDA approved treatment.

Managing all of this can be quite challenging for a patient attempting to live as best they can with a rare disease and navigate the complexity of the healthcare system.

**Example 4:** Payers may also mine clinical trial protocols to condition access to new treatments on tests or procedures employed within the trial but not necessary to identify or treat appropriate patients. In one case, the clinical trial required a biopsy of tissue for amyloid for confirmation of HATTR Amyloidosis. Upon approval of the drug, the manufacturer made a genetic confirmation test available for free to patients, so that a biopsy would be unnecessary. Nonetheless, some payers put a PA for a biopsy in place, not only incurring a cost to the health care system, but subjecting patients to needlessly invasive procedures that compound the burden with unnecessary out-of-pocket costs. Such biopsies are not readily available, for example in rural areas, nor are pathologists always able to translate biopsy results easily outside of academic centers. This wasn’t required by the FDA or the label, but the payer used the clinical trial criteria to deny or delay access.

Jim Caro  
CEO  
Haystack Project  
Jim.caro@haystackproject.org  
www.haystackproject.org