

The Access to Rare Indications Act could be a game changer for millions of Americans

By Darcy Krueger and Emanuel Maverakis

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The term “medically necessary” is the yardstick by which insurance companies, including Medicare and Medicaid, decide if they will pay for a particular treatment. For the millions of Americans living with rare diseases, most of which do not have FDA-approved treatments, identifying a treatment as medically necessary can be a lifeline — or the end of the line.

A [white paper](#) released in April by the influential Institute for Clinical and Economic Review offers several misguided proposals that attempt to balance public interest in incentivizing innovation with payer cost containment goals in rare diseases. The proposals are unlikely to benefit people living with rare diseases, clinicians like us who care for them, or those trying to bring rare treatments to market.

A far more thoughtful approach is embodied in the Access to Rare Indications Act ([H.R. 6160](#)), introduced in the House of Representatives by Reps. Doris Matsui (D-Calif.), Mike Thompson (D-Calif.), Mike Kelly (R-Penn.) and Markwayne Mullin (R-Okla.). The bill recognizes that the vast majority of people with rare diseases don’t have an FDA-approved treatment, meaning their

disease is not listed on the treatment's label, and their clinicians shouldn't have to struggle to convince payers that medically accepted off-label treatments are medically necessary. Specifically, this bill seeks to give people with rare diseases the same level of insurance coverage for off-label treatments as patients with "common" conditions such as cancer and insomnia.

A drug's [label](#) includes copious information about its chemical composition, dosing instructions, and what conditions it has been approved to treat. When a clinician prescribes a treatment for an off-label use — one not listed on the label — an insurer will make a coverage decision based on special medical references known as compendia. These include drug information summaries that are compiled and updated by experts who have reviewed clinical data on drugs. This seems to work well for common conditions like depression and fibromyalgia. The National Comprehensive Cancer Network (NCCN) [created a compendium](#) focused solely on cancer treatments, since off-label use is common in oncology, and because [Congress has ensured](#) that cancer patients covered by Medicare and Medicaid have access to promising off-label uses not yet incorporated into compendia but that are supported in published peer-reviewed articles.

Off-label use is often the only treatment option for people with rare diseases, much as it is for people with cancer. However, there are no rare-disease compendia to track off-label treatment protocols because rare disease mechanisms are so diverse and the variety of clinician specialties that treat them will never support the kind of compendia oncologists rely on. Treatment decisions that we and other physicians caring for people with rare diseases make are based on clinical guidelines, published research, and disease-specific expert consultation. But the resources we use to make treatment decisions are generally ignored by payers, who decide that the off-label treatments we prescribe are not medically necessary.

Just as insurers look for evidence of medically necessary cancer treatments in sources that contain up-to-date and reliable information, they should look for evidence on rare disease treatments in sources where that information exists:

- peer-reviewed journal articles for defining medical necessity — avoiding unreliable [predatory journals](#)
- clinical guidelines, which will increase over time as insurers indicate they will use them
- clinicians like us who are recognized by our specialty societies as having expertise in specific rare conditions

Many of our patients need this legislation, which merely extends to rare disease patients the precedent set for cancer patients many years ago. To illustrate the point, we share Marc Yale's story, since he has [talked publicly](#) about his experience. Marc lives with [mucous membrane pemphigoid](#), a rare, life-threatening autoimmune disease that causes blistering lesions throughout the body. He fought with his health insurer for two years to get the right treatment — a drug that is the standard of care for his very rare condition. He was told that the treatment his doctors prescribed was off-label, experimental, and not on any compendia, and his insurer insisted that he use other medications instead. While Marc fought with his insurer, he lost his eyesight, had to stop working, and was hospitalized. The financial and emotional burden of not only the disease but getting the wrong treatment was enormous.

People like Marc can get the treatment they need if they can find highly specialized clinicians working in institutions with the resources to push through prior authorizations, reconsiderations, and appeals. The two of us take time to fight insurance companies' efforts to insist that patients try cheaper alternatives first, which can be onerous when they are ineffective or can even be harmful. Educating payers, convincing their medical review staff of the evidence supporting off-label use, and demonstrating the ineffectiveness of cheaper alternatives does not save us time on the next such patient. The cycle begins all over again for every one of our patients, even when we are dealing with the same insurance company.

Patients living in rural and underserved areas and those unable to find specialists and subspecialists, or who do not have the means or ability to travel to them, have an even more difficult time getting treatment and insurance coverage.

Worse yet, if an individual is a Medicare beneficiary and the treatment is an oral or self-injectable product and so covered under [Medicare Part D](#), there is no coverage at all for drugs being used off-label for uses not listed in compendia. Individuals who cannot afford to pay for the treatment often have little choice other than to simply give up.

People should not have to live through experiences like Marc Yale's just because the diverse set of rare diseases — there are an estimated [7,000 of them](#) — will never support the kind of compendia that oncologists rely on. We learned of H.R. 6160 from Haystack Project, which developed the proposal and gained the support of [more than 60 patient organizations](#). We support the bill because we recognize that people living with rare diseases can't take for granted what those with more common conditions have — coverage for treatments they need. By ensuring that payers look for “medically necessary” treatments for rare diseases in sources that actually contain accepted off-label uses for these diseases, patients could avoid ineffective medications and spend more time with their families and on their jobs instead of fighting with insurance companies. It would also save their caregivers and doctors endless hours of uncompensated time dealing with insurers.

It will take federal legislation to apply the medical necessity standard equitably so rare disease patients have the same level of coverage for treatments that is available to everyone else. We call on Congress to begin that work.

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