

Importance of Access to Rare Indications Act

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Coming off the high of another inspiring Rare Disease Week, we reflected on all the patients we met, the rare disease experts doing amazing work around the country, the policy issues we encountered and the change we advocated for as a community. We each bring different perspectives to the challenges we see for rare disease patients, and yet we came together on a panel late last month and found lots of common ground on the arcane but important topic of 'medical necessity.'

Medical necessity is the yardstick by which insurance companies, as well as Medicare and Medicaid, decide if they will pay for something patients are prescribed. They typically look to a drug's label or sometimes a compendia (a compilation of evidence-based off label treatment options). This seems to work for the common conditions pretty well. It also works well in most cancers since we have a robust compendium for oncology because Congress acted many years ago to address the same problem we are seeing now in rare. About half of all chemotherapy drugs prescribed today are off-label. The rigorously monitored and updated NCCN compendia is the gold standard.

Off-label prescription is even more important in treating people with rare disorders, because there are often no approved drugs for one of these diseases.

However, each of us experiences every day in our own practices, the lack of on-label treatment options for the vast majority of really rare disease patients and conditions we treat. Without a similarly robust compendium for these conditions, we are left to research journals, clinical guidelines, and to consult with the one or two experts in the disease. As such experts ourselves, in this case in the conditions like pemphigus and pemphigoid and tuberous sclerosis, patients are often referred to us or we are asked for consults.

Not all doctors are dedicated to solving the puzzle a rare patient presents, or able to spend hours on with insurance companies. Often these doctors can't be found in rural areas or in health care deserts. Not all patients are well enough to take on the fight themselves either, and both patient and physician often give up.

Compounding the work of being both the patient and the physician is that each case, however laboriously won, sets no precedent for the patient who comes after. Each case, each time, regardless of the similarity of condition, state, or insurer. The hill must be climbed once more, knowing that it will do nothing to save time and anxiety for the next patient and physician.

What does this mean for patients? Fellow panelist, Marc Yale, told of his own journey through the insurance maze to access off label treatments. The story was all too familiar – and yet, it doesn't have to be.

If insurers looked to the sources where 'medical necessity' can be found for rare conditions, we could save doctors and patients from endless hours of uncompensated time, and time our patients could spend on their jobs, with their families, and reducing the burden on their caregivers. There is little point in celebrating new breakthroughs, new science, new insights into conditions so poorly understood for so long, if they can't reach patients.

Insurers can and should look to peer reviewed journal articles for defining medical necessity. Not all journals are discerning and lists of predatory journals are shared among practitioners, who know which journals to trust. Congress incorporated the need to look to journals for medical necessity for cancer many years ago, and it is time to do the same for rare conditions. The Centers of Medicare and Medicaid Services (CMS) promulgated regulations identifying specific journals and appropriate criteria for which journals should be used.

Insurers can also look to clinical guidelines. There aren't enough such guidelines available today, but as we see insurers using them, experts will work even harder to develop and publish them.

And finally, insurers should look to the experts, the handful of us who dedicate our careers to individual rare conditions. Such experts are few enough and not difficult to identify if there were a reason to do so. Medical societies will become an important source for identifying experts.

It will take Federal legislation to turn the tide on applying the medical necessity standard, just as it did many years ago in oncology. The opportunity to debate and discuss how to implement such a change during this year's Rare Disease Week left us eager to do more than talk about this.

It is too easy and short-sighted to get bogged down in the criticisms and hurdles of why an approach like this may not work. It takes the conviction that comes from seeing patients and their physicians struggle every day to get medicines to the patients who need them to know that we must do more. We must try. The old adage about the perfect being the enemy of the good applies here. We didn't know the Orphan Drug Act would work when we passed it. We didn't know the medical necessity exception for cancer would work when we passed it. We saw a problem and sought to address it thoughtfully but with the promise that we would start with a framework that could be revised annually, as CMS does with so many programs, to ensure a good result and few unintended consequences.

We call on Congress to begin.

Marc's Story

Marc's story makes a compelling case for our collective efforts. Marc was diagnosed with mucous membrane [pemphigoid](#), a rare, life-threatening disease. One of the defining characteristics of pemphigoid is blistering lesions that can spread throughout the body. Pemphigoid is not contagious, but rather an auto-immune disorder. Because his disease is rare, it took Marc two years to get the right treatment. In the meantime, he was put on a high dose of corticosteroids to reduce the blisters, as well as other drugs that were ultimately ineffective. While Marc was trying to get the standard of care treatment for his disease approved by his insurance company, he was on other medications. He was told the drugs were off-label, experimental and not on any compendia. During this time, he lost his eyesight, his career, and was hospitalized. The financial and emotional burden of not only the disease, but the wrong treatment, was enormous. Marc is now on the standard of care for pemphigoid, and is stable.

The best treatment is the one that doctors and patients decide on, guided by the latest scientific and medical information. However, these treatments aren't always covered. In fact, for patients on Medicare, the Medicare Part D [statute](#) limits the definition of medically necessary care to those on the drug label of included in compendia. Patients who are prescribed a less than optimal medication because it is what is covered have suffered irreversible harm, including the loss of function, loss of income and disease progression. Often, we have seen that insurers reject coverage of medication because they deem it "experimental" for the indication, or the medicine is off-label and off-compendia. But rare diseases don't usually have compendia - there are no professional or patient organizations with the capacity to shepherd the process and keep compendia constantly updated.

In tertiary settings - where we focus on specialties and sub-specialties - we have fellows and support staff to help our patients navigate the insurance process, and help with appeals. We can only do this for patients who have private health insurance or are on Medicaid; not Medicare. For patients who do not have access to specialists and subspecialists, treatment and insurance coverage may be even more fraught. Patients, and providers, spend an inordinate amount of time dealing with insurance - that's time they aren't getting treatment or living their lives.

Marc spent years getting the right treatment, but his journey set no precedent for other patients with his disease, regardless of insurance coverage. Each patient with a rare disease, and their providers, has to fight for the same treatment that is already medically appropriate, and may have already been paid for by insurance for another patient.

That is why we, along with [more than 60](#) patient organizations, support the Access to Rare Indications Act which builds on existing structures to improve coverage for people with rare diseases. Just like cancer treatment is paid for per the compendia, we think that treatment for rare diseases should be based on evidence - whether that is in guidelines or published studies. Rather than coverage policies dictating what patients get, we must lead with science.