



Alliance for
Patient Access



Addressing Access Barriers for IgG4-RD Patients

A White Paper from the IgG4-RD Initiative
of AfPA's Rare Diseases Working Group

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About IgG4-RD

A rare condition known as IgG4-related disease, or IgG4-RD, can burden patients with a complex web of symptoms, trouble obtaining a diagnosis and a seemingly endless series of treatment barriers. Many patients experience significant emotional and financial impacts too.

IgG4-RD is an immune-mediated fibroinflammatory disorder. It is often caused by abnormal accumulation of immune cells that produce IgG4 in various organs.

IgG4-RD disrupts the immune system and causes tumor-like masses, enlargement and other damage to organs. The disease can lead to pancreatitis, swollen glands, liver failure and liver disease. It can also cause substantial organ damage and organ failure. IgG4-RD can manifest in different ways, and its most obvious signs are often confused with symptoms of other, more common diseases.

If caught early, the disease may be managed. Health care providers have been using medications off label to help patients manage symptoms for years, but innovative FDA-approved treatments developed specifically for IgG4-RD are on the way. The path to treatment, however, can be long and bumpy.^{1,2}

Challenges In Diagnosis

Early diagnosis is crucial to effectively managing IgG4-RD.

Yet the disease often develops for months or even years before a patient experiences any symptoms. Even then, getting a diagnosis can be tricky given the spectrum of symptoms. The disease is also rare and has been identified only relatively recently, making diagnosis still harder. Many clinicians don't have much information about the condition, let alone enough to make a diagnosis.

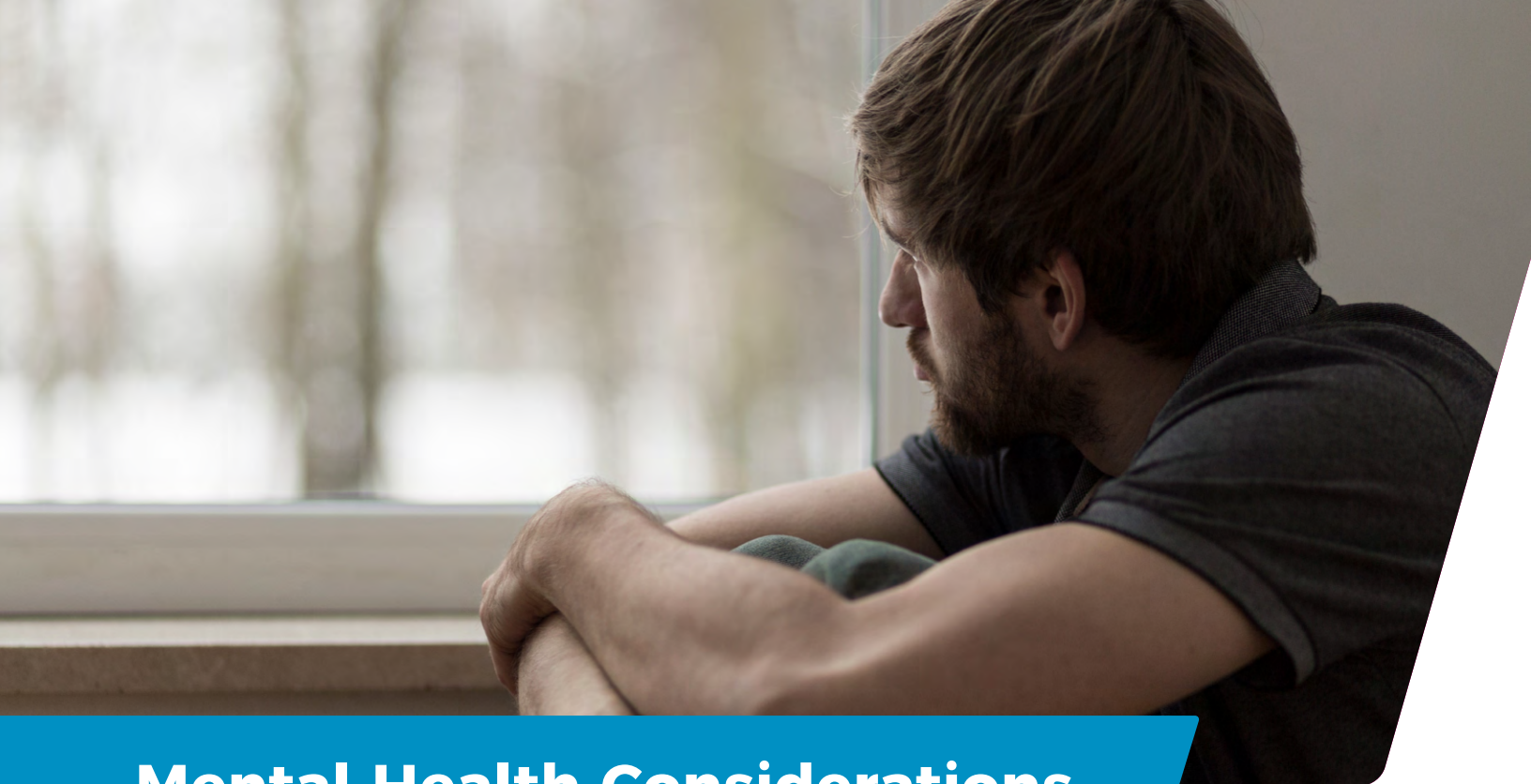
IgG4-RD symptoms are often misdiagnosed. Tumor growth or masses within organs, for example, are easily confused as a sign of several types of cancers. Some patients even undergo surgery, radiation therapy or chemotherapy, all designed to treat cancer. Ultimately, these bring them no relief, and the organ damage continues. Meanwhile, other common symptoms – headaches, weight loss, inflammation, shortness of breath, brain fog, double vision and urinary difficulties – make patients miserable and confound clinicians.¹

The wide variety of symptoms can lead patients to see several different medical specialists. The many appointments put a strain on patients and caregivers, while the out-of-pocket costs and travel expenses mount. Meanwhile, few health care providers, even specialists, can recognize IgG4-RD.



Determined patients and committed clinicians may look to lab tests and radiology studies to identify the source of the symptoms. But red tape often blocks access to these procedures. Histopathological exams, lab tests, biopsies, imaging, specialist visits and other diagnostic tools needed to identify IgG4-RD may not be covered by health plans. Initial blood tests may be covered by insurance, but acquiring coverage for PET scans, additional diagnostic tools and IgG4-RD treatment can be challenging.

When patients do receive an IgG4-RD diagnosis, they often experience conflicting emotions. On the one hand, they finally can put a name to their struggle. On the other, they must now battle a rare disease that has no cure and no approved treatments.



Mental Health Considerations

Because IgG4-RD has been recognized by the medical community only recently, patients don't have many of the support structures that are available for people with other debilitating conditions.

For instance, the IgG4-RD patient community has until recently lacked a formal support group, which allows patients to connect with others who understand their experiences and to learn more about their condition. Because of the broader lack of knowledge about IgG4-RD, employers may lack the information or empathy to accommodate IgG4-RD and its symptoms. As a result, patients may feel lonely and isolated, as though no one around them understands their condition. They may feel helpless as their physical health declines.

Because IgG4-RD is often identified after a long and draining diagnosis process, patients may already feel emotionally, physically and financially depleted by the time treatment begins. These negative mental health experiences do not create ideal conditions for healing and may further complicate patients' compliance with treatment.

Physical symptoms and incorrect diagnoses can also contribute to mental health struggles. For example, patients who learn that they have tumors must withstand the agony of wondering whether they have cancer. In other cases, symptoms may disrupt patients' day-to-day lives, intensifying their feelings of isolation.

Access to mental health support is a crucial component of disease management for people living with IgG4-RD.

Treatment Challenges



Limited Treatment Options

The FDA has not yet approved a treatment specifically for IgG4-RD.

Glucocorticoids, steroids that reduce inflammation, are the most widely prescribed treatment for IgG4-RD.³ Another medication called rituximab is also commonly used to treat the disease. Other off-label treatments are available for patients who do not respond to this treatment or who quickly relapse.

While these treatments work at least temporarily in many patients, the medications can introduce side effects that some patients simply cannot tolerate – underscoring the need for new treatment options developed specifically for those with IgG4-RD. And insurance companies don't always cover the off-label use of treatments, like rituximab, that are typically used to treat IgG4-RD. Patients may face difficult financial choices and high costs of care at the same time that they may be less able to work or require increasing care.

Physician Availability & Knowledge Gaps

Most health care providers don't fully understand IgG4-RD, and most patients encounter detours or dead ends on the path to diagnosis. Patients may wait weeks or months to see specialists. Some may be told that their numerous symptoms are unrelated. Because of the wide variety in initial symptoms, patients often begin their search with multiple specialists, including gastroenterologists, ophthalmologists, enterologists, ENT specialists, allergists, neurologists or others. It is most often rheumatologists who finally identify IgG4-RD.

As patients search for a diagnosis and treatment, they may struggle to find the right provider or specialist. Physician burnout and specialist shortages limit whom patients can see. These challenges impact not only IgG4-RD patients but also other patients throughout the health care system.

The good news is that clinical knowledge of IgG4-RD is evolving and expanding. That knowledge, however, is not evenly distributed geographically. IgG4-RD specialists are often concentrated in large cities or academic medical centers. As a result, patients who do find a provider may struggle to meet with him or her as often as testing and treatment require.

Utilization Management

As new FDA-approved treatments for IgG4-RD become available, patients are likely to face insurance cost-cutting barriers that often block access to innovative and targeted medications. This includes utilization management, which can lead to long waits, higher costs and poorer health outcomes for patients with IgG4-RD.



New-to-Market Exclusions

In an attempt to control costs, some health plans postpone coverage for medications that have just come to market. They may delay coverage for a period of months or even wait to add the drug to the formulary of covered medications at the start of the next calendar year.

Exclusions are especially unfair to patients with rare diseases, who have often had to wait for months or years already just to get a diagnosis. The health plan's delay adds insult to injury – and allows the patient's disease to progress despite the fact that safe and effective treatment has been approved by the FDA.



Prior Authorization

Many health plans require prior authorization before they will cover a prescribed treatment or diagnostic. As a result, the few clinicians who can treat IgG4-RD patients must take time away from seeing patients to comply with insurer requirements.

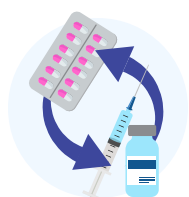
Badly needed treatment can be delayed, especially when the authorization process breaks down. For patients with a progressive disease, wasted weeks or even months off an effective treatment can be devastating.



Step Therapy

Step therapy is a cost-cutting process by which insurers require patients to try older, lower-cost treatments before they can access a newer or more expensive one. Because this process can take weeks or months, patients may see symptoms worsen and watch their disease progress while they are denied access to the medication their clinician prescribed.

A medication that doesn't work may still introduce significant side effects. Step therapy is another way treatment is often delayed, allowing symptoms to worsen and undermining quality of life.



Non-Medical Switching

Once patients start to see results from their prescribed course of treatment, they may feel some relief and hope about their future with IgG4-RD. That relief can evaporate if their health plan then compels them to switch from their current medication to an option that's less expensive for the insurer. This “non-medical switching” effectively puts the insurer, instead of the clinicians, in charge of medical decision-making for a rare and serious illness.



Copay Accumulator Adjustment Programs

Many patients rely on copay cards to offset the high costs of prescription drugs and to pay down their annual insurance deductible. In recent years, however, some health plans have changed their rules to no longer count a copay card's full value toward the deductible. These "copay accumulator adjustment programs" can leave patients with unexpected bills or huge upfront deductibles when they exhaust the balance of their copay card.

Faced with difficult financial choices, some patients diverge from their treatment regimen or forego treatment altogether. This decision can have a potentially devastating impact on patients' health.

This barrier, however, has drawn policymakers' attention. In October 2023, a federal court reversed the ruling that allowed copay accumulator programs. While policymakers and the courts have made progress addressing accumulator programs, they still pose a threat to patients.



Telehealth Barriers

Patients increasingly can access rare disease specialists without having to travel to those clinicians' offices in person. Telehealth, which became much more accessible and sophisticated during the COVID-19 pandemic, has been especially helpful for patients with rare conditions like IgG4-RD, who may not have a specialist that understands IgG4-RD nearby or even in their state.

Accessing services from home may be a gateway to care that would otherwise be out of reach, especially given the rarity of specialists. Sometimes, however, arbitrary restrictions can limit which health care providers a patient can visit virtually or which type of visit the health plan will cover. Patients may travel out of state for an initial consultation with a specialist, hoping to do follow-up appointments via telehealth. But telehealth restrictions often prevent providers from seeing patients across state lines, which can hinder optimal care.

Value Considerations

Imprecise or incomplete definitions of health care value can also impact IgG4-RD patients' ability to access timely care.

Insurers and pharmacy benefit managers frequently use health economics data to make coverage and reimbursement decisions. The result is that innovative treatments may be off-limits to those who need them most.

Health technology assessments, such as those conducted by the Institute for Clinical and Economic Review, sometimes take a population health view of value, focusing more on the health care system than the needs of individual patients and their health care providers. Typically, one of the key metrics for evaluating medications is the quality-adjusted life year – which gives a higher value to healthy patients and discriminates against those who are chronically ill.⁴

Patients with poorly understood conditions are at a particular disadvantage in these discussions, at the same time that their future access to treatment is on the line.

More direct engagement and a more patient-centered conversation with groups who utilize health technology assessments could encourage insurers and government entities to consider a more comprehensive, realistic and human definition of value.

Overcoming Treatment Challenges

The barriers that prevent seriously ill people from accessing the care they need create suffering for both patients and their families. Meanwhile, burdensome administrative tasks tie up clinicians who should be focused on patient care.

Lawmakers should minimize these practices, which make it more difficult for very sick patients to access quality care.

Conclusion

Patients with rare diseases such as IgG4-RD already face huge hurdles: treating their disease while maintaining their independence, quality of life and financial well-being. Bottlenecking timely and valuable treatment makes their situation worse – and unnecessarily so.

But innovation is opening doors, and it's an exciting time for many patients. Research into IgG4-RD and other autoimmune conditions stands to shorten the time to diagnosis and to improve available treatments. In the meantime, better coverage policies and a more patient-centered definition of health care value can empower clinicians to treat symptoms promptly, giving patients a better quality of life now.



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