HEALTH SYSTEM BARRIERS FOR AMYLOIDOSIS PATIENTS

A WHITE PAPER FROM THE **RARE DISEASES** WORKING GROUP

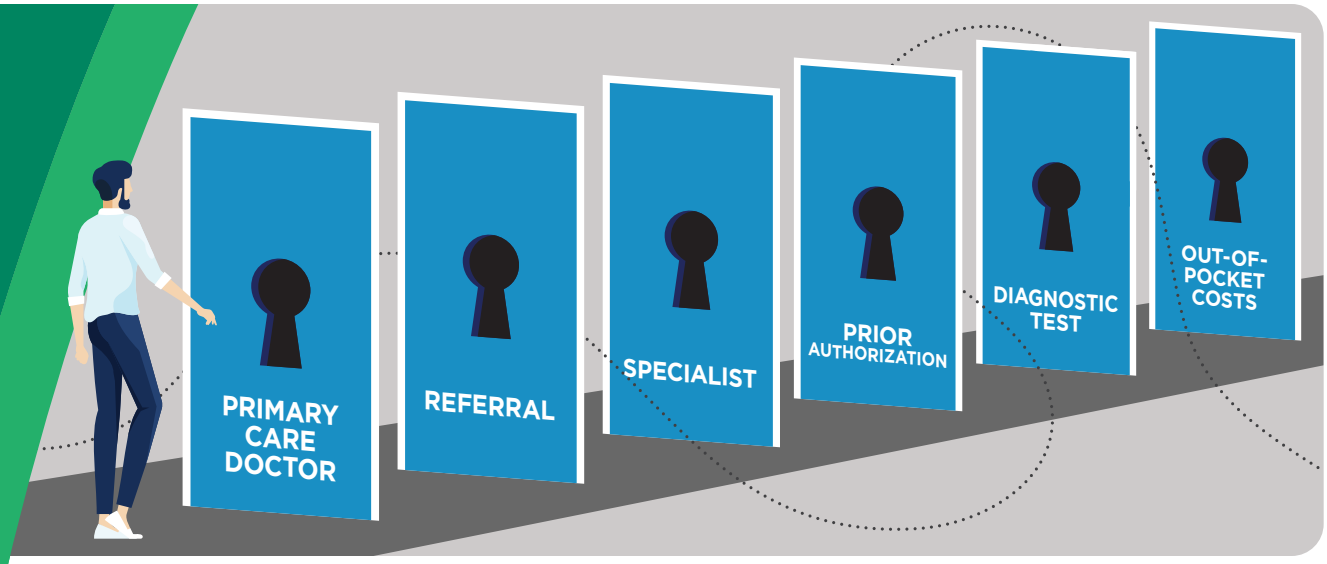
CONTENTS*

Rare diseases present unique challenges, often beginning with diagnosis. Symptoms may puzzle patients and even leave health care providers uncertain. Diagnostic tests and tools may be difficult to access, buried behind health plan red tape. For patients who do finally find answers, treatment may be out of reach.

These challenges ring true for a rare disease called amyloidosis, caused by a build-up of abnormal protein in different bodily organs. Depending upon how the disease manifests, patients can experience debilitating symptoms like dizziness and nerve tingling, nausea and vomiting, extreme pain, undetectable blood pressure, kidney damage, swollen legs or shortness of breath. As symptoms worsen, patients become less mobile and more dependent on the care of loved ones. Progressive and multifaceted, amyloidosis can levy a tremendous burden on patients and their families.

ABOUT TRANSTHYRETIN AMYLOIDOSIS

There are several types of amyloidosis. This white paper addresses transthyretin amyloidosis, which can be hereditary. The disease's symptoms may appear unrelated at first. They are often confused for other conditions. They may manifest at different times and can vary depending on which type of amyloidosis a patient has. Because no single, easy diagnostic test exists, patients can go for years without a



diagnosis or even get misdiagnosed. The condition worsens with time, leaving patients to experience effects on their nervous, cardiac, renal and digestive systems, among others.

Yet the outlook for patients with transthyretin amyloidosis is much brighter than that of some other rare disease patients. After years with no treatment options, patients with this type of amyloidosis now have three FDA-approved treatments. They vary in mode of delivery – oral, injection or infusion – and in which presentation of amyloidosis they treat. But all have the potential to significantly improve patients' lives, if patients are able to access them. Health care system complexities, coverage barriers and high out-of-pocket costs, however, can make accessing treatment difficult.

THE STRUGGLE FOR DIAGNOSIS AND TREATMENT

America's health care system can work well for everyday people with everyday aliments. But health plan policies can present challenges to patient-centered care for rare conditions like amyloidosis. There are access barriers at nearly every turn.

Health plans often require patients' first point of contact to be a primary care doctor. These family clinicians are adept at diagnosing common complications such as ear infections and establishing routine treatment of, for example, high blood pressure. But they may not connect seemingly unrelated amyloidosis symptoms. Patients may need to see a specialist, or several, before they can gain an amyloidosis diagnosis. Insurance may require a referral for each one.

Making matters worse, insurers may also require prior authorization or impose other coverage barriers for necessary diagnostic tests. Finding answers is important, but the many steps and delays in accessing critical testing is difficult for patients and their families.

Once patients have a diagnosis, other insurance barriers can delay

"Getting insurance approval for these new medications can be so challenging that, for some patients, enrolling in a clinical trial is the easiest way to get treatment."

Robert Gottlieb, MD, PhD

access to treatment for weeks or months. Commercial plans frequently provide better coverage for one amyloidosis medication than for another, requiring additional paperwork or posing onerous barriers for patients who need a different treatment.

Patients covered through Medicare or by the Veterans Health Administration also face challenges. Some depend upon independent patient assistance programs to help cover the high cost of treatment, though navigating these programs can be difficult. Those who can't secure financial assistance may face out-of-pocket costs that run thousands of dollars each month.

These patients' medication expenses can rival the cost of another mortgage payment—or more—each month. Patients who live near an academic medical center may enroll in a clinical trial to access treatment without the high out-of-pocket costs. Others, however, may simply forego their medication.

VETERANS HEALTH ADMINISTRATION CHALLENGES

Patients covered by the Veterans Health Administration may find it challenging to access treatment for amyloidosis. Hereditary transthyretin amyloidosis manifests primarily in one of two ways, through cardiac

complications or through neurologic complications. The three available medications treat different variations of the disease. Yet the VA's coverage policies can make it difficult for health care providers to match patients with the medication that's right for them.

For a patient with insurance through the VA, the oral medication for cardiac amyloidosis is the most accessible. But coverage is not consistent with the FDA's approved dosage of the medication. Some patients can access the treatment only in the lower dosage determined by their VA, though dosage varies from region to region.

The other two treatments, which are both for neurologic amyloidosis, are not on the VA's formulary of approved drugs. In other words, accessing these treatments is possible but can be complicated. To access the infused treatment for neurologic amyloidosis, for example, patients must first try and fail on the oral medication. Patients must undergo this process, known as step therapy, even though the oral medication is not FDA approved to treat their neurologic variation of the disease. Patients can also gain access to the infused treatment by demonstrating that they are allergic to the oral medication.

To access the injected medication, a patient must have his or her clinician undertake a burdensome multi-step paperwork process to secure VA coverage. This involves working alongside the VA site pharmacist to submit what's known as a non-formulary request.

These barriers run counter to patient-centered care. They are time-consuming for clinicians and make it difficult for patients to access the treatment that is best for them. Above all, they can cause harm. Patients do not regain the function lost while waiting for approval or while trying and failing on a medication that doesn't adequately treat their condition.

"Inconsistent VA coverage sometimes leads clinicians to suggest that patients travel to a different VA location for amyloidosis treatment. But not everyone can travel. And the trend of receiving patients from across the state may burden VA locations that have more favorable coverage policies."

Marcus Urey, MD

CHALLENGES WITH MEDICARE COVERAGE

Medicare is often more willing than other insurers to approve patients' use of innovative amyloidosis treatments. Yet Medicare's siloed structure and cost-sharing policies can keep patients from accessing their approved medication.

Patients covered by Medicare are often required to pay their entire deductible at one time because the monthly out-of-pocket cost for their medicine exceeds that amount. Most commercial plans accept manufacturers' co-pay coupons, which help patients meet their annual deductible without paying it all out of pocket. Medicare, however, does not permit their use. Medicare's limits on the use of co-pay coupons is detrimental to patients who don't have the ability to pay their annual deductible all at once. And the annual deductible is just the first of many out-of-pocket costs for patients.

Medicare Part D's approach to coinsurance requires patients to pay between 5% and 25% of their medication's list price, depending on which phase of coverage they are in. This ends up being a few dollars for most generics, but it can be thousands of dollars for a new, rare disease treatment.

In addition to Medicare's onerous cost sharing requirements, the program is slow to adjust its coverage policies to keep up with recent therapy approvals. For example, Medicare doesn't cover home infusion of the new amyloidosis medication – even though it covers home infusion for more than 35 other medications. Instead, advanced-stage amyloidosis patients must travel to a hospital or an outpatient center to receive the treatment. This is yet another obstacle that inhibits some patients' ability to access or continue treatment.

"Patients with coverage through Medicare who are treated where I work have a co-insurance payment of up to $4,000 for a 28-day supply—a cost they must pay entirely out of pocket. Patients with commercial coverage may be required to pay a similar amount, but they can offset their out-of-pocket costs with co-pay coupons and support from patient assistance programs."

Nitasha Sarswat, MD

CONCLUSION

Transthyretin amyloidosis is one of the few rare diseases that has FDA-approved treatments, yet health systems and policies inhibit patients from a timely diagnosis and access to medications that could slow the progression of their disease.

A significant percentage of amyloidosis patients have health insurance through the Veterans Health Administration or Medicare. Their coverage guidelines and high out-of-pocket costs can impede patients' ability to access groundbreaking treatments. Such barriers frustrate those living with the condition, their family members and the clinicians who treat them. Policy changes that make financial assistance more accessible could decrease patients' cost burden.

More treatment options mean more opportunities for clinicians to follow a patient-centered approach, one that allows for tailored treatment for each patient and his or her lifestyle. It's only possible though, if patients can access the innovative medications that could drastically improve their quality of life.

RARE DISEASES WORKING GROUP MEMBERS

Kelsey Barrell, MD

Emily Brown, MGC, CGC

Robert Gottlieb, MD, PhD

Dinesh Kalra, MD, FACC

Nitasha Sarswat, MD

Christina Shen, RN, BSN, MPP

Francy Shu, MD

Marcus Anthony Urey, MD

Please note that the views expressed in this document do not necessarily reflect those of the institutions with which working group members are affiliated.

ABOUT THE RARE DISEASES WORKING GROUP

The Rare Diseases Working Group is a network of policy-minded health care providers who advocate for patient-centered care.

To learn more, visit allianceforpatientaccess.org/rare-diseases

Rare Diseases
Working Group

**The access issues described in this white paper are long-running challenges that may temporarily be altered or impacted by policy responses to COVID-19.*