Rare Diseases
Working Group

Amyloidosis Initiative Meeting Summary

The Alliance for Patient Access hosted its annual meeting of the Amyloidosis Initiative of the Rare Diseases Working Group on July 27, 2022. The virtual event convened health care providers, advocates and other stakeholders to discuss policies that affect people living with amyloidosis, particularly hATTR amyloidosis.

Utilization Management

Utilization management tactics undermine the physician-patient relationship and delay access to care. As innovative treatments for amyloidosis continue to be developed and approved, policymakers should prioritize policies that limit utilization management and ensure appropriate patient access.

Prior Authorization

Members agreed that prior authorization processes can be frustrating and time consuming. Payers often require onerous prior authorization processes for amyloidosis patients who have had liver or heart transplants – even though transplants were standard of care prior to FDA-approved medications. “It’s nearly impossible to figure out how to navigate,” one physician emphasized.

Step Therapy

“Let’s face it, step therapy is essentially a fail first policy,” explained one member. Prolonged wait times for access to treatment can lead to irreversible disease progression.

Co-Pay Accumulators

One clinician noted that out-of-pocket costs are the biggest access-related pain point for his patients. Many insurance companies no longer count co-pay coupon cards toward patients’ annual deductibles. This can impose huge, unexpected costs on patients who cannot afford it. This can force patients into making choices between treatment and other life necessities.

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Genetic Testing

For amyloidosis patients, access to genetic testing is crucial because understanding whether a patient has hereditary TTR amyloidosis determines their treatment plan.

One clinician expressed concern that genetic testing may not always be accompanied by genetic counseling, which helps patients to understand the implications of testing and their results. Members agreed that access to genetic testing with counseling is important for proper care.

New Barriers

Clinicians also identified a new barrier that may be emerging for hATTR amyloidosis patients. Some insurance companies now require a repeat 6-minute walk test for patients before reauthorizing treatment. But there are factors that impact patients' functional capacity outside of their disease, one working group member explained, and payers must consider that when creating reauthorization requirements.

Policy Updates

During the COVID-19 Public Health Emergency, the Centers for Medicare and Medicaid Services broadened coverage for telehealth, along with some home infusion treatment, though that coverage is not guaranteed to continue.

Members agreed that they prefer to help patients gain access to home infusions, but that it often takes a persistent clinician dedicated to taking the necessary steps with payers. Changes are coming for Medicare recipients, as a $2,000 out-of-pocket cost cap for Part D was recently signed into federal law.

Veterans

Many amyloidosis patients who seek care from the Veterans' Health Administration cannot access the full, approved dosage for treatment of cardiomyopathy. One clinician noted that persistence in advocating for patients has improved access. VA amyloidosis patients with neuropathy, however, do not have a path to access without significant restrictions.

A guest presenter, Dan Donovan of rareLife Solutions, introduced survey data that identifies three main challenges for veterans: affording medication, geographical distance to treatment centers and barriers to appropriate treatment, such as the aforementioned dosing issue.

Future Advocacy Efforts

Members agreed that geographical access to care and treatment in conjunction with lack of awareness continue to be dangerous to patients' health.

To combat this, the working group will continue to engage on rare disease advisory councils, which exist on the state level. State legislatures can pass legislation to create councils made up of patients, providers, payers and other stakeholders to inform policymaking, ensuring that rare disease patients' specific needs are considered in the legislative proves.

AfPA's Rare Diseases Working Group will continue to build on its current advocacy efforts. New treatments are being approved for amyloidosis, which often leads to new barriers. The working group stands prepared to fight for patient access.

Get Involved

To learn more about AfPA's Rare Diseases Working Group and its Amyloidosis Initiative, contact Elizabeth Simpson at esimpson@allianceforpatientaccess.org.

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