



Rare Diseases Working Group Amyloidosis Initiative **2026 MEETING SUMMARY**

The Alliance for Patient Access hosted its annual meeting of the Amyloidosis Initiative of the Rare Diseases Working Group on April 17 and 18, 2026 in Washington, DC. The event convened clinicians, patient advocates and other stakeholders to discuss policies that impact people living with amyloidosis.

UTILIZATION MANAGEMENT

Prior Authorization and Reauthorization

Prior authorization continues to place significant strain on clinicians and delay patient care. Clinicians described it as a complex process requiring repeated calls, documentation and peer-to-peer reviews. One clinician said, “The process is designed to exhaust providers to convince them to give up.” These administrative demands can discourage clinicians from prescribing effective treatments and divert time away from patient care.

Reauthorization requirements further disrupt continuity of care. Even patients who are stable on long-term treatments may be required to restart the approval process. Insurance changes, particularly within Medicare Advantage plans, can reset access entirely, forcing patients to begin treatment approval pathways from the start.

Step Therapy

Clinicians identified step therapy as a persistent barrier to timely, appropriate treatment. Patients are often required to try and fail therapies — including off-label options — before accessing the prescribed medication. One clinician said, “Payers will insist on a stabilizer before going to a silencer even if the silencer is in the patient’s best interest.”

Participants noted that as we have multiple approved cardiac amyloidosis therapies, patients have more options, but may face added step therapy requirements.

Non-medical Switching

Non-medical switching remains a significant concern, particularly for patients who are stable on therapy. Clinicians reported that insurer coverage decisions often drive treatment changes rather than clinical need. One clinician said, “Patients have a lot of anxiety because they’re being told to move to a totally unfamiliar medication for arbitrary reasons.” These switches can introduce new side effects, reduce treatment effectiveness and create adherence challenges, especially when patients cannot tolerate the alternative therapy.

Formulary Exclusions

Clinicians emphasized that FDA approval does not guarantee coverage. Formulary exclusions and delays in covering new treatments can limit access, even when treatments are clinically appropriate or recommended in guidelines. In rare diseases like amyloidosis, where alternatives may be limited, these restrictions can leave patients without viable treatment options.



BARRIERS TO DIAGNOSIS

Delayed diagnosis continues to define the amyloidosis patient journey. Clinicians noted that the disease is often misidentified, with significant variation across subtypes. One clinician said, “We are not making the diagnosis any earlier.” Patients frequently experience prolonged referral pathways and are often diagnosed only after significant organ damage has occurred, underscoring the need for earlier detection and improved clinical awareness.

ACCESS TO TESTING AND SPECIALISTS

Access to testing has improved, including the availability of genetic testing in some cases. Gaps remain, however, for underserved populations. Clinicians also highlighted ongoing challenges in accessing specialists and coordinating multidisciplinary care. The setting of care often determines which specialist patients can see. For community clinics and rural areas, there are fewer clinicians and resources, as opposed to larger health systems and cities that have greater access.

One clinician said, “Seeing a cardiologist, nephrologist and neurologist is a pipe dream for many patients.” Workforce shortages, geographic barriers and limited infrastructure continue to restrict access, with telehealth offering only partial relief.

CLINICAL GUIDELINES

Clinical guidelines play an important role in informing care but are not always reflected in coverage decisions. Clinicians reported that even when treatments are supported by guidelines, they must still justify their use to payers. One clinician said, “There’s no patient focus at the payer level — we still have to prove the guidelines exist.” Participants also raised concerns about the growing role of cost and payer preferences in clinical decision-making, which may shift focus away from individualized, patient-centered care.

SPECIALTY TIERS

Specialty tier placement continues to drive high out-of-pocket costs for patients with amyloidosis who largely have treatments that fall under specialty tiers. Unlike traditional tiers with fixed copays, specialty tiers often require coinsurance based on a drug’s cost, creating significant financial barriers. Clinicians noted that these costs can delay treatment, impact adherence and increase overall patient burden.

NEXT STEPS

Participating clinicians and patient advocates were eager to use their voice on behalf of other patients. Participants offered ideas for new educational resources and expressed interest in engaging with policymakers.

GET INVOLVED

To learn more about AfPA’s Amyloidosis Initiative and Rare Disease Working Group, visit www.AllianceForPatientAccess.Org/rare-diseases or contact Isabelle Logsdon at ILogsdon@allianceforpatientaccess.org
