Rare Diseases Working Group
Amyloidosis Initiative
2025 MEETING SUMMARY

The Alliance for Patient Access hosted its annual meeting of the Amyloidosis Initiative of the Rare Diseases Working Group on October 15, 2025. The virtual event convened clinicians, patient advocates and other stakeholders to discuss policies that impact people living with amyloidosis.

UTILIZATION MANAGEMENT

Prior Authorization

Prior authorization remains a barrier to care for patients with amyloidosis. Meeting participants noted how prior authorization delays treatment access for rare disease patients who already have few treatment options.

One clinician said, "The reality is that you can probably get the medication approved eventually, but that's time taken from direct patient care."

Step Therapy

Meeting participants agreed that step therapy is still an issue for patients and clinicians. Many expressed that insurance companies are denying medication approvals if patients haven't undergone genetic testing, despite a confirmed diagnosis.

This barrier to care can lead to disease progression and negative health outcomes.

Formulary Exclusions

Formulary exclusions omit coverage for certain medications and pose an ongoing issue for rare disease patients, especially those with heart issues.

New-to-market exclusions in particular block access to new therapies for a period before they become available to patients. This roadblock impedes access to life-changing treatment for patients who already have few available options.

DIAGNOSTIC ODYSSEY

Diagnosing a rare disease is often a long and arduous journey, for various reasons. Awareness among patients and clinicians is often the most prominent barrier to diagnosis. Meeting participants alluded to knowledge gaps among general and community practitioners, which can contribute to delays in referrals to specialists.

Misdiagnosis prolongs the process and ultimately leads to delayed treatment. Patients shared common conditions they were diagnosed with prior to receiving an accurate amyloidosis diagnosis.

ACCESS TO SPECIALISTS AND TELEHEALTH

Few clinicians specialize in treating amyloidosis. This lack of providers creates an access gap that makes it difficult for patients to receive appropriate care, especially if they live far away from a specialist. Participants shared that improving education and awareness among community cardiologists, who often see amyloidosis patients prior to a specialist, can help bridge the gap.

Centers of excellence were also mentioned as a possible solution because they are better equipped to treat complex patients. Telehealth also has helped expand reach, especially for patients who are already diagnosed with amyloidosis and just need follow-up care.

SPECIALTY TIERS

As more therapies are approved, ensuring access will be critical. Newer medications are often placed on a higher, or specialty, tier of drugs, making them more expensive and ultimately less attainable. Meeting participants shared their experience with the impact of specialty tiers and how insurers are increasingly relying on coinsurance, causing patients to pay a percentage of the list price for their medications, instead of a flat copay.

One patient advocate shared, “The cost of my medications went down, but my out-of-pocket costs went up.”

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 legislative engagement.

GET INVOLVED

To learn more about AfPA’s Amyloidosis Initiative and Rare Disease Working Group, visit
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