



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 October 22, 2024. The virtual event convened health care providers, patient advocates and other stakeholders to discuss policies that impact people living with amyloidosis.

Utilization Management

As amyloidosis treatment continues to evolve, patients and providers will have more options to treat the condition. They may also face more stringent utilization management that drives a wedge between patient and provider. As treatments become available, policymakers must prioritize patient-centered care and ensure that patients can access appropriate therapies in a timely manner.

Prior Authorization & Reauthorization

Meeting attendees noted that prior authorization remains one of the most significant utilization management barriers that they face. For patients beginning a new therapy, the administrative burden on the clinician's office is significant. The battle often continues, year over year, as patients and physicians are also forced to seek reauthorization.

Step Therapy

Step therapy is another challenge for amyloidosis patients and providers. With step therapy, patients are required to try and fail an insurer-preferred treatment before accessing the medication their provider prescribed. This can lead to disease progression and other negative outcomes. Policies that streamline step therapy and prioritize patients' needs are critical, particularly as additional treatments become available.

Formulary Exclusions

As new therapies become available to treat amyloidosis, ensuring that people can benefit from the full range of treatment options will be essential. In recent years, the number of medications excluded from health plan formularies has continued to rise. This is true in commercial insurance, as well as in insurance systems like the Veterans Health Administration, where patients have limited access to amyloidosis therapies. Reforms are critical to ensure that patients and providers can access all approved treatments.



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Telehealth

Working group members noted that expanded access to telehealth services during the Public Health Emergency improved their ability to see patients who historically had not received proper care. Since the Public Health Emergency ended, however, some of these patients were lost to follow-up. One attendee noted significant licensing issues for treating patients who live out of state. Others noted that they now can see patients via telehealth for follow-up visits only, a limitation that has restricted their ability to see newly diagnosed patients. Attendees strongly supported permanently extending telehealth waivers.

Mental Health Considerations

For patients living with a rare disease like amyloidosis, mental health implications can be significant. Diagnosis can bring many questions, and patients may feel isolated. Attendees commented that advocacy organizations have been critical in connecting patients to the broader amyloidosis community, while support groups allow patients to share common experiences. Online and virtual support groups also play a role in providing avenues for patients who may be harder to reach. Yet gaps remain. Attendees noted that the mental health considerations of life with amyloidosis need to be addressed holistically and with a patient-centered approach.

Future Advocacy Efforts

Members agreed that the future of amyloidosis treatment, given the recent and coming innovation, is exciting. But they acknowledged that with new options come new barriers.

Attendees acknowledged that advocacy will be critical in ensuring that patients and providers across the country benefit from these new treatment options. Policies that prioritize patient-centered care, limit onerous utilization management, and emphasize continued innovation will continue to be the focus of the Rare Diseases Working Group and its Amyloidosis Initiative.

Get Involved

To learn more about AfPA's Rare Diseases Working Group and its Amyloidosis Initiative, contact Ryan Crump at rcrump@allianceforpatientaccess.org.



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