



## 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 August 16, 2023. The virtual event convened health care providers, advocates and other stakeholders to discuss policies that affect people living with amyloidosis.

## Utilization Management

While several therapies have been approved for hATTR amyloidosis, utilization management tactics continue to drive a wedge in the provider-patient relationship and delay access to appropriate care. As amyloidosis care continues to evolve and additional therapies become available, policymakers must prioritize patient-centered care and limit the negative impacts of utilization management.

### Prior Authorization

Working group members agreed that prior authorization continues to block and delay access to appropriate and timely care. Approval for both testing and medications remains a challenge. One provider also mentioned reauthorization challenges. While a patient's disease does not disappear when the calendar changes, insurers may still use arbitrary timelines to interrupt continuity of care.

### Step Therapy

Step therapy, which requires patients to "fail" insurer-preferred treatments before receiving the originally prescribed treatment, remains an issue for amyloidosis patients. This tactic can lead to disease progression, with one provider noting that "the patient can't get that time back." Policies must limit step therapy's impact to ensure timely access to current and future therapies.

### Non-Medical Switching

Working group members agreed that, as additional treatments for amyloidosis become available, patients may face non-medical switching. Non-medical switching is a practice in which an insurer or pharmacy benefit manager forces a stable patient from their prescribed treatment for non-medical reasons. This can lead to new side effects, disease progression and other negative health impacts.



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## Cost-Sharing

In recent years, new insurance cost-sharing methods have emerged that increasingly shift medication costs onto patients.

Copay accumulators, copay maximizers and alternative funding programs subject insured patients to higher costs and ultimately poor outcomes. Whether it be learning their copay coupon does not count toward their deductible or losing drug coverage due to an alternative funding program, patients are forced into difficult situations where they may need to choose to pay for their treatment or to forgo the treatment entirely.

AfPA and the Rare Diseases Working Group is committed to advocating on behalf of policies that ensure all payments made on behalf of a patient are applied to their deductible and that patients are protected from programs that limit coverage of life-changing treatments.

## Amyloidosis and the Veterans Health Administration

AfPA also continues to prioritize equitable access within the Veterans Health Administration. While there are several treatment options approved for the treatment of hATTR amyloidosis, the Veterans Health Administration covers only treatment for the cardiovascular presentation of amyloidosis. Patients with polyneuropathy do not have a covered option and are forced to try and fail an off-label treatment, or file a non-formulary request, to access FDA-approved treatment for polyneuropathy.

Even for patients with cardiomyopathy, the FDA-approved dose is not covered. Providers acknowledged that the struggle

to prescribe the appropriate dose is a significant barrier. AfPA has engaged with the Veterans Health Administration on this issue and recently presented a poster at the World Orphan Drug Congress on the restrictive care for the health care system's patients.

## Future Advocacy Efforts

Members agreed that future innovation for amyloidosis treatment is exciting; however, they recognized access will be a challenge for their patients.

To ensure patient-centered care remains at the forefront of policies impacting rare disease, the working group will continue to engage on amyloidosis-specific barriers, as well as broad rare disease policies like rare disease advisory council legislation and implementation, protection of the accelerated approval pathway, and access to genetic testing and counseling. AfPA's Rare Diseases Working Group will continue to build on its current advocacy efforts. As current and new treatments face access 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 Casey McPherson at [cmcpherson@allianceforpatientaccess.org](mailto:cmcpherson@allianceforpatientaccess.org).



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