



Rare Diseases Working Group

2021 Meeting Summary

The Alliance for Patient Access hosted its annual Rare Diseases Working Group meeting virtually on May 7. The event welcomed health care providers, advocates and fellow stakeholders to discuss policies that affect people living with a rare disease, and particularly, amyloidosis.

Utilization Management

Utilization management tactics used by payers delay patient access to care and hurt the physician-patient relationship.

- **Co-Insurance**

“Co-insurance’ is just a fancy way to say out-of-pocket cost,” one clinician noted. Members agreed co-insurance can impose unmanageable out-of-pocket cost for patients and block access to the therapy they need.

- **Specialty Tiers**

Working group members said specialty tiers are especially harmful to rare disease patients, who already deal with expensive conditions and rely on innovative treatment options. New or more costly drugs are routinely placed on specialty tiers and saddled with high out-of-pocket requirements.

- **Co-Pay Accumulators**

Some insurance companies no longer accept co-pay card coupons toward patients’ annual deductibles. These “co-pay accumulator adjustment programs” may require patients to forego essentials such as groceries or paying utilities to afford their medication. “No one should have to make that choice,” a physician emphasized.

- **Prior Authorization**

Members agreed the prior authorization process is frustrating by design. Patients get discouraged and give up, or busy clinicians simply don’t have the bandwidth to complete the onerous paperwork on time. “Of the patients who have a prior authorization, 60% will not get access to the drug,” one physician added.



Policy Updates

The Centers for Medicare and Medicaid Services loosened their coverage restrictions during the Public Health Emergency. Patients with mobility issues who use injectable medications were allowed access to treatment from the comfort and safety of their homes. While this helped with patient adherence, several members noted patients with no mobility issues were not granted the same access. Members agreed coverage for home infusions for all patients would help with continuity of care.

Additionally, existing billing codes were made eligible for telehealth, and payment parity allowed providers a fiscally viable way to expand telehealth offerings. One working group member said the technology helped decrease barriers for patients this past year. Audio-only calls are also now available, expanding access to patients who live in rural areas where broadband might be lacking.

Veterans

For amyloidosis patients, access to the three available FDA-approved medications is restricted, leading to significant access challenges for veterans. Several members shared stories of veterans crossing state lines in order to access the medication they need with fewer restrictions. One member noted barriers at the VA could stem in part from the

VA's partnership with the Institute for Clinical and Economic Review. The group of economists uses controversial metrics and limited clinical trials data to make judgment calls about drugs' cost-effectiveness.

Future Advocacy Efforts

Members agreed that lack of awareness continues to be a problem and can even be dangerous for patients' health. Patients' conditions will continue to progress without proper diagnosis and treatment. One physician emphasized the need for educational materials to help expand public outreach and awareness, adding, "We say 'rare disease', but they aren't as rare as we think."

The Rare Diseases Working Group will continue to build on its current advocacy and educational efforts in the coming year to support policies that better care for both amyloidosis patients and the rare disease community as a whole.



**Alliance for
Patient Access**