HAYSTACK PROJECT
The Voices of Rare & Ultra Rare
AEI
Advocacy Empowerment Institute

Haystack Advocacy Empowerment Initiative (AEI) Speaker Series
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National Academy for State Policy
March 10, 2022

The National Academy for State Health Policy is a nonpartisan forum bringing together **state** government policymakers to discuss health policy challenges – for the purpose of sharing and learning from each other and then leading and implementing in their home states. Discussing the latest policy intelligence and ideas, and sharing best practices across states is central to NASHP's mission.

Hemi Tewarson, JD, MPH, comes to this role most recently from serving as a senior fellow at the Duke-Margolis Center for Health Policy, but also with almost six years of state experience with the National Governors Association's Center for Best Practices, and over 8 years at GAO, the investigative arm of Congress.

Hemi spoke at Haystack Project's March Speaker Series meeting about the healthcare issues that state policymakers are currently focused on, shared insights on rare disease health issues from the state perspective, and graciously agreed to continue the conversation to see how some of Haystack Project's issues might be addressed at different state forums.

Discussion

Hemi started off the hour talking broadly about state priorities. The **healthcare workforce** shortages, especially in nursing, behavioral health, and long-term care, loom large for states. They are looking for ways to connect healthcare workers with the community. While this is a more recent top tier issue, exacerbated by the COVID-19 pandemic, the perennial issue, Hemi noted, is **cost of healthcare**, including health system costs overall and the price of prescription drugs.

Although some of the specific drug pricing ideas have fallen away from immediate action at the Federal level, Hemi noted there is widespread interest in working on them at the state level. NASHP views these policy ideas – e.g., price transparency, pharmacy benefit manager (PBM) reform, prescription drug affordability boards, unsupported price increases, importation and international reference pricing -- as not just relevant to Medicaid budgetary concerns, but also as ideas that may reduce costs for consumers. NASHP has model legislation on some of these topics. States are looking to ways in which they can use data to control pricing. We shared our concern that these policies are largely not relevant or applicable to really rare conditions. Our community represents a very specific and unique segment of 'rare patients' who have specific health care needs that are not represented in the larger, population of general 'consumers.' Hemi acknowledged that states have mostly not looked at these policies with rare patients in mind, and encouraged us to educate state policymakers about our unique circumstances.

On the specific topic of importation, one of our patient groups noted that "in our experience being an international organization, HTA/Canada is even harder [on access barriers] given that the government make the determination about what drugs they will even put on their formulary. It's a huge fight to even have [a treatment] available for [our] patients - then you have to fight for access. What we have ended up with is lack of good therapies being available for anyone."

Finally, another looming health policy challenge for states at the moment is behavioral health, both mental health and substance use disorder.

The political overlay to these pending challenges was interesting, as Hemi explained the backdrop of an election year in which 36 governors are running for re-election. Our patient community, spread across all 50 states, can watch for campaigning messages centered on these three health care issues. Of note, Hemi agreed that she has not heard about rare disease issues as much of a concern at the state level. States have not been clamoring for NASHP to lead or convene on rare disease issues.

Lightning Round

Given Hemi's deep experience in state based health care, we asked Hemi to give us her "first impressions" of what states might think about issues we care about in the rare and ultra-rare community. She was game to participate, although caveated that some of these issues are new to her, and NASHP has not considered them before. Dubbed a "lightening round" or "speed dating" through a series of issues, we agreed to see if there might be an opportunity to return to some of these issues off-line if they were of interest to NASHP. Here's what we learned:

Medical necessity: We discussed the need to *broaden* 'medical necessity' to include clinical guidelines, peer reviewed journals, and expert opinion so rare disease patients could access the same medically necessary care other patients enjoy.

Response: Hemi explained that the concept of 'medically necessary' care has generally come up at the state level as a lever to ensure appropriate levels of care and control costs. She acknowledged that doing so had been a "blunt tool," and one that the courts pushed back on in some states. She encouraged rare disease advocates to continue to educate policymakers on why and how "medically necessary care" needs to be thought of differently for rare patients, while helping states to ensure that our approach does not erode state coffers.

COVID/Telehealth/Network Adequacy: We discussed how rare disease patients have long needed, and will continue to need the flexibility to go out of state for our care, regardless of the PHE. We highlighted the need for states to study if in fact costs for rare patients really went up or outcomes improved during the PHE, so that states may not be as negative as they have been in the past to grant these flexibilities to rare disease patients.

Response: Hemi said the "telehealth conversation" is happening right now, as we consider the end of the PHE and the potential end of COVID flexibilities. States are struggling with what kind of oversight they need after the PHE ends, and if there is benefit to keeping the existing flexibilities in place. The workforce shortage means there are not enough providers, which may bolster the argument to keep telehealth flexibilities in place. States don't have the bandwidth to find pilot programs, but the rare disease community could certainly propose this, perhaps in conjunction with some of the researchers, Hemi said, who are currently looking for study ideas to capture PHE-learnings to inform how health care might function differently post-PHE.

Bundled payments: We explained how settings of care that rely on bundled payment reimbursement, based on 'average' costs of 'average' patients with 'average' conditions does not translate well for rare disease patients. In fact, it systematically discriminates against those with rare diseases, making access to standard of care very difficult.

Response: Acknowledging bundled payment systems are a way to save costs, Hemi nonetheless said the issue had not been framed with rare diseases in mind. She once again encouraged us to educate state policymakers on this important nuance.

Drug Pricing: We led this topic by sharing that patients often feel they have no role in pricing, but are learning quickly about the important job they have in ensuring reimbursement structures do not inadvertently deny or limit care for their rare conditions. We are particularly sensitive to any incentives/ disincentives or financial pressures rare disease providers or academic centers face in treating rare patients. We also welcomed any ideas on truly tangible, practical ideas for patient advocacy on drug pricing, but distinguished the need to avoid "boil the ocean' or 'sound bite' approaches.

Response: Hemi advised that states are starting to coalesce around drug affordability boards - as Maryland and Colorado have. States are looking at the 10-25 highest cost drugs, and figuring out how the state can have more authority over their pricing. States rely on many sources of data, ICER is one; international reference pricing could be another. States are also working on "unsupported price increases" – where, for example, increases are two times the rate of inflation. She specifically mentioned New York, where the state negotiated supplemental prescription drug rebates for their Medicaid program; Massachusetts, Hemi highlighted, has also implemented a similar approach. Of note for our community, though, Hemi accepted that such state approaches generally were not considering the cost of drugs for rare diseases, and that this nuance or segment is not generally incorporated into these conversations, because they may not be a top cost driver for state programs. She added that the issue of pricing is not only about the state Medicaid budgets, but also out-of-pocket expenses for patients.

Haystack Perspective: Out-of-pocket costs are minimal in Medicaid. But our community appreciates that this does not mean we are immune from the debate over costs. Even if our patients are paying minimal cost sharing/copays, the pressure on state budgets results in simply being denied access to treatments critical to our patients. So it is important to engage in this debate regardless of minimal out of pocket costs to our patients.

Accelerated Approval: We provided Hemi some context on our concerns that companies should not be dissuaded from using this pathway for rare diseases.

Response: Hemi concurred that perhaps the same types of concerns are not there in the rare context, citing instead the Aduhelm example. Hemi noted that rare treatments are not the cost driver that a more common disease like Alzheimer's would be.

Pharmacy & Therapeutics (P/T) Committees: We kicked off this discussion expressing our appreciation that the role of a P&T committee has in helping to tamp down costs with formulary placement and other tools. However, that important role only exists when there is more than one treatment for a condition. Most rare conditions have no treatments or are lucky enough to have one. Until they have two, the role of the P&T committee is simply to delay access to treatment.

Response: Hemi noted that P/T Committees play an important role for states. At the same time, she acknowledged the challenge with rare conditions and why Haystack is interested in limiting the role of P/T Committees for at least the first drug to market for a rare condition.

Conclusion

Hemi advised our community to think more about pricing transparency and the "black box" of drug pricing and where there could be commonality in identifying ideas on how to lower the cost of prescription drugs. Haystack asked that she give some thought to specific and tangible ways in which our community might be supportive, but that we would stay away from "sound bite" or "boil the ocean" ideas. Haystack's success with policymakers has relied heavily on building credibility by bringing forward targeted, impactful, incremental, thoughtful ideas for change that a viable bipartisan solutions for our community.

She emphasized the need for Haystack Project's work in ensuring that "Rare gets on [states'] policymakers' radar. Throughout the hour-long discussion, Hemi returned to the idea that states had not and were not considering rare and especially ultra-rare patients in their policymaking. We agreed to stay in touch with NASHP as a good source for ways in which to do just that.

And finally, asked if there was one thing she would change about state Medicaid, she said that states could do a better job of focusing of simplifying the system to acknowledge and improve the beneficiary experience. It was hard to disagree since we have often discussed that the system feels adversarial at best, when our community is at its most vulnerable.