



PATIENT VOICES, PATIENT VALUE: DEVELOPING PATIENT-CENTERED SOLUTIONS

INTRODUCTION

At the Partnership to Improve Patient Care's (PIPC) [Seventh Annual Forum](#), patient and disability advocates highlighted their ongoing efforts to ensure all stakeholders are equipped with the tools they need to fully and meaningfully engage with patients and achieve value for the people served by health systems. Panelists discussed the significant strides the patient-centeredness movement has made through the creation of the Patient Centered Outcomes Research Institute (PCORI), the FDA's program for Patient-Focused Drug Development, and the Precision Medicine Initiative, while acknowledging the crossroads the movement currently faces. Both panels debated the struggle between determining the value of health care based on what is cost effective for the average patient or based on achieving outcomes that matter to individual patients and people with disabilities. Moderator Tony Coelho, Chairman of PIPC, led two panels with representatives from the National Health Council, National Patient Advocate Foundation, Faster Cures, Cancer Support Community, Autism Speaks, and the National Alliance on Mental Illness.

OPENING REMARKS

Tony Coelho, Chairman of the Partnership to Improve Patient Care, spoke to the importance of continuing to push hard with the beginning of the new administration to ensure patient voices are heard and explained that his passion for the issue is derived from his own battle with the healthcare system. As a young man with epilepsy, his dreams of a normal life were dashed, and he took to advocacy to make a difference in patient care. He clarified that Ted Kennedy originally encouraged him to take the chairmanship role at PIPC, and he has since continued to work hard for the effort on both sides of the aisle. He asked that advocacy groups carry a new commitment to the beginning of the Trump administration, in order to bring fresh urgency to the cause. He warned that lawmakers who chose only to reside in "ivory towers" will not be able to fully grasp what their constituents need, and that Congress ought to push funding for research that will be relevant to the healthcare decisions we will need to make in the future. Mr. Coelho pleaded that healthcare reform celebrate the difference between patients, and not try to capture individual needs in broad averages in order to achieve cost-effectiveness. He also recommended that value-based frameworks be paired with precision medicine in order to fully advance healthcare brings benefit to every patient.

FIRST PANEL DISCUSSION

Lorri Unumb, of Autism Speaks, gave personal testimony of healthcare woes with Autistic children as well as highlighting her work and expertise with the organization. As a mother with two sons who have been diagnosed on the Autism spectrum, she was motivated to write and advocate for the passage of “Ryan’s Law” in South Carolina that requires healthcare providers to cover Applied Behavioral Analysis (ABA) and associated therapies. She has gone on to promote the bill to passage in 44 total states. Ms. Unumb stated that insurers are not attempting to apply metrics based on average effectiveness to determine which therapies and which conditions are most widely “successful,” and discontinue coverage of ABA for some patients. She challenged healthcare providers to think about what it means to determine a therapy “didn’t work” for an Autistic patient, and to allow exploration of therapies and time to pass before discontinuing coverage for a patient. She also specified that success of treatment for some patient groups, for example Autism, cannot be easily conveyed and the rate at which a patient improves is clear only to those closest to them.

Ms. Unumb declared that appropriate, patient-centered treatment was cost-effective in the long run, as patients who are properly treated will require less treatment or therapy in the future. She stated that there is a misconception that patients simply want the most expensive treatments, but in fact, they simply want the treatment that will most effectively treat their needs. She explained that it was important to present data that explains this, and beats the misconceptions, so that lawmakers will take the issue seriously. Additionally, she advised that impact on the entire family be considered, as well as quality of life, as a measure when determining frameworks. She argues that grassroots advocacy could be effectively used to promote the issue, and urged that patient advocates challenge payers to be transparent about their decisions.

Maureen Japha, JD, of FasterCures, highlighted how value-frameworks can be improved using steering committees with diverse industry representation. She explained that FasterCures was working with a steering committee to determine what patient perspectives were still missing from the existing framework, and that all groups impacted by the issue were well represented. She described the [five domains](#) they had developed to properly assess existing framework as patient preferences, patient-centered outcomes, patient and family considerations, quality and applicability of evidence, and usability and transparency. These domains, she stated, would give advocates and researchers a closer look at how the patient perspective could be provided.

Ms. Japha insisted that paying for treatments that are personally valuable to patients can increase adherence, and thus over time can be cost effective. She also acknowledged the importance of attempting to define “quality of life” in a manner than benefits all patients while maintaining personal needs and preferences, and acknowledged it is an issue FasterCures is exploring. She recommended looking at data that included individualized data in an attempt to better understand patient preferences.

Eleanor Perfetto, PhD, of the National Health Council, offered a clear definition of “value-frameworks” and described the work the NHC is doing to give patients the tools to recognize true

patient-centered frameworks. She clarified value frameworks are an approach used by payers or third parties to determine the value of available treatments or therapies based on the potential overall benefit to a patient. While this may involve quality of life as a measure, she explained that, much like clinical trials, it was not a requirement. Dr. Perfetto also revealed that while patient input is a crucial element of developing frameworks, recommendations and concerns are not always included in the final product. She illustrated the recent efforts of the NHC to develop guidelines in which patient advocates can evaluate the strength of available frameworks and determine if patient-centered approaches are being taken. Dr. Perfetto stated that it was too early to create a scoring system, but that the guidelines put out by the NHC can begin necessary conversations that need to take place and spark interest in the issue.

Ms. Perfetto strongly advocated for research that focuses on the correct endpoints rather than taking traditional approaches. She explained that data that studies actual outcomes that patients prefer are more useful to informing future policy and developing value-frameworks. Additionally, she stated that it is important not to make assumptions for patient preferences and seek out real input instead of focusing value frameworks on a vague definition of “quality of life.” She noted that value framework developers are beginning to improve their processes for patient engagement, but have a long way to go.

SECOND PANEL DISCUSSION

Andrew Sperling, of the National Alliance on Mental Illness, stated that he was encouraged that the work of PIPC was beginning to take root in some agencies, and could better inform other administration offices of how to engage patients to start necessary reform. He expressed that he was optimistic about the work CMS could do to develop alternative payment models, although wished they were more thoroughly researched and received patient and physician input before being brought to a national and public demonstration. Mr. Sperling also advocated for further research of comorbidity between conditions, and specified the need for comorbidity to be included in measures of value framework. He warned of using “quality-adjusted-life-years” to develop yet another broad framework, instead of effectively achieving patient-centeredness.

Joanna Buzlago, PhD, of Cancer Support Community, spoke of the necessity to connect patients and physicians in meaningful and constructive ways. She explained that the Cancer Support Community teaches patients how to talk to their doctors in order to ensure they are receiving the appropriate care for their circumstance and preferences, and informs doctors of ways in which they can take a more active role in providing patient-centered care. Dr. Buzlago specified that increasingly productive conversations between patients and doctors can help facilitate more informed research. Additionally, she advocated for research and engagement that targets a diversity of patients to ensure the patient voice is effectively captured in the development of new payment and delivery models. She also noted the implications for family, both financial and personal.

Rebecca Kirch, JD, of the National Patient Advocate Foundation, reminded the panel that frameworks are currently focused on treating conditions, and do not properly acknowledge the

human needs of a patient. She encouraged advocacy groups to place importance on involving secondary caregivers that provide treatment targeting those human needs, and promoted including access to secondary caregivers in the development of value frameworks and payment models. She declared that physicians are “flying blind” if they fail to establish the goals and preferences of a patient before they begin treatment. She also acknowledged the need to consider the implications for the family and community when considering quality of life.