PIPC Partnership to Improve Patient Care

VALUE FOR WHOM? Incorporating Patient Perspectives into Value Assessment for Novel Cell and Gene Therapies

On Oct. 8, 2020, Partnership to Improve Patient Care (PIPC) and EveryLife Foundation for Rare Diseases hosted a webinar on the findings of the new Health Advances White Paper: *Value for Whom? Incorporating Patient Perspectives into Value Assessment for Novel Cell and Gene Therapies*.

The expert speakers discussed the use of traditional cost-effectiveness analyses to determine the "value" of innovative cell and gene therapies. The speakers concluded there were many shortcomings in using CEA on these novel therapies and discussed new and innovative models that are moving in a better direction and trying to truly determine value to patients.

Read some of the key highlights:

"There are so many shortcomings with respect to what the QALY can capture…one of the things I had mentioned is the QALY really focused on one intervention. It looks at one intervention versus standard of care, so it really doesn't look at societal preferences about where to allocate resources…. so for example in England where the QALY and CEA is really sort of reigns supreme as one of the main ways that country determines coverage and access, what you see - having studied it - is that they tend to favor hypertension, diabetes, and they have very hard time with coverage for rare diseases for very few patients, because, again, the QALY just can't factor in that society has decided that we want to allocate resources to patients with rare disease. In addition to it being proven to be discriminatory against people with disabilities, seniors, a whole range of patients. It can't capture hope, it can't capture the value of downstream innovation, it can't capture that value of insurance. We can go on and on. There are so many shortcomings in the QALY. It's surprising it's lasted this long honestly." *– Julia Gaebler, PhD, Health Advances*

"Your state Medicaid is making decisions based on these reports. New York and my home state of Massachusetts currently use ICER reports in their decision making. What does this mean for my family? Max and Austin are managed Medicaid recipients and as of today they do not receive insurance coverage for any of the only treatments available for Duchenne. The drug that was approved in part by Max's four open muscle biopsies over the year, he is not eligible for."
– Jenn McNary, Rare Disease Patient Advocate

"Value assessments are impacting payer determinations and access…and now we are starting to see some of these hit other policy proposals like for example the international pricing index or most favored nations executive order, so again this matters, because it now shapes and informs policy that could again have negative implications for patient access."
– Annie Kennedy, EveryLife Foundation for Rare Diseases

"What Value of Hope is really trying to capture is something inherently important to patients and families, just like you heard Jenn mentioning. It's the value to that community of having a potential therapy that will cure their disease or could maybe help them regain some independence, and then there could be a curative therapy somewhere down the line for future patients, and we need to figure out how to incorporate that value somewhere in value assessments."
– Jennifer Bright, Innovation and Value Initiative