

PIPC Patient Blog: Our Children Are Worth It

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Should one consider the life of a person with a disability less valuable than the life of a person without a disability? Alarming, states are increasingly relying on policies that do just that, in order to determine the value of health care services and treatments. For children suffering from life-threatening and debilitating disorders, like my daughter, reliance on any standard that considers her life less valuable than someone without a disability, is simply unacceptable.

For example, starting this year, New York State's Drug Utilization Review Board used measurement of costs per quality-adjusted-life-years (QALYs) to assess the value of treatments to Medicaid beneficiaries. Also, Massachusetts sought a Medicaid waiver (currently under review) to exclude certain drugs from coverage based on a "cost effectiveness" calculation. For the first time, the federal government is seriously considering state proposals to use average "cost effectiveness" to limit formularies under Medicaid.

This recent trend is not the first instance of states attempting to use cost effectiveness data to limit or justify limiting patient access.

When Oregon received a Medicaid waiver in 1993 to establish the Prioritized Health Services List of covered services for its citizens on Medicaid, they quickly moved to reduce access to treatments for ultra-rare disorders, including MPS, Gaucher disease and Fabry disease among others, in part justifying its decisions using QALY-based cost effectiveness analysis. The blowback from patients and people with disabilities was swift who contend that QALYs fail to capture the value of a therapy to each individual patient. Many stakeholders contended the use of QALYs to determine care covered for patients with ultra-rare disorders is not only inappropriate, but discriminatory.

As the mother of a 12-year-old daughter with MPS, I can't help but get the shivers when new policies come out that could discriminate against children with disabilities. My daughter Annabelle has big blue eyes and a smile from ear-to-ear that lights up a room. She has hopes

and dreams that are as big and exciting as any other young, vibrant child. But Annabelle's daily life is dramatically different than what the average American faces. Along with all the regular stresses of school, peer relations, and so forth, Annabelle is short in stature (approximately the size of a 3- or 4-year-old), she has severe bone and joint problems, hearing loss, corneal clouding, leaking heart valve, lower airway obstruction, along with other aches, pains and difficult-to-manage medical issues.

Through all of it, Annabelle is joyful and living a life with less pain and more stamina - thanks in large part to an FDA-approved orphan drug. To be sure, bypassing hurdles to get access to this product were in no way an easy feat. But this was one mountain well worth the climb because it gave Annabelle a new lease on a less painful and longer life. Each week, Annabelle has a 6-hour infusion of this life-improving medicine. It isn't a cure but what it is... is critically important.

Within months of starting this treatment, 5-year-old Annabelle was able to walk longer distances to the playground, climb up ladders, and "be a child" more often with less overall pain. She went from sitting on the park bench with big tears in her eyes to joining in with the rest of the kids. She can't do anything close to what those other kids can do but she is at least able to try. No academic cost effectiveness study captures the value of Annabelle's smile.

As Americans, we hold ourselves to a higher standard than just average.

We place value on all individuals and enforce laws to make sure those values are upheld and honored. If there was a medicine that gave a significantly improved quality of life to a small number of patients, don't those patients have equal value as the ones that have less complex or better understood diseases? Does the value of a patient's life reduce because it is harder and more expensive to treat? If it is your child or loved one, the answer is a no-brainer.

Wading into the waters of whether a patient is worth a treatment is dangerous territory.

One day, that may directly impact you or your loved one. When I watch my daughter walking rather than riding in a wheelchair and enjoying after-school activities like drama or coding club rather than being exhausted and isolated because of pain and exhaustion, the answer is very clear to me that she is worth it. She is living her best life because she has access to a treatment that is genuinely improving her quality of life. And I can only hope for the same for other kids just like Annabelle.