

February 20, 2019

Dr. Steven D. Pearson
President
Institute for Clinical and Economic Review
Two Liberty Square, Ninth Floor
Boston, MA 02109

Dear Dr. Pearson:

The Partnership to Improve Patient Care (PIPC) has a long history advocating for the perspectives of patients and people with disabilities to be considered in the value assessment of treatment options. It is a step in the right direction for the Institute for Clinical and Economic Review (ICER) to advance a new project to develop and test alternative methods for the evaluation of potentially curative treatments.¹ As part of this work, we encourage ICER to consider innovative methodologies beyond flawed cost effectiveness methodologies that use a quality-adjusted-life-year (QALY) or similar metric. This process could be an opportunity to learn from the mistakes of other countries that have embraced the use of a cost-per-QALY metric to determine treatment value with serious implications for access to care by people with disabilities and serious chronic conditions.²

We know that applying a single cost-per-QALY threshold fails on all patient-centeredness domains.³ While there is no single alternative method that succeeds on all domains at present, we are encouraged that several other organizations and approaches are underway in developing value assessment models that better reflect principles of patient-centeredness. We applaud ICER for recognizing the need to improve affordability for patients, and hope you take this opportunity to learn from the ongoing efforts of others to develop patient-centered methods for value assessment that incorporate a range of evidence to determine coverage and care decisions, and reject a single, one-size-fits-all measure of value. By following their lead, ICER has an opportunity to be part of the solution for patients and people with disabilities to access innovation.

In advancing this project, first and foremost, it is vital to ensure value assessments are not conducted in a manner that justifies barriers to care based on one-size-fits-all determinations that in turn create disincentives to develop innovations, including cures. We appreciate new drugs both approved and utilized in the U.S. health system over the last decade that primarily manage disease or alleviate symptoms. And, we also want to ensure that drugs and technologies that cure diseases are developed and seen as a long-term return on investment by our nation's system of healthcare.

¹ See <https://icer-review.org/topic/valuing-a-cure/>

² See <http://www.pipcpatients.org/access.html>

³ See <http://www.pipcpatients.org/resources/white-paper-uses-and-misuses-of-the-qaly-ethical-issues-and-alternative-measures-of-value>

ICER has stated that the goal of their new initiative is *“to ensure that assessment methods are tailored appropriately to the distinctive nature of the evidence base for potential cures.”* To this end we would like to raise a number of points that we think are essential to ensuring access to cures and their continued innovation.

ICER should appropriately acknowledge the long-term health benefits and cost savings resulting from curative therapies.

As an example of the barriers that patients and people with disabilities face in accessing innovative cures, we just have to look at recent innovations that have offered the potential of a cure for many, such as HIV drugs a few decades ago, HCV drugs a few years ago and most recently CAR-T therapy in some cancers. All are examples of “cures” by most definitions of the term, but all have had to run the gamut of speculation that their prices were “too high” even though they were actually providing enormous long-term value to patients, the health system and to society. Yet, each time, this was a problem of perception and time, not of value; over a long period of treatment significant health benefit was accrued, not to mention significant financial, emotional and personal cost savings to patients, as well as system-wide advantages.

Any treatment for a widespread or expensive-to-treat disease, especially a curative therapy, has impacts beyond the individual patient. An example was seen recently in a study looking at the impact of widespread access to a cure for HCV in the United States, which estimated that over 53,000 life years would be gained in non-HCV sufferers due to the wider availability of livers for transplantation that resulted from the HCV cure⁴. Traditional cost effectiveness studies would not have captured this benefit.

Generic patient-reported outcome measures are insufficient.

Generic patient-reported outcome measures are often a tool for encompassing all of the quality of life benefits that are accrued from a treatment. Yet, there are distinct quality-of-life outcomes associated with cures. For example, studies have shown significant differences in anxiety and stress components of QOL across varying levels of predicted prognoses,⁵ with worsening prognosis being a predictor of poorer anxiety and depression.⁶ Therefore, the value

⁴ Jena AB, Stevens W, Gonzalez YS, Marx SE, Juday T, Lakdawalla DN, Philipson TJ. The wider public health value of HCV treatment accrued by liver transplant recipients. *The American journal of managed care*. 2016 May;22(6 Spec No.):SP212-9.

⁵ Arrieta Ó, Angulo LP, Núñez-Valencia C, Dorantes-Gallareta Y, Macedo EO, Martinez-Lopez D, Alvarado S, Corona-Cruz JF, Onate-Ocana LF. Association of depression and anxiety on quality of life, treatment adherence, and prognosis in patients with advanced non-small cell lung cancer. *Annals of surgical oncology*. 2013 Jun 1;20(6):1941-8.

⁶ Johanes C, Monoarfa RA, Ismail RI, Umbas R. Anxiety level of early-and late-stage prostate cancer patients. *Prostate international*. 2013 Dec 1;1(4):177-82.

of a cure may not be fully encompassed in a tool measuring value only during, and immediately after, treatment.

On top of this, any quality-of-life measure will inevitably be applied only to people being treated. For those who may be at risk of the disease or even awaiting confirmation of diagnosis, knowledge of the existence of a cure and its impact on quality-of-life will have considerable impact on the anxiety and stress suffered over this period. Although ICER favors a relatively narrow definition of the scope of benefits attributed to a new healthcare technology, it must acknowledge that this conservative approach is insufficient, especially for cures.

ICER should work with foreign collaborators to develop more patient-centered methods for value assessment that do not impede access.

Because ICER has chosen to partner with both the United Kingdom’s National Institute for Health and Care Excellence (NICE) and the Canadian Agency for Drugs and Technologies in Health (CADTH), we hope that they too are participating in this project for the purpose of improving their own assessment methods, as opposed to simply bringing their expertise to ICER. In both the U.K. and Canada, their value assessment methodologies have had serious implications for real patients and people with disabilities seeking access to innovative treatments, with a discriminatory outcome that should never be tolerated in America’s healthcare system.

For example, cancer patients in other developed countries have access to new cancer medicines on average two years later than patients in the U.S.⁷ Even when other health authorities eventually approve new medicines, additional access restrictions, such as limiting treatment durations, continue to create barriers for patients.⁸ Nearly 80% of cancer medicines approved for coverage in the U.K. between 2007 and 2014 had some kind of access restriction.⁹ And patients pay the price for delayed and restricted access to life-saving medicines – five-year survival rates for breast, colon, lung, and prostate cancers are higher in the U.S. than in Canada, France, Germany, Italy, Japan, and the U.K.¹⁰

Karen McLaren and Ashley McDonald

The stark contrast between the experiences of Karen McLaren and Ashley McDonald, two Canadian women who met in elementary school and then worked together in their early twenties, illustrates the profound impact of restricted access to cancer medication. The friends

⁷ IQVIA. Global Oncology Trends 2018, May 2018.

⁸ “Mum with lung cancer taken off life-saving drug,” November 7, 2018. Accessed December 5, 2018. [Web](#).

⁹ Zhang Y et al. [Comparing the Approval and Coverage Decisions of New Oncology Drugs in the United States and Other Selected Countries](#). *J Manag Care Spec Pharm*. 2017;23(2):247-54. [Web](#).

¹⁰ World Health Organization (WHO). WHO mortality database. PhRMA analysis of WHO mortality database, using age-specific death rates. Accessed May 2018. [Web](#).

were both diagnosed with breast cancer as young women and underwent mastectomies and aggressive chemotherapy, only to have their cancer return and metastasize. McDonald, a dual U.S.-Canadian citizen, receives health insurance through her American employer, and was prescribed a drug that ultimately put her cancer into remission.¹¹ McLaren has extended Canadian medical coverage, and the same drug was not a covered drug until April 2018.¹² At that time, the British Columbia Cancer Agency announced a severely restricted coverage policy, covering it only for “post-menopausal women with ER-positive, HER2-negative advanced breast cancer who have had no prior treatment for their metastatic disease.” At 42, this eliminates coverage for McLaren, who has been paying out-of-pocket for the treatment, as do thousands of other women across Canada.¹³

In the U.K., a new treatment was approved for relapsing and remitting multiple sclerosis (MS) but not for primary progressive MS, despite its potential to delay wheelchair use for as long as seven years.¹⁴ Cost-effectiveness assessments, particularly those based on QALYs, tend to undervalue medicines that halt or slow disease progression and where there may be less of a measurable incremental improvement in health.¹⁵

The Ford and Elias Families

Holly Ford is one of those 15,000 Brits unable to receive treatment for her primary progressive MS. Diagnosed in early 2018, the 25-year-old call center worker must already use a wheelchair for long distances and says the thought of further erosions to her independence is “terrifying.”¹⁶ She knows that without treatment, MS will eventually result in further mobility impairments, and that a new drug can delay the progression of the disease. She said the treatment, “won’t make me better... but it could stop me from getting worse.”¹⁷

A new drug brought hope to individuals with spinal muscular atrophy (SMA), a rare, debilitating condition that previously had no treatment for its underlying cause.¹⁸ Unfortunately, because it failed to meet the QALY-based cost-effectiveness review used by the United Kingdom and

¹¹ Ghoussoub, Michelle, “A tale of 2 friends with breast cancer; 1 has coverage for costly drug, the other forced to pay,” Canadian Broadcasting Company, January 20, 2018. Accessed December 5, 2018. [Web](#).

¹² Taminga, Monique, “No celebrating cancer drug coverage, says B.C. woman,” *The Langley Times*, April 17, 2018. Accessed December 5, 2018. [Web](#).

¹³ <https://www.cbc.ca/news/canada/british-columbia/a-tale-of-2-friends-with-breast-cancer-1-has-coverage-for-costly-drug-the-other-forced-to-pay-1.4495123>

¹⁴ Knapton, Sarah, “MS patients denied drug which could keep them out of wheelchair”, *The Telegraph*, September 10, 2018. Accessed December 5, 2018. [Web](#).

¹⁵ Partnership to Improve Patient Care, “Measuring Value in Medicine: Uses and Misuses of the QALY,” White Paper. Accessed December 5, 2018. [Web](#).

¹⁶ Knapton, Sarah, “MS patients denied drug which could keep them out of wheelchair”, *The Telegraph*, September 10, 2018. Accessed December 5, 2018. [Web](#).

¹⁷ Knapton, Sarah, “MS patients denied drug which could keep them out of wheelchair”, [The Telegraph](#), September 10, 2018.

¹⁸ National Organization for Rare Disorders, “Spinal Muscular Atrophy,” Accessed December 5, 2018. [Web](#).

several other national health systems, it remains unavailable to many patients seriously in need.

The Newell Family

Finley Newell, from Haddenham in Buckinghamshire, has spinal muscular atrophy, which means he can't walk and a common cold could be enough to kill him. A drug which could reverse his illness already exists but the NHS initially argued it is not value for money. His mother called the decision "an abomination" and "discrimination." A former nurse said, "It's incredibly cruel to use the cost-effectiveness line and it makes me shake with rage."¹⁹

There are more stories we could tell which emanate from a disconnect between the value attributed to innovation by value assessments and the value to patients and people with disabilities. We do not want to go down the path that other countries have embraced where treatment is covered for one population and not another because it is "cost effective" for one and not the other. For example, there are countries where treatment for SMA is covered for children, but not for the adults who have spent their lives dreaming of the day a cure emerges simply because the cure will not reverse their disease progression so they are deemed not worth it. There are countries that will cover a cancer drug for patients that haven't received other treatments, leaving patients behind for whom the treatment shows evidence to be more effective but were diagnosed before it was available. In America, these patients should know they are worth treating, or even curing, regardless of the stage of their disease.

Ongoing Efforts to Improve Value Assessment

It has been widely acknowledged that traditional methods for value assessment fall short on standards for patient centeredness, as outlined by the National Health Council's Patient-Centered Value Model Rubric, particularly when it comes to incorporating the full range of outcomes that matter to patients and acknowledging heterogeneity among patient populations. Promising practices are emerging from other organizations and approaches to value assessment that better reflect principles of patient-centeredness including the following:

Center for Patient-Driven Value Assessment (PAVE): Academics at the University of Maryland are working directly with patient representatives to adapt cost-effectiveness analyses to explicitly account for patient goals and preferences. Development of more patient-driven methods by PAVE will advance the utility of economic evaluations for coverage decisions. PAVE also created a program to expand patient-community capacity to meet growing demand for representatives engaged in value research, economic model development, and value assessments.

¹⁹ Blanchard, Sam, "Parents beg officials to approve the life-saving £450,000 drug which can treat the rare condition crippling their five-year-old son", The Daily Mail, October 19, 2018.

Multi-Criteria Decision Analysis (MCDA): MCDA offers a comprehensive and more flexible alternative to conventional cost effectiveness analysis to inform coverage decisions by explicitly accounting for multiple criteria and preferences for available alternatives that are characterized by multiple, sometimes conflicting, attributes. MCDA can be most helpful when there is a need to combine data with subjective judgements involving multiple decision-makers or stakeholders. For example, therapeutic options have a number of criteria to consider such as efficacy, safety, tolerability, and cost - each of these criteria may have a different level of importance to certain stakeholders such as patients, providers, and health insurers.

Innovation and Value Initiative (IVI): IVI has begun developing models that are open-source and are based on a range of approaches including augmented cost-effectiveness analysis and multi-criteria decision analysis. We are impressed that IVI is developing a platform to facilitates robust and rigorous patient-centered value assessment of health technologies tailored to the needs and interests of individual decision makers. Their research is focused on improving care for the patient and efficiency across the health care system and they are utilizing existing disease-specific model to demonstrate that interventions that maximize health outcomes at a population level may not be the best choice for a specific individual. These findings will help focus value assessment to optimize the outcomes achievable with available treatments given heterogeneous patient populations rather than focusing on the best treatment for an average.

Conclusion

We very much appreciate being given the opportunity to express our very real concerns about the limitations of current approaches to estimating the value of new innovations, and cures in particular, and the very real harm that can be done if the value of potential cures are not measured to capture their holistic value for patients, people with disabilities, families, and society.

Sincerely,



Tony Coelho
Chairman, Partnership to Improve Patient Care