

April 1, 2019

Ms. Jennifer Bright
Executive Director
Innovation and Value Initiative
2 Bethesda Metro Center, Ste 850
Bethesda, MD 20814

Dear Ms. Bright:

The Partnership to Improve Patient Care (PIPC) is pleased to provide the following comments related to the Innovation and Value Initiative's (IVI) first oncology-specific Open-Source Value Platform (OSVP) model focused on non-small cell lung cancer (NSCLC). We are impressed by the detail and comprehensiveness of the model. We understand that producing such detailed information takes significant time, effort and expertise, and we appreciate that IVI is invested in developing such high-quality reports. There is tremendous value to an open-source model, allowing for a more robust review and understanding of its methods. It is also very useful that IVI presented both a basic and advanced model to allow those outside academia and health economics to review the information.

Nevertheless, we oppose reliance on the use of quality-adjusted-life-years (QALYs) and look forward to IVI being part of the solution to advance methods that do not rely on QALYs. Because people with disabilities, seniors, and patients with chronic conditions may experience a potential for health that is lower than their "healthier" counterparts, treatment that extends or improves their life may result in fewer QALYs than a treatment developed for a non-disabled or younger population that is able to return the patient to so-called perfect health. As QALYs are assigned by both quality as well as quantity of life, an incremental QALY assessment would prioritize providing treatment to a non-disabled population with a longer theoretical life expectancy, and otherwise perfect health, over a population with a disability or chronic condition. It is our hope that IVI chooses to be innovative in moving beyond the QALY.

The multi-criteria decision analysis (MCDA) component of the model is a more patient-centered method than others for assessing the value of treatments.¹ In the MCDA, IVI allocates weights to different components of value thus making the results more transparent and consistent. This method of assessment is particularly well-suited for evaluating drugs for rare diseases, where improvements in the status quo present tremendous value for patients and people with disabilities. Capturing this information is useful to impress upon decision makers the value of treatments, especially orphan drugs.² In the real world, health care decisions are complex, a reality that is better articulated under an MCDA model.

We would provide to IVI the following suggestions and comments:

¹ Garatini L, Padula A. Multiple criteria decision analysis in health technology assessment for drugs: just another illusion? *Appl Health Econ Health Policy*. 2018;16:1-4.

² McCabe C, Tsuchiya A, Claxton K, Raftery J, et al. Assessing the economic challenges posed by orphan drugs: a comment on Drummond. *Int J Technol Assess Health Care*. 2007;23:397-404.

- **QALYs:** As discussed, PIPC has significant concerns with IVI using QALYs in what is an over-simplistic two-dimensional value framework. To be innovative, it is necessary to move beyond cost-per-QALY to a tool that incorporates the multitude of facets going into health policy decision making, such as IVI has outlined in the MCDA framework. We would advocate for innovation in value assessment to incorporate disease-specific measures of health-related quality of life and to incorporate patient preferences in a quantifiable, systematic way.
- **Expert Panel:** PIPC would appreciate increased clarity on the role of the expert panel and how their input was incorporated into the final structure of the model.
- **Treatment Cost:** PIPC appreciates that IVI incorporated recent cost data sources from 2015-2018, all from U.S. studies. We would like to better understand why the model assumes a flat mean cost throughout the treatment. We are concerned that in the real world, the mean overall monthly non-drug cost of the last 6 months of life will typically be higher than the mean overall monthly non-drug cost of the last 12 months, and that this difference might be significant when carried over into actual differences in survival across treatments.
- **Utility weight source data:** PIPC would like to better understand why the model did not source all health states from the same study, rather than use one study for one health state³ and another study for another health state.⁴ Additionally, PIPC is concerned about the risk of underestimation of the effectiveness of therapies from the categorization of continuous outcomes which can be an over-simplification of often more nuanced data. It also seems the utilities were translated from health states derived from studies that have not actually evaluated the drugs under investigation, another form of simplification of outcomes that could undermine actual differences at the margin. We would also suggest soliciting patient input on the assumption that utilities do not vary across treatment strategies.
- **Discounting:** PIPC is concerned that the model uses equivalent discount rates for costs and benefits. We would suggest that there is enough theoretical and empirical weight behind differential discounting for this to be the default for more progressive organizations like IVI.⁵ At a minimum it should be possible to offer differential discount rates as an alternative set for sensitivity analyses, particularly given that this choice can so significantly alter the cost-effectiveness of a sequence.
- **Productivity estimates:** Current estimates of productivity costs are limited to absenteeism (days off of work), whereas recent studies have suggested that *presenteeism* can have a bigger effect than absenteeism — or at least as large.⁶ Presenteeism is where sick people

³ Nafees B, Stafford M, Gavriel S, Bhalla S, Watkins J. Health state utilities for non small cell lung cancer. Health and quality of life outcomes. 2008 Dec;6(1):84.

⁴ Nafees B, Lloyd AJ, Dewilde S, Rajan N, Lorenzo M. Health state utilities in non-small cell lung cancer: an international study. Asia-Pacific Journal of Clinical Oncology. 2017 Oct;13(5):e195-203.

⁵ Claxton K, Paulden M, Gravelle H, Brouwer W, Culyer AJ. Discounting and decision making in the economic evaluation of health-care technologies. Health economics. 2011 Jan;20(1):2-15.

⁶ Schultz AB, Edington DW. Employee health and presenteeism: a systematic review. Journal of occupational rehabilitation. 2007 Sep 1;17(3):547-79.

come to work but are less productive when they are there, as compared to not coming in at all. Productivity losses based on absenteeism alone will undoubtedly underestimate the total cost of productivity losses of any health state or disease.

Overall, PIPC finds the IVI model and reporting mechanisms to be superior for value assessment than other models, although the use of QALYs is troubling. We are hopeful that IVI will move beyond QALYs and generate additional models relying on methods such as MCDA in the future as there are many more areas of treatment that would benefit from a robust and comprehensive assessment.

Sincerely,



Tony Coelho
Chairman, Partnership to Improve Patient Care