Asthma and Allergy Foundation of America

October 15, 2019

Steven D. Pearson, MD, MSc
President
Institute for Clinical and Economic Review
Two Liberty Square, Ninth Floor
Boston, Massachusetts 02109

Dear Dr. Pearson:

The Asthma and Allergy Foundation of America ("AAFA") thanks the Institute for Clinical and Economic Review ("ICER") for the opportunity to comment on ICER's proposed updates to the 2020 Value Assessment Framework. AAFA appreciates ICER's willingness to engage with us and to better understand patients' perspectives. We believe that thoughtful inclusion of patient experience data is essential to accurately reflect the true impact, and therefore "worth," of new and evolving treatments. We offer the following comments on the current proposal:

Modified Social Perspective: In our initial comments in response to the Framework's revisions, we urged ICER to include the modified societal perspective as part of the base analysis to more accurately reflect the patient perspective. Particularly but not solely in the context of food allergies, direct medical costs are just one component of the impact, and focusing on direct medical costs for the base case analysis with a modified societal perspective in the sensitivity analysis seriously misrepresents the value of a treatment for any food allergy. We understand that the direct medical costs are of interest to many stakeholders (payers, in particular) and should be explicitly reported. However, we agree with the Second Panel on Cost Effectiveness that cost-effectiveness analyses should report two reference case analyses, one on the health care perspective and another the societal perspective, and produce an impact inventory to aid in decision-making.[1]

We appreciate that ICER acknowledged and responded to this input from AAFA and other commenters. However, we are disappointed that ICER proposes to continue to use the health system perspective for its default base case. ICER notes only one category of exceptions:

- As per our methods adaptations for treatments of ultra-rare diseases, however, when the societal costs of care for any disease are large relative to the direct health care costs, the societal perspective will be included as a co-base case, presented directly alongside the health care sector perspective analysis.

[1] Sanders GD, Neumann PJ, Basu A, et al. Recommendations for Conduct, Methodological Practices, and Reporting of Cost-effectiveness Analyses: Second Panel on Cost-Effectiveness in Health and Medicine. *JAMA.* 2016;316(10):1093–1103

We are concerned that this application of a societal perspective is both unduly narrow (limited only to ultra-rare diseases) and vague (societal costs "large relative to" healthcare costs). Using a societal perspective as one of two reference case analyses for *all* treatments where relevant societal data is available would provide a more robust and meaningful approach.

Customized Data Sets: As ICER noted in the draft proposal, multiple stakeholders argued for the inclusion of real-word evidence in ICER's analyses. AAFA had been one of these stakeholders; we argued that, when available, real-world healthcare data, including claims and enrollment data sets, should be used to estimate the potential patient population and treatment effectiveness. We appreciate that ICER is committing to continuing to use high-quality real-word data where available, and that ICER proposes to identify opportunities to generate new real-world evidence when appropriate.

Sensitivity analyses: In our earlier comments, we encouraged ICER to run sensitivity analyses using multiple scenarios when appropriate. As noted in our asthma letter, we found that when we combined variables to assess a range of scenarios, relatively modest changes in ICER's cost and utility assumptions had a significant impact on cost per QALY. We appreciate that ICER has proposed adding a section on "Controversies and Uncertainties" to the cost-effectiveness section of its reports in order to reflect alternative assumptions and models proposed by stakeholders, including but not limited to a manufacturer. However, we remain concerned that ICER's approach will not sufficiently reflect the sensitivity of its models to multiple overlapping variables. In addition to the important step of identifying "Uncertainties" in its model, ICER should proactively run sensitivity analyses that reflect the impact of multiple variables. This information should be presented in a way that is clear and accessible to stakeholders.

Caregiver Burden: As AAFA has commented on multiple occasions, food allergies affect a whole family – as do nearly all health conditions. As we noted in the peanut allergy treatment letter, analyses should reflect not only potential diminished burden on caregivers, but should also reflect potential quality of life gains attributed to the caregiver, in order to reflect the true societal value of treatment. We encouraged ICER to fully reflect caregiver burden and potential benefits of interventions for caregivers in future analyses.

In the current proposal, ICER acknowledges such concerns, but argues that data on caregiver quality of life is limited and has key areas of uncertainty, including which family members to consider in such an analysis, and whether caregivers adjust to their burdens over time. Given the importance of caregivers and the enormous value of their quality of life along with the whole family's quality of life, AAFA respectfully requests that ICER take a stronger stance on caregiver quality of life research, perhaps developing collaborations – as ICER proposes to do with regard to real-world-evidence overall – to define research needs and to generate knowledge in this area. In the meantime, we strongly support ICER's proposal to use caregiver utility impact data when available.

Conclusion

ICER has an opportunity to expand its inclusion of the patient perspective and to lower both economic and quality of life costs for patients and their families. To do so, in addition to building relationships with patient groups, ICER should incorporate the patient perspective as part of their base-case economic analyses; use appropriate real-world data sets for their analyses to reflect the actual patient community;

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meaningfully present alternative assumptions and sensitivity analyses; and work to incorporate and grow the data on caregiver impact and quality of life.

Thank you very much for your time and attention. We look forward to continuing to work with ICER to incorporate the patient and family perspective in your analyses.

Sincerely,

Kenneth Mendez
President and Chief Executive Officer
Asthma and Allergy Foundation of America

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