

**Asthma and Allergy Foundation of America (AAFA)**  
**Comments Regarding the Institute for Clinical and Economic Review (ICER) Draft Report on Nucala®**  
**(Mepolizumab) for Asthma**

### **Key Issues and AAFA's Recommendations**

We offer comments that address specific issues in the ICER report as well as comments that we believe may improve future analyses. As an organization that represents the voice of patients with asthma and allergic disease, our comments reflect our underlying belief that patients, and the patient perspective on matters of value, are recognized as important inputs to research and analysis relevant to asthma, and given appropriate weight in (for example) analyses that speak to the value of alternative approaches to care. As a result, our comments focus on:

- Assuring that the patients' voice is adequately captured and appropriately weighted in analyses of value;
- Assuring that analysis of the cost part of the value equation reflects costs as patients perceive them;
- Recognizing that we do not lose sight of the opportunities that exist to improve outcomes for patients with asthma, through improvements in "Standard of Care."

### **Capturing the patients' voice in analyses of value**

AAFA believes that analyses of value must begin with an understanding of the outcomes that matter most to patients, and that that, in turn, implies the need to capture and weight patient-sourced data that describe outcomes that patients agree are relevant to them.

Given that, we want first to communicate our concern that the framework that we assume guided this work (and will guide ICER's work in the future) appears to have been constituted with little input from patients. We note that the group that provided input to the development of the ICER value assessment framework<sup>1</sup> included six payers and eight organizations representing manufacturers—but only one organization representing patients. And we believe, additionally, that that organization—Families USA, one that we respect greatly—would agree that it does not have the deep understanding of the needs and values of people with asthma, to be able to inform an assessment of value that adequately reflects patient needs.

We acknowledge that patient outcomes data were included in the ICER analysis—as these data were collected in the clinical trials upon which the work draws. That said, we are concerned that the impact of these inputs to the analysis may have been limited, given that that analysis appears primarily to have been configured to assess outcomes as payers see and value them.

Given these considerations, we recommend that:

- In revisions to this report and in analyses that ICER and others undertake in the future, efforts are made to bring the voice of patients more directly to the design of the methodology. AAFA is fully prepared to assist in those efforts.
- An effort is made to further evaluate the set of instruments available to capture patients' perspectives on Quality of Life—or the more general set of outcomes that are relevant to how patients with asthma perceive the value of care—and that the output of that evaluation guides the use of instrumentation for future analyses. Clearly, as ICER's (secondary) analyses often depend upon data collected by others for other (primary) analyses, it will be important to drive the use of preferred instruments into those primary analyses

(e.g., industry-sponsored clinical trials). We believe this is consistent with the intent of the FDA’s Patient-Focused Drug Development initiative, and will serve to accelerate efforts to realize the intent of that initiative. We note, finally, that our recommendation seems to be well aligned with a group of experts called together to evaluate asthma Quality of Life instruments, whose summary in 2012 included “(r)esearch is strongly recommended to develop and evaluate instruments that provide a distinct, reliable, measure of the patient’s perception of QOL, and important outcome that is not captured in other outcome measures.”<sup>2</sup> Perhaps there is an opportunity to leverage the progress this group made to move this important work forward.

To the extent that that evaluation of current instrumentation reveals gaps—issues of great importance to people with asthma that are not adequately assessed by any instrument currently extant—we recommend investment in the development of measures and instruments that accomplish that assessment; and the expedited use of them in analyses that attempt to assess “value.”

AAFA is fully prepared to participate in national efforts to assure that there are valid and reliable methods to assess the outcomes that matter to patients with asthma, and in fact eager to bring its registry development efforts to support the capture of data that a broad group of stakeholders (including patient organizations like AAFA, but also methodologists, researchers, providers, payers and manufacturers) suggest may be required to bring the patient voice reliably and cost-effectively to this work.

### **Analyzing costs as patients experience them**

The ICER report evaluates cost from the perspective of the health care payer, including the perspective of society that ultimately must make decisions not only about investments in health care, but investments in other goods and services that matter to Americans. Without disputing the importance of these perspectives, we want to make clear that the costs that patients with asthma face—and therefore the “value” that mepolizumab may or may not represent to them—are quite distinct from the costs that payers and society as a whole face.

It is important both that the report (and future reports like it) make that clear, and make some effort to consider the out-of-pocket costs that patients with asthma face. We recommend that:

- The report specifically calls out the issue of cost from the patient perspective and, as possible, includes data that speak to the costs that patients face. ICER is to be commended for including Appendix C that provides data about payer coverage policies. To the extent that it were possible to translate those policies into “expected” (or “typical”) costs for patients with eosinophilic asthma, readers of the report may have a much better sense of the challenges that those who are expected to benefit from mepolizumab are likely to face, realizing that benefit.
- ICER consider supplementing its outputs, to include models where the cost inputs are the costs patients face (rather than the costs payers face). Acknowledging that the level of effort here may be non-trivial, we believe that it would provide important information—and send an important signal—about value as patients who are expected to benefit from mepolizumab see it.
- There be additional exploration of issues related to the total economic burden of asthma, and on options to address that;
- There be additional public policy research, to explore options regarding prescription drug pricing that will promote affordability while continuing to provide the incentives to manufacturers that are needed to stimulate further innovation;
- There be investment in efforts to develop tools to help patients understand the cost and value of different therapeutic options available to them;
- That there be continued investment in efforts to assure coverage and reimbursement for evidence-based treatments beyond pharmaceuticals:

- Treatment options in accordance with the Guidelines for the Diagnosis and Management of Asthma (Expert Panel Report 3, EPR-3);
- Educational and preventive services conducted by clinicians, health educators, and other health and allied professionals both within and outside of the clinical setting;
- Home-based multi-trigger, multicomponent intervention and prevention services to help reduce and/or remediate asthma triggers in homes and schools.

### Improving the “Standard of Care” (SoC)

We are struck by a finding in the report (that we acknowledge is not central to it), and by a significant literature that establishes that—for patients with asthma as with so many other chronic conditions—there is a gap (or a chasm) between what we **know** and what we **do**: between the outcomes that could be achieved if the science that we have were consistently and reliably implemented, and the outcomes we observe in the real world.

The finding in the report that we would like to highlight (on page 16 of the Executive Summary) relates to the observation that “there was a marked decrease in the annual rate of asthma exacerbations in the placebo group of the MENSA trial;” in fact, that “marked reduction (was) greater than the difference...between the mepolizumab and placebo groups.” While the authors note—and we agree—that we may be seeing some regression to the mean here, they also suggest the possibility that this improvement may reflect “optimization of the standard of care, highlighting the potential benefits of greater attention to maximizing adherence to standard therapy in patients with severe asthma.”

We believe that point needs to be called out and further discussed, and would recommend that it receive further attention in the final report. In the national conversation about new and high cost drugs, we are concerned that others may lose sight of the fact that there is the opportunity to make investments in what may be very cost-effective policies, strategies and tactics that do “no more than” improve “Standard of Care” for patients with chronic conditions—and that these represent an important alternative track to creating value. For asthma, that may mean:

- Investing in patient education and tools and strategies that promote more effective patient self-management;
- Investing in—and reimbursing for—strategies and tactics that reduce exposure to triggers (for example, home assessment and remediation);
- Revising payment policies, to maximize access to lower cost treatments which would lead to improve outcomes—if patients could afford them;
- Developing a deeper understanding, and solutions based on that deeper understanding, about what barriers to care patients with severe asthma face.

While recognizing the strength of the work summarized in the report, AAFA believes that there are opportunities to increase its relevance and value. In particular, we have highlighted in our comments ways that we believe the report can help to capture—or at least to signal the importance of capturing in future reports—the patients’ perspective on both the cost and the quality/outcome elements of the value equation. And we have highlighted the opportunity for the report to consider the potential value of an obvious (but often unconsidered) use of funds; namely to improve “Standard of Care” through a set of interventions that are likely to prove to be quite cost effective.

<sup>1</sup> <http://www.icer-review.org/wp-content/uploads/2014/01/Slides-on-value-framework-for-national-webinar1.pdf>; slide 4.

<sup>2</sup> Wilson SR, Rand CS et al. Asthma Outcomes: Quality of Life. J Allergy Clin Immunol 2012; 129: S88-S123.