

September 12, 2016

Steven Pearson, MD, MSc
President, Institute for Clinical and Economic Review
Boston, MA 02109 USA

RE: ICER National Call for Proposed Improvements to Value Assessment Framework

Dear Dr. Pearson,

On behalf of the 52.5 million adults and more than 300,000 children in the United States with doctor-diagnosed arthritis, the Arthritis Foundation appreciates the opportunity to provide comments to ICER on the *National Call for Proposed Improvements to Value Assessment Framework*. The term arthritis encompasses more than 100 rheumatic diseases and conditions that affect joints, the surrounding tissue and other connective tissues. Arthritis is a complex, chronic disease that is often difficult to treat because it is systemic and can affect multiple organs. As such, each person with the disease has a unique set of experiences with symptoms and treatments. People with RA often do not fit the profile of an “average patient,” which can make comparative effectiveness analyses and value frameworks difficult to design for this population. A treatment that works well in one patient might not work in another patient with seemingly identical disease characteristics, leading to two critical conclusions: a robust cross-section of patient representation must be included in the design of value frameworks and policy decisions; and patient and prescriber access to a broad range of treatment options must be available to adequately treat people with this disease.

The Arthritis Foundation believes robust stakeholder engagement is a critical component of any value framework that will have a direct impact on people with arthritis and the providers who treat them. Information from clinicians who have daily contact with this patient population is also an important component of developing a robust stakeholder engagement process. We hope our comments will inform ICER’s value framework methodology and help to ensure the treatment needs of the people who suffer from arthritis are met. Please find our specific comments on the value assessment framework in the subsequent sections.

Timeline. We appreciate the opportunity for patient advocacy group engagement in the comment process. However, we are concerned that the comment deadlines are too short for many patient and provider groups. We understand that ICER’s timeline is based on the Food and Drug Administration’s review and approval process, in addition to payers making coverage decisions. We urge ICER to re-evaluate the processes and timelines given to the patient advocacy and provider communities for feedback. Allowing more time for comments would allow for more detailed patient-centered input.

Value Assessment Inputs. The Arthritis Foundation is concerned that patient advocacy groups were not adequately represented during the original framework creation and we urge ICER to

engage patient advocacy groups for input in the future. Hearing one patient story is only one patient story and patient advocacy organizations are in a unique position to gather robust data about the patient experience. As an advocacy organization, the Arthritis Foundation maintains regular contact with a broad cross-section of patients, and uses this data to inform public policy affecting people with arthritis. The key to success in ensuring health policy reflects the needs of patients is routine interactions with the people living with the disease through mechanisms such as patient surveys, focus groups and story-banking. Like many patient advocacy organizations, the Arthritis Foundation routinely engages with people with arthritis in these ways and wants to be a valuable resource to ICER. In order to fully integrate the patient perspective, it is critical to forge patient partnerships, provide transparency to patients, include patient perspectives, acknowledge the diversity of patient populations, include outcomes important to patients and utilize patient-centered data resources. In the current value framework, ICER includes areas for descriptive/qualitative data input, and we ask that ICER elaborate on how patients, caregivers and providers will be engaged, what methods will be utilized and how that input will be presented quantitatively. For these reasons, we urge ICER to incorporate feedback from patients, patient advocacy organizations, care givers, and provider panels in the design of the value framework and throughout the evaluation process.

Comparative Clinical Effectiveness. The current value framework states that patients will inform opportunities for using or generating real world evidence (RWE). We urge ICER to provide more transparency on the methods by which patient groups will be involved in the comparative clinical effectiveness category of the framework. We are concerned that the current scientific literature does not adequately incorporate patient, caregiver and provider perspectives. ICER should rely on additional means for capturing information, for example by partnering with patients and patient groups who can provide robust information on the patient experience. The Arthritis Foundation would welcome the opportunity to partner with ICER on this initiative.

The Arthritis Foundation supports the collection of meaningful data for metrics with an emphasis on the quality of the evidence. We recommend that data from patient reported registries (PROs) should also be part of comparative clinical effectiveness, with robust input from patient and provider stakeholders.

Incremental Costs. ICER has deemed a standard of 100-150k per quality adjusted life years (QALYs). We urge ICER to consider whether this calculation is generalizable, and seek further clarification on the specific processes used to calculate QALYs for different populations and disease groups. As ICER continues to refine this section of the value framework, we encourage you to develop mechanisms to determine whether a QALY and the subsequent data are from relevant patients and populations. Relying on population-based assessments that do not reflect the heterogeneity of disease subpopulations, patient treatment responses and patient preferences increases the risk of mischaracterizing the value of the treatments being compared. ICER should recognize that no single QALY threshold estimate can or should be generalizable to all populations, and that QALY thresholds vary by decision-maker, population and disease. Further, we seek clarity on how specific indirect and direct costs and caregiver costs are calculated. Many

patients with arthritis also suffer with comorbidities such as cardiovascular disease, mental health conditions, infections and malignanciesⁱ. Of adults diagnosed with arthritis, 47% also have at least one of the previously listed conditions and as many as 40% of people with rheumatoid arthritis (RA) experience significant symptoms of depression, which can lead to more physical function problems, higher disease activity, poorer health overall and an increased need for medical careⁱⁱ. We urge ICER to elaborate on how comorbidities will be accounted for in the incremental costs outcome measures, including disability, quality of life, mental health and mortality.

Other Benefits or Disadvantages. Objectivity is important in any evaluative process. ICER mentions the use of independent public appraisal committees; we seek clarity on who comprises the committees and the methodology they utilize. We are concerned this approach may be insufficient to incorporate the impact of important patient-centered factors, such as prescribing patterns, treatment adherence, patient preference and work limitations. The voting panel may not have the expertise or appropriate context to meaningfully evaluate this category. We urge ICER to develop a formalized patient engagement process, including patient and provider panels that will engage and provide feedback during all facets of the framework.

Treatment adherence and off-label prescribing are major issues for patients. A study by Cush and Dao (2012) found that off-label use (OLU) accounted for 21% of all drug use. Further, agents closely aligned with rheumatologic care including immunosuppressant's, gabapentin, corticosteroids, anti-spasmodics and sleep medicines were often prescribed off-label. For groups like oncology, it is estimated that 50-60% of patients are using drugs off-labelⁱⁱⁱ. We seek clarity on the process ICER will use to account for off-label prescribing and medication adherence.

Patient preference must be taken into account when evaluating treatment value, as there are multiple reasons a patient may prefer a certain drug. For example, some patients are not able to self-inject, and therefore prefer infused drugs. The many factors contributing to medication adherence should also be taken into account, and range from affordability to access issues for people living in rural areas, drug synchronization for people on multiple drugs and a lack of education about the importance of completing a drug regimen.

More than 43.2% (22.7 million) of the 52.5 million adults with doctor-diagnosed arthritis report limitations in their usual activities due to their arthritis. Further, 31% (8.3 million) of working age adults with doctor-diagnosed arthritis report being limited in work due to arthritis^{iv}. Therefore, we seek additional transparency on methods used to judge worker productivity not included in the comparative effectiveness category.

Contextual Considerations. These considerations include ethical, legal or any other issue that influences the relative priority of illness and interventions. We seek clarity on the methods ICER will utilize when new classes of drugs like biosimilars come onto the market. Two biosimilar medications for RA have already been approved by the FDA, and these drugs may come to market as soon as the fall of 2016. Robust details explaining how the framework will be revised

when biosimilar introduction occurs is needed to understand the full economic impact of disease groups and medications. Again in this category, ICER mentions the use of independent public appraisal committees, and we seek clarity on who comprises the committees, the methodology they utilize and how the patient voice will be integrated and documented in these deliberations.

Care Value. Keeping patients stable on the right medication is critical to maintaining positive health outcomes and greater productivity for patients. There are parts of the care value equation that could threaten a medically stable patient, such as the absence of patient, caregiver and provider input as well as the non-disclosure of the precise equations used in the evaluation. We seek full transparency on the equations used to calculate care value, specifically how ICER will quantify patient involvement and impact as it relates to the significant benefits or contextual factors in the care value calculation.

Budget Impact. Focusing budget impact on the short-term payer timeline can result in patients having restricted access to life changing treatments. We urge ICER to consider long-term outcomes and impacts from the patient and payer perspective. In some instances, cost savings may not be realized for several years, as with the introduction of biologic medications. Biologics for arthritis did not immediately show cost savings. From 1993 to 2008, after the introduction of biologics to treat inflammatory diseases, there has been a 32% reduction in joint replacement procedures^v. For people with chronic disease, their disease state does not exist in 1-year increments; therefore the current approach to assessing budget impact is too narrowly focused and can miss potential cost savings over a lifetime horizon.

Value-Based Price Benchmarks. Calculations in this category of the ICER framework are based on the list price of drugs. The list price does not adequately reflect the actual price paid for the drug. Further clarity is warranted on the specific methods used to calculate the discount and rebate rates. We urge ICER to consider third party data and existing research when available to help provide a more realistic estimate of the industry-wide discount rate.

Other Frameworks. The value discussion is of vital importance to patients who are directly challenged by barriers to treatment and limitations in their daily life that impact quality of life. The majority of current value frameworks aim to help payers with formulary decision-making and may leave out the patient and caregiver perspective when determining value. For a framework to be comprehensive, it is critical to incorporate robust patient input, especially if these models are used to inform decisions that affect access to treatments for patients. We are concerned that the methodology for the incorporation of the patient voice is unclear in the current ICER framework. We urge ICER to not only provide a clear process for integrating the patient voice, but to also work with patient advocacy organizations and others such as the National Health Council (NCH) to ensure the updated framework is comprehensive.

Overall, the Arthritis Foundation wants to ensure people with arthritis have access to the medications they need to function in daily life. Attempting to make decisions about the value of a drug without robust supporting data from patients and providers in daily contact with patients

is a questionable practice. We ask that ICER consider its current process to evaluate and make decisions regarding treatments. Further, as new treatments and more robust information about these treatments becomes available, we urge ICER to consider a protocol for how these decisions will be revised in the future. The Arthritis Foundation cannot support any value based recommendations that could result in a patient on a stable drug no longer having access to that drug. To that end, we urge ICER to consider the critical need for adherence to drug regimens, and the perspective of patients, caregivers and other stakeholders to ensure that their working value assessment framework has the broadest possible relevancy.

Again, thank you for the opportunity to comment on the *National Call for Proposed Improvements to Value Assessment Framework*. Please contact Sandie Preiss, Arthritis Foundation National Vice President of Advocacy and Access, at 202-887-2910 or spreiss@arthritis.org with questions or for more information.

Sincerely,



Sandie Preiss
Vice President, Advocacy and Access
Arthritis Foundation

ⁱ Centers for disease control and prevention. (2015). *Comorbidities*. http://www.cdc.gov/arthritis/data_statistics/comorbidities.htm.

ⁱⁱ Arthritis Foundation. (2016). In *Rheumatoid Arthritis and Depression*. <http://www.arthritis.org/living-with-arthritis/comorbidities/depression-and-arthritis/depression-rheumatoid-arthritis.php>

ⁱⁱⁱ Cush.J.J.,Dao,K.H. (2012). *Drug Safety Quarterly*. 4 (3). from. <http://www.rheumatology.org/Portals/0/Files/DSQ%20Fall%202012.pdf>

^{iv} Centers for disease control and prevention. (2016). In *Disabilities and Limitations*.,from http://www.cdc.gov/arthritis/data_statistics/disabilities-limitations.htm

^v Guy,D.,Tandon,N.,Waters. C.H., Gunnarsson.C.,Kavanaugh.A., (2014) *Am J Pharm Benefits*. 2014;6(6):256-264.,from http://www.ajpb.com/journals/ajpb/2014/ajpb_novemberdecember2014/rheumatoid-arthritis-and-joint-replacement-impact-of-biologics