Value in the States Principles

Patient engagement in health care leads to better health outcomes, better care, and lower costs. Value assessment methodologies and other efforts to lower health costs while preserving or improving quality must prioritize value to the patient and put optimal health outcomes front and center. Increasingly, state legislators and regulators are looking to policies that use value assessments to lower prescription drug costs. We believe patients are underrepresented throughout the value assessment enterprise and that their active, meaningful engagement in health care decisions from clinical trial design all the way to treatment decisions would improve system-wide health care quality and outcomes while achieving significant progress towards the goal of lowering costs. By prioritizing patient preferences and outcome goals, health care stakeholders will better align both immediate and long-term care needs, which will ultimately help patients receive optimal care and reduce unintended costs.

Recognizing the impact these issues can have on patient access and the need for patient representatives to be a meaningful partner in the full continuum of value assessment, the following principles have been adopted by a collaboration of patient groups across a broad range of therapeutic areas.

1. **All stakeholders should engage in meaningful patient engagement to inform their processes.** Patients and patient groups should be highly engaged by those with a touchpoint with value assessments, from pharmaceutical manufacturers to payers to policy-makers considering cost effectiveness policies. Patients should be engaged in the drug development process to ensure that data collected in clinical trials are relevant to real-life patient needs and experiences. Representatives that reflect the diverse patient perspectives should be invited to serve on Pharmacy & Therapeutics (P&T) Committees**.** Any advisory committee considering cost-effectiveness should include robust and representative patient participation. Patient representatives should be invited to assist in crafting value assessment/cost-effectiveness strategies.
2. **Patient experience and preference metrics must be utilized and prioritized in cost-effectiveness methodologies.** Cost-effectiveness methodologies cannot accurately measure value if they do not include patient experience, preference, and outcome metrics. One current standard for measuring value is the Quality Adjusted Life Year (QALY), which does not always capture where a patient is in their disease or treatment journey and therefore may not take into account patient goals, preferences, and impacts. QALYs can be discriminatory by placing a lower value on treatments that extend the lives of people living with disabilities and chronic conditions who inherently cannot achieve “perfect health.” Multiple additional criteria and methods must be used to adequately assess value, such as patient-reported outcomes and registry data; multi-criteria decision analysis; and caregiver considerations.

3. **Value assessments should incorporate real-world data.** Value assessments cannot fully incorporate all of the necessary and relevant data to be truly patient-centered until the treatment being assessed is on market. Clinical trial data are insufficient to capture the heterogeneity of disease and diversity of affected populations**,** market access factors, and other environmental factors that are crucial for understanding inequities and the disparate impact of the treatment on diverse disease populations.
4. **All stakeholders should be transparent about their processes, methods, and utilization of value assessments.** Value assessors, state boards, and others employing value assessments should be transparent about their methods and allow sufficient time for public input throughout the process. Payers should be transparent about how they are utilizing value assessments in their formulary decisions. Value assessors, payers, and others should establish a continuous feedback loop with the patient community to inform post-value assessment decision-making. Value assessments should be updated regularly to reflect changes in coverage, cost, and other factors.

Disclaimer: These principles are intended to inform policymakers and other health care stakeholders as they consider policies and decisions surrounding value assessment issues and are not intended to support any specific legislative or policy initiative.

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Autoimmune Association
American Cancer Society Cancer Action Network
Allergy & Asthma Network
American Kidney Fund
Arthritis Foundation
Asthma and Allergy Foundation of America
Autistic Self Advocacy Network
Cancer Support Community
Chronic Care Policy Alliance
EveryLife Foundation for Rare Diseases
Hemophilia Federation of America
HIV + Hepatitis Policy Institute
Immune Deficiency Foundation
International Cancer Action Network
International Foundation for Autoimmune & Autoinflammatory Arthritis
Lupus and Allied Diseases Association
Lupus Foundation of America
National Eczema Association
National Multiple Sclerosis Society
National Patient Advocate Foundation
National Psoriasis Foundation
Parent Project Muscular Dystrophy
Prevent Blindness
Sick Cells
Susan G. Komen

June 29, 2023

Maryland Prescription Drug Affordability Board
16900 Science Drive, Suite 112-114
Bowie, MD 20715

Dear Members of the MD Prescription Drug Affordability Board:

On behalf of the nearly 60 million American adults and 300,000 children living with arthritis, the Arthritis Foundation would like to provide written comments to the MD Prescription Drug Affordability Board (PDAB). People with autoimmune forms of arthritis often rely on biologic medications to maintain their health, and as these are expensive medications, issues of high drug costs and access are always a top priority.

Our positions are based on the experiences of arthritis patients that come from our extensive archive of patient surveys, focus groups, discussion forums, and other venues in which we've elicited patient experience data. Affordability looms large for people who rely on specialty drugs to manage their disease: in a 2021 survey we conducted, 37% of those surveyed had trouble affording their out-of-pocket costs in the previous year. Of those, 54% said they had incurred debt or suffered financial hardship because of it. Trouble affording out-of-pocket medical expenses had a significant impact on care: 45% delayed refilling a prescription, 41% say their health care worsened, and 41% switched medications as a result.

We would like to provide specific comments on the Draft Cost Review Study Process, in addition to providing our perspective on patient-centered value assessment.

Draft Cost Review Study Process

On Title 14, .04, Request for Information for Cost Review, B, we recommend the PDAB add patient advocacy groups in the list of entities from which it will collect prescription drug information. Though patients and patient groups are not directly involved in manufacturer supply chain processes, the patient experience and the factors that contribute to their ability to access and then remain adherent to their prescription drug could provide vital insights and context to the PDAB as it considers its work. There are multiple data points around benefit design and patient out-of-pocket costs the PDAB seeks from other entities, and we believe gathering the patient perspective on these criteria is an important component to understand the complete impact of prescription drugs in the marketplace. Examples of these data points could include: the impact of utilization management protocols on patient health outcomes; the impact of formulary tiering and cost-sharing structures on patient adherence; reasons why a patient may or may not be able to take a therapeutic alternative; and reasons why a patient may or may not be able to self-administer a medication, among many others.

On Title 14, .05 Cost Review Study, B. Analysis and Data Compilation, we recommend the PDAB include patient registries, patient reported outcomes, and other patient data sets that patient advocacy organizations often collect. The Arthritis Foundation has collected Patient

Reported Outcomes data for the past 5 years and has conducted many surveys in that same timeframe to understand the patient experience with arthritis treatments. For example, in a 2016 study we conducted for an Institute for Clinical and Economic Review (ICER) review of rheumatoid arthritis drugs, over 50% of respondents were required to try two or more drugs before they could receive the drug prescribed by their provider. The same survey showed that patients on average had to try 2 or 3 drugs before finding one that worked for them. These data points showed that formulary decisions like step therapy protocols can have large implications on a patient's disease progression and management and as such ICER indicated in their final report that step therapy is not appropriate in all cases.

On Title 14, C. Factors Considered in Cost Review Study, e. Cost and Comparative Effectiveness Analyses, the draft lists information derived from health economics and outcomes research as sources of data. We urge the PDAB to ensure any health economics and outcomes research study it utilizes meets our criteria for patient-centeredness. This is outlined in our position statement on patient-centered value assessment that is discussed below. For example, studies should be representative of the patient population and include patient experience data to inform their models. We believe health economics studies are not utilizing adequate assumptions if they do not measure factors that matter to patients. When considering value and affordability, there are many factors that impact affordability that may not be captured otherwise, including caregiver expense and/or productivity loss, and the indirect costs associated with the administrative management of health care, which many of our patients have likened to a "full time job."

Patient-Centered Value Assessment

We encourage robust engagement of the patient community in any processes involving affordability reviews and value assessments of drugs. The Arthritis Foundation has engaged in value assessment-related activities since 2016 when we participated in an ICER review of rheumatoid arthritis drugs, and in 2022 we published a position statement on patient-centered value assessment that guides all our activities in this field. Our position statement lays out 6 principles for patient-centered value assessment we urge the PDAB to consider in its deliberations, including:

1. Utilizing patient-centered methodologies. Key points include:

- A widely used approach for estimating quality and quantity of life in economic mode is calculating Quality Adjusted Life Years (QALYs). QALYs may contribute to informing a value assessment. However, data inputs used to calculate QALYs do not holistically reflect patient experiences, preferences, goals and benefit-risk tolerance.
- Current approaches to calculating QALYs often rely on generic questionnaires, which may not reflect health-related quality of life as defined by arthritis patients, nor where patients are in their disease or treatment journey. Further, QALYs can be discriminatory by placing a lower value on treatments that extend the lives of people living with disabilities and chronic conditions. Economic models calculated using QALYs should only be used in combination with other value assessment methods

and should only play a partial role in the comprehensive assessment of treatments. Instead of using a QALY-only value assessment model, we would suggest the following:

- Value assessments must use multiple additional criteria and methods to account for patient preferences, goals and experiences.
- Value assessors and others who utilize QALYs should improve the way in which they use QALYs, ensuring that surveys are disease-specific and given at intervals that are most appropriate for that particular disease. Survey tools should be fit-for-purpose such that policymakers assessing arthritis treatments can evaluate:
  - Was the tool appropriate for arthritis?
  - Did it have questions related to the disease?
  - Did it consider validated joint-specific measurement tools?

- We support the utilization of methods like Multi-Criteria Decision Analysis (MCDA) which can incorporate patient preference data, Patient Reported Outcomes data, and other sources of data that measure value to the patient.

2. Utilizing real-world evidence. Clinical trial data is insufficient to capture the heterogeneity of disease, market access factors and other environmental factors crucial for understanding the impact of the treatment on the disease population. Value assessments cannot fully incorporate all necessary and relevant data to be truly patient-centered until the treatment being assessed is on market. Value assessments should be updated regularly to take into account cost and formulary data, patient-reported outcomes data and any other real-world data that would inform true cost-effectiveness.

3. Utilizing comprehensive claims data, such as all payer claims databases (APCD) to inform models. Robust APCDs can help inform value assessment analyses by providing data across sites of care and longitudinally about patients, allowing value assessors to identify trends and patterns in health care costs and better tailor coverage and cost decisions.

4. Prioritizing transparency. Transparency across the health care ecosystem — from manufacturers to payers, pharmacy benefit managers and value assessors — is essential for implementing patient-centered value assessment. Currently, it is difficult to know the full set of processes and factors that contribute to any given value assessment — and importantly how payers and other stakeholders are utilizing them. We believe value assessors should be transparent about their methods and allow sufficient time for public input throughout the process. We believe payers should be transparent about how they are utilizing value assessments in their formulary decisions. And value assessors, payers and others should establish a continuous feedback loop with the patient community to inform post-value assessment decision making and any subsequent updates.

5. Meaningful Patient Engagement. A truly patient-centered value assessment would engage patients in a meaningful way from start to finish. Key points for the PDAB to consider are:

- Patient engagement should never be considered a check-the-box activity. Instead, patients should be equal stakeholders throughout the process, and patient representatives should have voting privileges in any advisory councils or roundtables.
- The value assessment should not be the beginning or end of patient engagement. Patients should be part of the decision-making process during clinical trial design to ensure manufacturers are measuring endpoints that matter to patients.
- Pharmacy benefit managers and payers should include patients in their formulary review processes to ensure they have a robust understanding of the patient experience. For example, detailed data on the impact of step therapy on patient health outcomes can more precisely guide appropriate step therapy protocols, including the number of steps included in a protocol and the appeals process.
- Manufacturers should incorporate patient preference data in their clinical trial design and should include patients in the identification of study endpoints.
- Patient representatives should be invited to serve on Pharmacy and Therapeutic (P&T) Committees and other forums that determine formulary coverage decisions.
- Any committee or board considering cost effectiveness should include robust patient representation, including voting membership and extensive quantitative and qualitative patient data.
- Patient representatives should be invited to craft value assessment methodologies and strategies, including legislative and regulatory processes and value assessment methodology design.

We urge the MD PDAB to develop a robust, structured process for patient engagement in its activities and decisions going forward. Engaging a diverse set of patients and patient groups can ensure the PDAB has a robust set of quantitative and qualitative patient data to better inform its methods.

6. Value-based Insurance Design (VBID). Value-based agreements and other value-based policies can help bridge the gaps in real-world value-based care. There are many examples of value-based care models in rheumatology, orthopedics, and other arthritis-related specialties to draw from — and there are specific ways state and federal policymakers can promote value-based policies. While aspects of VBID may fall outside the scope of the MD PDAB, for context we want to highlight two areas in which we believe states can have an impact in promoting value-based insurance design:

- States should incentivize Medicaid programs and state payers to use patient-centered value assessment and consider value-based agreements.
- State reinsurance programs can be a good tool for ensuring better access in a way that reduces health care costs.

Additionally, in 2021 the Arthritis Foundation convened a coalition of patient and consumer groups called Value in the States to coordinate opportunities to elevate patient engagement in value assessment-related processes at the state level. While the coalition does not take a position on specific legislation or regulations, 25 of the organizations signed onto a set of principles for policymakers and other health care stakeholders to use as a guide in designing

patient-centered policies (see attached). These groups believe that the patient community is underrepresented in the value assessment paradigm, and as such laid out four core principles, some of which directly mirror our value assessment position statement:

1. All stakeholders should engage in meaningful patient engagement to inform their processes.
2. Patient experience and preference metrics must be utilized and prioritized in cost-effectiveness methodologies.
3. Value assessments should incorporate real-world data.
4. All stakeholders should be transparent about their processes, methods, and utilization of value assessments.

We appreciate the opportunity to provide comments and would welcome the opportunity to work more closely with the MD PDAB to bring the patient perspective to future proceedings. Should you have any questions or if we can be of assistance, please contact me at ahyde@arthritis.org or 202-843-0105.

Sincerely,

Anna Hyde

Anna Hyde
Vice President of Advocacy and Access
Arthritis Foundation