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November 13, 2020

Nakela L. Cook, MD, MPH
Executive Director
Patient-Centered Outcomes Research Institute
1828 L Street, NW, Suite 900
Washington, DC 20036

Re: Consideration of the Full Range of Outcomes Data: PCORI's Principles for Public Comment

Dear Dr. Cook,

The Alliance for Aging Research appreciates the opportunity to provide input on Patient-Centered Outcomes Research Institute's (PCORI) proposed principles on applying the full range of outcomes data in funded research.

The [Alliance for Aging Research](http://www.agingresearch.org) (Alliance) is the leading nonprofit organization dedicated to accelerating the pace of scientific discoveries and their application to improve the universal human experience of aging and health. The Alliance believes that advances in research help people live longer, happier, more productive lives, and that innovation will reduce healthcare costs over the long term.

We believe PCORI's expanded scope for the next decade will ensure a continued research focus on outcomes important to patients, family caregivers, and the broader set of healthcare stakeholders.

Principle #1: PCORI-funded research may consider the full range of outcomes important to patients and caregivers, including burdens and economic impacts.

We support PCORI's efforts to expand consideration of the broad scope of issues that impact patient health. Patient well-being and health should serve as the focus of every healthcare stakeholder's mission and valued outcomes. Historically, assessments have been overly narrow and fail to account for many factors that impact patients and their caregivers.

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The proposed principles are a good starting point in evaluating outcomes. Time in a hospital, time home from work or other activities, cost/time for transport, childcare costs while seeking care, and out-of-pocket expenses are important metrics for patient outcomes. Importantly, PCORI also proposes to incorporate the burden placed on family caregivers, as well as changes or substitutions in utilization or cost. The Alliance also encourages PCORI to use the following criteria in all relevant analyses:

- Cost of avoided or deferred care
- Additional expenses incurred as a direct or indirect result of a care plan or resulting side effects, such as caregiving, assistance with instrumental activities of daily living, and opportunity cost associated with the care plan, communicating with providers or payers, or ease of access to information that permits informed decision-making

The Alliance also encourages PCORI to support research projects that evaluate systemic issues that increase patient burden or difficulty in accessing appropriate care pathways. For example, we recommend that PCORI focus on identifying systems that create economic costs for patients, especially for the aging and disabled populations who typically face additional barriers to care. We also encourage PCORI to invest in research that examines the cost impacts of social determinants of health, such as racism, public safety, and inequities in care access. Historical injustices and structural barriers often create different and/or disproportionately large burdens to minority and economically disadvantaged communities, and traditional cost models do not account for these differences.

We also believe PCORI has the mandate to increase understanding of the interactions between patient outcomes and cost in healthcare decision making. Significant growth in patient cost liability has occurred over the last two decades. Patients are increasingly liable for out-of-pocket costs as a result of changes in employer-sponsored health insurance and plan benefit structures. Patient costs impact behavior and, as a result, health outcomes. As such, PCORI should study the impacts of various insurance and cost-sharing arrangements to evaluate the impact of patient cost exposure.

We also ask PCORI to continue advancing the availability of real-world evidence (RWE), such as patient registries, to ensure continued reporting of outcomes after clinical trials conclude. While the Alliance continues to work with stakeholders to increase the representation of older adults in clinical trials, most of the evidence on efficacy in the aging population is presently found in post-market data, which is usually not published. Understanding effectiveness for tested interventions among various age demographics is essential, as our immune systems, metabolism, and benefit-risk attitudes change with age.

Finally, the Alliance believes PCORI can provide value through investment in comparative clinical effectiveness research of outcomes within a given treatment modality using RWE. We encourage

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PCORI to collaborate with the Food and Drug Administration (FDA) to advance this important research category.

Principle #2: PCORI-funded research may consider the full range of outcomes relevant to other stakeholders when these outcomes have a near-term or longer-term impact on patients.

The Alliance supports the expanded ability of PCORI to consider outcomes related to other stakeholders involved in and impacted by patient care. At the same time, patient and caregiver outcomes should be awarded precedence in terms of importance, as they are the end-users of the healthcare system.

PCORI outlines several categories for outcomes for healthcare stakeholders, including costs related to a treatment or intervention, utilization, changes in utilization, the program costs of establishing/creating the intervention, as well as employer burden in the form of reduced productivity or absenteeism. These factors should be assessed in the context of weighted interdependencies. For example, in the case of growing ownership of physician practices by hospital systems and insurance plans, the priorities of the entity maintaining ownership will ultimately take precedence in an ownership arrangement.

Similarly, studies should examine how a treatment or service paid for by one stakeholder has benefits that accrue to other stakeholders and how the distribution of these benefits impacts the likelihood of investment. For example, Medicare would provide coverage for effective therapies that prevent or delay Alzheimer's disease. However, Medicaid is the primary payer for long-term services and supports, including custodial care in nursing homes and home care programs, and would experience significant savings from disease-modifying therapies. Traditional cost analyses only count as benefit when investment in one program saves money in the same program.

We also encourage PCORI to conduct research that provides insight into the effectiveness and impact of underlying functional and structural factors. Examples of these types of analyses include the influence of financial incentives on outcomes. We also encourage PCORI to examine the impact of income, access, and socioeconomic determinants to illuminate differences that may be addressable through targeted interventions. Research should include review common factors considered in healthcare disparities, as well as factors including wage depression due to healthcare costs, infrastructure spending reductions, and education and public health spending reductions.

The Alliance requests that PCORI research the alignment of real-world experience with our understanding of the relative value of outcomes. Over the last decade, there have been many efforts to redesign care to increase value and quality. However, these efforts have experienced mixed

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and/or limited results in changing the trajectory of healthcare costs, addressing the impacts of consolidation, or improving quality. The incentives in these programs have been structured around assumptions of important outcomes to stakeholders. However, expected outcomes have not materialized, raising the need to examine model inputs. To fulfill the principle of considering the full range of outcomes relevant to stakeholders, PCORI should assess the relative importance of meaningful outcomes to stakeholders. This investigation should extend beyond survey work and include research into the economic and non-economic drivers of systemic change, as well as the sensitivity of behavioral response. Findings from this research can serve as foundational elements to inform research and care redesign.

Similarly, research and behavioral analysis of outcomes could examine administrative and economic obstacles to improvements in patient-centered outcomes. Examples of such activities include monopolistic behavior or vertical integration leading to higher prices or limited access, lack of comprehensive data from Medicare Advantage plans and patient registries, and barriers to information transfer leading to failures of cooperation and clinical integration.

The Alliance also encourages PCORI to consider economic measurements outside of traditional cost-effectiveness, such as contribution to the public good. Measuring and placing value on the public good merits effort, as the absence of value assessed to these considerations may lead to decisions that do not align with the best interests of society. For example, in the context of health systems, there is continual downward pricing pressure that encourages efficiency and streamlined operations. However, this approach fails to account for societal benefits such as capacity/availability of clinicians and hospital beds during an emergency or pandemic, or the training of new medical professionals. Understanding the value of the public good can help ensure that the healthcare system evolves to meet the needs of patients and society, rather than focus on commoditizing healthcare treatments and services.

Principle 3: The collection of data on burdens and economic impacts of treatment options must be appropriate and relevant to the clinical aims of the study.

The Alliance for Aging Research supports PCORI's expanded mandate to include a broader range of outcomes relevant to patients and their caregivers, specifically economic impacts and a full range of burdens. This type of research informs patient decision-making and provides a greater level of understanding of the non-clinical impact a treatment may have on their life. This expansion is in keeping with PCORI's mission of helping people making better healthcare decisions, improving healthcare delivery, and advancing healthcare outcomes through the development of high-integrity and evidenced-based information. Furthermore, we appreciate that the Principles document states in no uncertain terms that PCORI will not fund studies for which cost and economic impacts are the

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primary outcomes. This clarification is necessary to keep the focus of PCORI-funded studies on information that is best for patients. We ask that PCORI carefully evaluate the communication of cost information to ensure support for treatment options most appropriate for a clinical indication and preferred patient-centered outcomes.

The Alliance is supportive of PCORI requiring applicants to engage stakeholders – particularly patients – as they develop research questions, study design, and outcome measures. We believe firmly in the patient-centered mission of PCORI, and that patients and their caregivers know the outcomes and impacts that are most meaningful to capture. To support this end, we recommend further guidance for applicant engagement with relevant stakeholders in the development of study designs. Guidelines should include the expected number of stakeholder groups consulted in study design as well as appropriate ways to aggregate patient preferences.

We also encourage PCORI to include a more comprehensive list of examples of burden data that applicants should include in their applications. This list can serve as a guidepost for applicants who are considering the types of data most relevant to patients and other stakeholders. We recommend that this list incorporate the additional factors included in this comment letter.

Burdens on older adults can be different from other aging cohorts. To ensure that data is relevant to the older adult patient and other stakeholders, the Alliance encourages age-specific burden data in PCORI-funded studies. The aging population has unique healthcare needs including an increased likelihood of pre-existing conditions, a lack of healthcare professionals (i.e. geriatricians and qualified nursing professionals) specifically trained to care for this population, and less access to healthcare services in rural communities. We ask that as PCORI evaluates applications for studies on older adults that it considers the unique needs of this population.

Furthermore, older adults are not a homogenous group. We request that burden data for older adults be separated into subgroups, when possible. Examples of these groupings include data categories for care setting (community, nursing home, etc.), age range (i.e., ages 65 to 75, and 75 and older), cognitive status, and community setting.

Principle 4: Beyond the collection of burden and economic impact data, PCORI may support the conduct of certain types of economic analyses as part of a funded research study to enhance the relevance and value of this information to health care decision-makers.

The proposed principles reiterate that PCORI will not fund research whose primary purpose is cost-effectiveness assessment and clarifies that research should not be designed to inform resource allocation. To this point, we are very supportive of PCORI's reaffirmation that funded studies cannot

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use cost-per-quality-adjusted life year metrics in its analysis. Metrics such as the Quality-Adjusted Life Year (QALY) are antithetical to the mission of PCORI, as the underlying methodology assigns lower value to patient subgroups. When QALY is utilized in healthcare coverage determinations, older adults, disabled persons, and patients with chronic conditions can be deemed as "too expensive" to receive care in comparison to a person in "perfect health."

The Alliance would like to see PCORI take a leadership role in identifying non-QALY economic approaches that emphasize patient-centered outcomes and preferences. While PCORI has funded external groups to explore such approaches, we believe the organization should make this effort a core internal initiative and convene external groups to help advise measure development.

Conclusion

Thank you for the opportunity to provide feedback on PCORI's principles for incorporating the full range of outcomes data in supported research. These principles establish a roadmap for the next decade of PCORI's leadership in clinical effectiveness research. We encourage PCORI to capture the wider set of costs and impacts associated with care pathways to provide a full assessment of outcomes for patients, their caregivers, and other stakeholders. We also encourage PCORI to support wide-ranging research examining the drivers of systemic change. This research can serve as a foundation to advance the essential work of ensuring that our healthcare system evolves in ways that are both patient-centered and consumer-friendly.

If you have any questions, please do not hesitate to contact us. Inquiries can be directed to the Alliance for Aging Research's Director of Public Policy, Michael Ward, at mward@agingresearch.org.

Sincerely,



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