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Joseph V. Selby, MD, MPH  
Executive Director  
Patient-Centered Outcomes Research Institute  
Public Comments  
1701 Pennsylvania Avenue, NW  
Suite 300  
Washington, DC 20006

Dear Dr. Selby:

On behalf of the physician and medical student members of the American Medical Association (AMA), I appreciate the opportunity to provide comments on the Patient-Centered Outcomes Research Institute's (PCORI) Draft Methodology Report: "Our Questions, Our Decisions: Standards for Patient-centered Outcomes Research" (Methodology Report). PCORI's active engagement with physicians and other stakeholders throughout this process is welcome and will significantly advance the goal of promoting the rapid acquisition and adoption of relevant evidence-based medicine that will improve patient outcomes. Overall, we believe the Methodology Report supports efforts to strengthen the rigor and quality of the evidence generated by PCORI-funded activities. However, we have three major areas of concern and urge reconsideration of these key approaches taken in the Methodology Report.

First, as we have shared before with PCORI staff and the PCORI Board of Governors, registries serve an important role in identifying patient-centered outcomes research priorities and will amplify the quality of the comparative research around treatment techniques under real world conditions. Furthermore, registries will drive the uptake and dissemination of this evidence rapidly. Related to the foregoing, it will expand the number of investigators engaged in this research beyond the traditional researchers engaged in randomized clinical trials (RCTs). In addition, as highlighted in the report, "when properly designed, [registries] are able to provide data on groups of patients sometimes not included in clinical trails, and they can be responsive to rapid changes in medical practice."

While the intention may not have been to place an emphasis on more traditional (and difficult to scale or sustain) methods for conducting research in the Methodology Report, we urge PCORI to include in the report a more explicit and detailed discussion on registries and the essential role they play in the portfolio of methods and evidence generation. Specifically, the authors of the report could engage the Agency for Healthcare Research and Quality (AHRQ) and physician medical specialty societies to better understand and reflect the capability of *existing* registries for immediate comparative effectiveness research (CER). There is an unprecedented opportunity to re-think traditional approaches to research that build on the early investments in registries and other clinical data networks. PCORI should better highlight the importance of these two powerful infrastructure

mechanisms, clinical registries and clinical data networks, and dedicate additional consideration to these mechanisms.

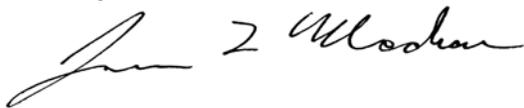
Clinical data registries allow health care stakeholders to observe patterns of care and the effectiveness of various interventions over time. Randomized controlled clinical trial studies, although ideal, are not always feasible to obtain, especially for surgical procedures and rare diseases. We also share the concerns expressed by others that the Methodology Report does not adequately address the particular research needs in the area of personalized medicine given the current focus on traditional research methods. The traditional research methods have not and will not adequately capture relevant personalized medicine findings—some of which promise to significantly transform medicine as we know it over the next several years and beyond. For example, reviewing molecular diagnostic tests will require a new paradigm for collecting and analyzing evidence such as registries.

Second, the Methodology Report does not include support or endorsement of social behavioral research (versus the biomedical research). We are very concerned with this omission given the patient-centered mandate of PCORI. If social behavioral research methods are not accepted, there will be limited interest and data for patient-centered data or programming. The AMA does not believe that it is the intent of PCORI to take such a narrow view, but the tone throughout the Methodology Report was consistently present. Ultimately, the draft Methodology Report lacks the clarity needed to address evidence-based practice.

Finally, we would like to underscore our comments above related to personalized medicine. This area of investigation and emerging clinical practice has already begun to challenge well-established medical practice in areas such as oncology and infectious disease. However, we have only begun the journey to understand the medical and clinical implications of the human genome—each week there seems to be another significant discovery. We urge PCORI to establish the Personalized Medicine Expert Advisory Panel to assist with the development of additional guidance in the Methodology Report vis-à-vis personalized medicine.

The AMA appreciates this opportunity to provide input to the PCORI and welcomes the opportunity to continue working together to increase the quality and rigor of information available to physicians at the point of care in order to drive improved patient outcomes. If you have any questions or need additional information, please do not hesitate to contact Jennifer Meeks, Division of Federal Affairs, at 202-789-4688 or by email at [jennifer.meeks@ama-assn.org](mailto:jennifer.meeks@ama-assn.org).

Sincerely,

A handwritten signature in black ink, appearing to read "Jim L Madara". The signature is fluid and cursive, with a large initial "J" and "M".

James L. Madara, MD