

September 1, 2011

Joseph V. Selby, M.D., M.P.H.
Executive Director
Patient-Centered Outcomes Research Institute
5185 MacArthur Blvd. NW Suite 632
Washington, DC 20016

Re: Recommendations concerning patient-centered outcomes research national priorities,
patient centered outcomes research definition, and proposed Tier 1 grant projects

Dear Dr. Selby:

The undersigned organizations representing physicians and medical students commend the Patient-Centered Outcomes Research Institute (PCORI) Board of Governors (Board) for embracing a transparent process that allows stakeholders to play a key role in PCORI's activities. This is a unique opportunity to build the infrastructure and mechanisms that draw patients and a significantly broader number of practicing clinicians from varied practice settings, specialties, and geographically diverse locations into the important work of the PCORI. The charge of PCORI, and the comparative effectiveness research (CER) enterprise more broadly, is to ensure that patient-centered outcomes research (PCOR) findings are available to physicians and patients (and their caregivers) at the point of care in order to support decision-making, not dictate care. The transparent process adopted by the Board will facilitate our efforts to engage our members in the PCOR enterprise and their participation in all aspects including the critical uptake and application phase.

In addition to our overarching recommendations concerning 2011-2012 national priorities, as discussed below, we are submitting general comments concerning the proposed PCOR definition, as well as feedback on the proposed initial topics for PCORI's Tier 1 grant projects. We applaud the Board's efforts to solicit input on these items. As discussed more fully below concerning the Tier 1 grant projects, we strongly support the creation of an established PCORI mechanism that seeks out and obtains regular input from individual physicians, state medical associations, and national medical specialty societies concerning national priorities as well as comments concerning other components of the PCOR enterprise. We recommend that the staff of PCORI develop a series of methods to obtain additional information on a regular basis from physician organizations.

Definition

We support a broad definition of PCOR that involves a comparison of different modalities, including health delivery models, to manage a specific health problem, condition, or disease. Besides the more typical areas of research, such as pharmaceuticals, medical devices and diagnostics, PCOR should also focus on implementation and dissemination issues that would shed light on the most effective strategies that promote a learning health care system and improved clinical outcomes.

The proposed PCOR definition offered for comment is broadly consistent with the definition contained in the statute establishing PCORI. We generally support the definition, with two caveats. The definition proposed by the Board is laid out as follows:

Patient-Centered Outcomes Research (PCOR) helps people make informed health care decisions and allows their voice to be heard in assessing the value of health care options. This research answers patient-focused questions:

1. “Given my personal characteristics, conditions and preferences, what should I expect will happen to me?”
2. “What are my options and what are the benefits and harms of those options?”
3. “What can I do to improve the outcomes that are most important to me?”
4. “How can the health care system improve my chances of achieving the outcomes I prefer?”

To answer these questions, PCOR:

- Assesses the benefits and harms of preventive, diagnostic, therapeutic, or health delivery system interventions to inform decision making, highlighting comparisons and outcomes that matter to people;
- Is inclusive of an individual's preferences, autonomy and needs, focusing on outcomes that people notice and care about such as survival, function, symptoms, and health-related quality of life;
- Incorporates a wide variety of settings and diversity of participants to address individual differences and barriers to implementation and dissemination; and
- Investigates (or may investigate) optimizing outcomes while addressing burden to individuals, resources, and other stakeholder perspectives.

We understand that the Board intends to utilize the PCOR definition for purposes of highlighting the mandate of PCORI to focus on patient outcomes. We appreciate that the definition covers a

broad range of categories from preventive, diagnostic, therapeutic, or health delivery system interventions. We believe the foregoing is fully consistent with the law and all are important to patient outcomes. In light of the foregoing, we believe that the definition should reference patients and their caregivers (as opposed to the more generic term “people”) as well as physicians. Furthermore, we do have questions concerning the last component of the definition that provides that PCOR: “[i]nvestigates (or may investigate) optimizing outcomes while addressing burden to individuals, resources, and other stakeholder perspectives.” We seek further clarification toward the Board’s intentions regarding this last component and whether this includes cost analysis. If that is the case, we do not believe that it is consistent with the PCORI’s enabling statute and the language should be excised.

Proposed Initial Topics for PCORI’s Tier 1 Pilot Projects

We are pleased that the Board has concluded that it would be both prudent and strategic to seek additional information and feedback on what will work as it develops the PCORI infrastructure. The pilot projects represent a smart investment to identify the most promising way forward in developing the PCORI infrastructure, national priorities, and research methods. A number of the proposed eight initial topic areas for research limit the categories to novel methods, approaches, instruments, and processes. We would urge PCORI to consider that these grants be expanded to include research on existing methods, approaches, instruments and processes. While the PCORI could elect to give priority to novel approaches, valuable information could be gathered on existing efforts that have not been fully studied or documented.

General Priorities and Parameters

As you know, physicians today have access to a wide array of medical information. However, there remains far too little rigorous evidence readily available to physicians and patients when they need it most about which treatments work best for which patients. Although PCOR would benefit from a broad range of areas and topics, we urge the PCORI to strategically target support for PCOR where it will significantly improve health care value by enhancing physician clinical judgment, fostering the delivery of patient-centered care, and producing substantial benefit to the health care system as a whole.

All aspects of the PCOR process, including priority setting, must be transparent and include a set of mechanisms to support physician engagement and participation. This support applies to physicians in all practice settings, including small and medium-size practices as well as practices in rural and otherwise underserved areas. Given the nascent nature of this endeavor, the perception among physicians that the CER/PCOR agenda is being driven by payers who only have cost-containment as their goal, would seriously undermine efforts to advance physician support as we continue forward with PCOR. It is imperative that physicians, including practicing clinicians and their organizations, have an active, ongoing, and central role at all stages of the PCOR process. Physicians trust what they have had a role in developing and creating. While there is physician representation on the PCORI Board, we urge you to consider the opportunity to expand the PCOR enterprise to physician practices outside of academic medical centers and into small-and medium-size physician practices, e.g., actively engage and communicate with medical state and specialty organizations, etc. We also urge the PCORI to

allocate sufficient resources, including staff, to promote physician participation and engagement in this PCOR enterprise. This will represent a strategic investment because it will provide a rapid feedback loop and an opportunity to address concerns and adjust course as necessary.

We support the view that the initial priority areas of PCOR should focus on high volume, high cost delivery models, modalities, and other health services which evidence significant variation in practice.

In terms of methodology and study design, PCOR should include long-term and short-term assessments. PCOR should not be limited to new treatments. In addition, the findings should be re-evaluated periodically, as needed, based on the development of new alternatives and the emergence of new safety or efficacy data. We recognize that the latter will pose a significant challenge, but strongly believe that clinical registries offer the best means of realizing a real-time health care learning environment.

While the Board should establish a diverse portfolio of priorities, the national PCOR priorities should, at a minimum, address the prevention, management, and treatment of preventable disease which collectively represent a major cost-driver in today's health care system. Areas in need of further study and research include cardiovascular, endocrinology and metabolic disorders (including diabetes), and nutrition (including obesity). For example, in the area of wellness, prevention, nutrition, and obesity there is a paucity of PCOR findings. It is an area with a wide range of available interventions with little clarity about which is most effective.

CER usually considers technology and pharmaceuticals, but behavioral interventions potentially could have the greatest impact for individual patients and system-wide. Prioritizing interventions designed to change physician behavior and to effect behavioral change in patients is necessary, as are other clinical interventions, technologies, and pharmaceutical remedies. Because prevalence rates and the most effective interventions for many diseases vary greatly by race, ethnicity, gender, age, geography, and economic status, we support the inclusion of racial and ethnic health disparities—and health disparities more generally—as a PCOR priority area.

Focusing PCOR on areas that have the most clear effectiveness data is an attractive and logical decision since the more data that is available, the easier it is to generate the research. However, this approach could underemphasize critically important areas where the body of research is thinner or newer, as is the case with pulmonary, critical care, and sleep medicine. It is important that PCORI consider this issue in assigning priorities.

Existing Sources for PCOR Priorities

In 2010, the Patient Protection and Affordable Care Act (ACA) charged the Department of Health and Human Services (HHS) with developing a National Quality Strategy (NQS) to improve the delivery of health care services, patient health outcomes, and population health. The new law requires HHS to develop the strategy with input from a range of stakeholders to ensure a strategy that is most likely to positively impact public health. In October 2010, under contract with the Centers for Medicare & Medicaid Services (CMS), the National Quality Forum (NQF)

convened the multistakeholder National Priorities Partnership (NPP) to provide collective input to HHS for consideration as it develops this national body of work.

In March 2011, HHS released the first NQS, which built on the earlier work of NPP, including its goals of reducing harm, improving patient-centered care, eliminating health care disparities, and removing waste from the system. In this first strategy, HHS specifically offered six national priorities, which closely corresponded to the NPP goals. HHS will build upon this strategy each year, pulling from NPP's recommendations to promote a strategy that fully addresses the three overarching domains of the NQS—better care, affordable care, and healthy people/healthy communities. Utilizing the NQS and the NPP National Priorities and Goals as a reference point will help the Board identify national PCOR priorities that will build the evidence-base in a targeted fashion in the areas that are likely to produce substantial system-wide improvements.

Clinical Registries and Clinical Data Networks

We urge the PCORI to prioritize support for two powerful infrastructure mechanisms, registries and clinical data networks, that not only produce research findings, but play a key role in priority-setting as well as uptake and adoption of findings in a rapid cycle. Clinical data registries allow health care stakeholders to more clearly observe patterns of care and the effectiveness of various interventions over time. Methodologically speaking, the PCORI should recognize that data from randomized controlled studies, although ideal, are not always feasible to obtain, especially for surgical procedures and rare diseases. Effective PCOR will require opportunities to incorporate data from alternative sources, such as clinical registries. Registries allow health care professionals to identify clinical research priorities in real-time, to generate and test hypotheses, and to develop clinical guidelines in a very rapid cycle. The development and use of registries also align with the Institute of Medicine's current effort to promote a "learning healthcare system"—a system that delivers the best care every time, and to learn and improve with each care experience.

Clinical registries have been used by specialty societies, such as The Society of Thoracic Surgeons, American College of Cardiology, and the American College of Radiology, and have markedly improved quality and patient safety. The National Surgical Quality Improvement Program (NSQIP) and the Northern New England Cardiovascular Collaborative are two examples of organizations utilizing these two mechanisms to advance quality and obtain research data at the point of care, and to create what our country needs, a learning network. Expansion of existing clinical registries and databases would provide a strong foundation when conducting PCOR and at the same time these registries would also provide an excellent beginning point for PCOR. Utilizing, replicating, expanding, or integrating existing clinical registries would constitute an invaluable investment in the much needed infrastructure for accurately comparing clinical outcomes based on "real life" conditions where care delivery settings vary, patients may have numerous co-morbidities, and the patient populations are diverse. In turn, the clinical registries are not identical and may, to a greater or lesser extent, be able to promote a learning health care environment. Thus, evaluating the relative clinical effectiveness of various clinical registry models and alternatives to them remains a vital priority. Building the PCOR infrastructure and capacity, in part, upon registries and clinical data networks will leverage PCOR resources and boost the capacity of the system as a whole to learn and adapt in real-time.

As part of its efforts to launch a clinical outcomes data registry to foster PCOR, organized neurosurgery has seen significant regional variability in Institutional Review Board (IRB) requirements. In some instances, contribution of data to a national registry has been considered a quality reporting initiative and therefore IRB-exempt. At the other extreme, a significant number of IRBs have treated registry participation like a research effort, requiring the added burden of individual patient consents and standard research protections. While this presents an obstacle in terms of current registry projects, it is also a problem that individual physicians and hospitals will face with increasing frequency as they are held accountable for numerous private sector and ACA-authorized quality reporting requirements, many of which will necessitate the inclusion of patient-reported outcomes. Without some sort of governmental clarification indicating that registry reporting and data collection related to PCOR are considered IRB-exempt quality reporting activities and do not require consent, this added regulatory burden will severely inhibit our ability to generate PCOR data on the scale that will be required to support clinical decision-making at the point of care.

Finally, PCOR should be designed, communicated, and used in ways that recognize variation in individual patients' needs, circumstances, preferences, and responses to particular therapies, rather than encouraging one-size-fits-all solutions based on population averages. Similarly, PCOR should support personalized medicine and the ability of physicians to tailor treatments to the needs of individual patients based on genetic information and other factors.

PCOR has the potential to have a profoundly positive impact on the quality of the information available to physicians and patients and, when used appropriately and with care, may help address escalating health care costs. We look forward to working closely with the PCORI to ensure that physicians remain engaged, enthusiastic, and involved stakeholders in this complex and very important process.

Sincerely,

American Academy of Dermatology Association
American Academy of Family Physicians
American Academy of Ophthalmology
American Academy of Otolaryngology - Head and Neck Surgery
American Association of Neurological Surgeons
American College of Cardiology
American College of Emergency Physicians
American College of Osteopathic Family Physicians
American College of Osteopathic Surgeons
American College of Radiology
American College of Surgeons
American Congress of Obstetricians and Gynecologists
American Medical Association
American Osteopathic Association
American Osteopathic Academy of Orthopedics

American Society for Reproductive Medicine
American Society of Anesthesiologists
American Society of Nuclear Cardiology
American Urological Association
College of American Pathologist
Congress of Neurological Surgeons
Joint Council of Allergy, Asthma and Immunology
North American Spine Society
Society of Gynecologic Oncology
Society of Interventional Radiology
Society of Nuclear Medicine
The Society of Thoracic Surgeons

CC: PCORI Board of Governors