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March 14, 2012

Patient-Centered Outcomes Research Institute
Public Comments
1701 Pennsylvania Ave. NW, Suite 300
Washington, DC 20006

Dear Madam or Sir:

The Personalized Medicine Coalition (PMC) appreciates the opportunity to comment on the national research priorities recently published by the Patient Centered Outcomes Research Institute (PCORI).

PMC is an education and advocacy organization dedicated to advancing personalized medicine to benefit patients and the health care system and has strongly advocated for aligning comparative effectiveness research and personalized medicine. PMC supports PCORI because the legislation creating the Institute included a mandate to align personalized medicine and comparative effectiveness research (CER), and required procedures to assure that alignment.

To paraphrase the President's Council of Advisors for Science and Technology, personalized medicine refers to the tailoring of medical treatments to the individual characteristics of each patient and enables preventive or therapeutic interventions to be concentrated on those who will benefit, sparing expense and side effects for those who will not.

Personalized medicine ensures that the health care system delivers the right treatment to the right patient at the right time. CER that considers differences in patient responses, for example, based on genetic characteristics, clinical biomarkers and other differentiating features, will deliver evidence that can be used to improve the health care system and the quality of care for individual patients. The development of an infrastructure that is able to support the subgroup analysis of diverse sources and types of patient data is a prerequisite for effective CER. Defining PCORI's research priorities and agenda through a clear, open process is one critical, early step to implementing CER that is patient-centered and aligned with personalized medicine.

PCORI was designed to address specific, practical questions of national importance. However, the broad and vague drafting of the research priorities is more appropriate for traditional, investigator-driven research, which may or may not address the types of questions PCORI must answer. When specific studies are identified for possible funding, we will be better able to evaluate if PCORI is focused on research that answers the practical questions, as intended by legislation. Broad drafting does not allow for an examination of individual research proposals, topics, or research questions, thus, it is not possible to say whether PCORI's work will support personalized medicine or not.

PMC makes the following requests:

1. Release draft research priorities that are specific to individual interventions and research questions (e.g., specific treatments, health care delivery interventions or designs, infrastructure-building projects);
2. Develop the infrastructure necessary to carry out PCORI's mission and engage the public as that infrastructure is built.

While the focus of this letter is on the research priorities as currently drafted, we will take this opportunity to outline some suggestions related to the development of PCORI's infrastructure.

Priorities for Research

Defining clear, specific research priorities will provide the personalized medicine community assurance that PCORI is implementing a research agenda that is both patient-centered and aligned with personalized medicine. Below we examine some of the priorities for research and the degree to which they will support personalized medicine and, by extension, patient-centered health care.

Research conducted in the category "**Comparative Assessment of Options for Prevention, Diagnosis, and Treatment**" will compare treatments, but at what level? Researchers now know more about individual response to some drugs based on biomarker information. When comparing, for example, a red pill to a blue pill, it is imperative that biomarker information be included in those examinations, especially when biomarker information is in the label for the drug. But, without clearly defining the details of PCORI's research, we cannot know whether or how the Institute will consider biomarker information in its priorities and research agenda. (A list of drugs with biomarker information currently in the label can be found on pages 20-25 of *The Case for Personalized Medicine, 3rd edition* available for free download at <http://personalizedmedicinecoalition.org/>.)

When studies evaluate how the **healthcare delivery system** can support patient-centered care through innovations, PMC suggests that the research examine the extent to which the system is designed to support predictive, preventative, personalized and participatory health care. By emphasizing these "4Ps," health system evaluation can

support personalized medicine. However, without more specific research priorities, we do not know whether PCORI will include research that addresses these aspects of care delivery. PCORI, and the underlying statute, appropriately recognize the central role of delivery system improvement in driving high-quality, personalized health care. In addition to learning what works for whom, research must answer the questions of how the health care system can ensure each patient receives optimal care and achieves the outcomes that matter to them.

To support personalized medicine, the “**Communication and Dissemination Research**” priority should focus on explaining who is most likely to benefit from an intervention and ideally, why. It is not enough, in the PMC’s opinion, to say that one therapy works for most people in the aggregate. To enable personalized medicine, research must explain why a therapy works and for what types of patients. Or, it must provide access to the data and tools that patients and physicians can use to develop an informed, individualized treatment plan. For example, in research comparing red and blue pills, the communication and dissemination of research results should emphasize that although the red pill works for most people, the blue pill is particularly effective for people with a specific biomarker or other particular characteristics.

Infrastructure

The statute creating PCORI contained some important directives that must be incorporated into PCORI’s structure to comply with Congressional intent and, by extension, support the science of personalized medicine.

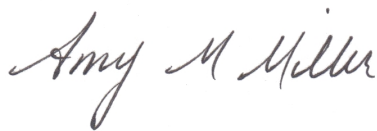
1. Define a public engagement process: Outline an open, transparent process for obtaining input from all stakeholders including patients, clinical experts, and scientists and detail how stakeholder input will be used. One possibility is to outline all comments in a draft of proposed documents and demonstrate how each comment informed the final draft.
2. Personalized medicine expert advisory panel: PCORI has the statutory authority to create expert advisory panels, on any topic, to carry out its mission. To assist PCORI with assuring that their work supports personalized medicine, PMC strongly requests that the Institute develop an expert advisory panel devoted to personalized medicine. As an education organization dedicated to advancing the field, and populated by stakeholders from all sectors of the health care universe, we offer our clinical science committee’s assistance in identifying potential members for this proposed expert advisory panel.
3. Improve CER science: One of PCORI’s Congressionally-mandated tasks is to improve the quality of CER by incorporating new information and technological innovations into its studies, by reviewing and updating the evidence as necessary, and by outlining what future research will be needed to address perceived information gaps. PMC suggests building the infrastructure and the processes to achieve this goal now, as the foundations of the organization are being established.

4. Provide specific research priorities: Importantly, the PCORI research priorities envisioned by statute were both broad – encompassing all aspects of the health care system that relate to high-quality, effective patient care – and specific, calling for a transparent process to identify and prioritize research topics based on explicit criteria and public input. PCORI’s materials appear to miss the second point, assuming that because the statute envisions a broad scope of research, Congress was not calling for specific research priorities.
5. In-house capacity to engage broad scientific and clinical expertise: The mission of PCORI is unique and to carry it out, PCORI must have a unique set of individuals to develop calls for research proposals, evaluate them, make awards, follow the progress of the research, and engage the public at all steps along the way. Having this infrastructure “in-house” is a necessary step in the Institute’s development.

Conclusion

PMC realizes that as PCORI moves from national priorities to a focused research agenda, and then to individual requests for applications, messages will get more specific and details will be clarified. We look forward to reviewing those research priorities and research agenda items and hope that they give us confidence that patient-centered CER will, in fact, enable personalized medicine’s growth as Congress intended. We also look forward to reviewing plans regarding the development of PCORI’s infrastructure. If you have further questions, please contact me by phone, 202-589-1770, or email, amiller@personalizedmedicinecoalition.org.

Sincerely,

A handwritten signature in cursive script that reads "Amy M. Miller".

Amy M. Miller, Ph.D.
Vice President, Public Policy