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Letter from Washington

When the USA was debating healthcare reform and negotiating how to provide the best quality care for the lowest cost to all Americans, some looked to the UK's NICE, and thought that developing a similar organization might be a good idea. Americans were hesitant, however, to copy the British model due to fears that comparative effectiveness research (CER) would lock the US healthcare system into a 'one-size-fits-all' paradigm and restrict patient access to new treatments that may benefit many patients. By design, the Patient Centered Outcomes Research Institute (PCORI) put in place by America's Affordable Care Act, included language intended to align CER with personalized medicine.

Still a young organization, PCORI has already chosen an Executive Director, seated both its Board of Governors and its Methodology Committee, hired a few key staff, and asked the public for feedback on its definition of Patient Centered Outcomes Research, Draft Translation Framework Components, and Draft National Priorities for Research and Research Agenda.

While PCORI has laid a foundation, thus far, its broad and vague research priorities leave the personalized medicine community uncertain about whether that foundation will support the continued development of personalized medicine. Amy Miller, Personalized Medicine Coalition's (PMC's) Vice President for Public Policy, noted in a letter the PMC sent to the Institute: "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."

The challenges ahead for PCORI, however, do not stop with the drafting of specific research priorities. The Institute must also create an infrastructure that supports its mandate to align personalized medicine and CER. Congress required procedures to assure that alignment. But thus far the Institute has not fully developed the internal structure or expertise necessary to develop calls for research proposals, evaluate them, make awards, and do so through an open, transparent and focused process.

In the PMC's letter to the Institute, the coalition offered five recommendations about how PCORI could better define its research agenda and build the infrastructure needed to execute its mission as congress intended.

■ Define a public engagement process

PCORI should 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.

■ Create a 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 its work supports personalized medicine, the PMC strongly suggested that the Institute develop an expert advisory panel devoted to personalized medicine.

■ 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. The PMC recommended building the infrastructure necessary to achieve this goal now, as the foundations of the organization are being established.



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■ Provide specific research priorities

Importantly, the PCORI research priorities envisioned by statute were both broad – encompassing all aspects of the healthcare 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 proposals miss the second point – assuming that because the statute envisions a broad scope of research, Congress was not calling for specific research priorities.

■ Develop in-house capacity to engage broad scientific & clinical expertise

The mission of PCORI is unique. To carry it out, PCORI must foster a cadre 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.

Conclusion

To facilitate personalized medicine, research must explain not only what works best – but also for whom. Answering that critical question upon which the future of medicine depends cannot be done without careful construction and consideration.

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