

**PIPC Comments on the *Innovation for Healthier Americans* Report
February 23, 2015**

The Partnership to Improve Patient Care (PIPC) supports the goals of *Innovation for Healthier Americans* of accelerating discovery, development and delivery of innovative treatments. We applaud Chairman Lamar Alexander and Senator Richard Burr for issuing this report, and urge the Health Education Labor and Pensions (HELP) committee to work toward expanded innovation and access in a bipartisan manner.

Since its founding, the Partnership to Improve Patient Care (PIPC) has been at the forefront of patient-centeredness in comparative effectiveness research (CER) – both its generation at the Patient-Centered Outcomes Research Institute (PCORI), and its translation into patient care. Having driven the concept of patient-centeredness in the conduct of research, PIPC looks forward to bringing the patient voice to the discussion of how to advance patient-centered principles throughout an evolving health care system.

We would urge the HELP Committee to explicitly focus on incorporating patient perspectives into the regulatory process and to help address their unmet medical needs as part of building the foundation for 21st Century medicine. We also urge the Committee to more explicitly recognize as a goal of its work the need to ensure health care decisions are made based on the principles of shared decision-making. This means arming patients and providers with the evidence and tools necessary to make informed decisions based on the needs, preferences, and circumstances of the individual patient. It also means establishing policies that incentivize providers and empower patients to make informed treatment decisions within new payment models.

We agree with Dr. von Eschenbach’s statement that, “In order to accelerate and enhance the efficiency of our nation’s healthcare ecosystem – discovery, development, and delivery – all three of these core components must be well- coordinated, consistently evaluated, and effectively nurtured.” To be patient-centered, each stage of innovation should be evaluated to determine its ability to inform and empower patients and their providers by enabling the use of tools to deliver personalized care. Our comments will therefore focus on the need to develop policies that ensure patient engagement and capture patient preferences to support the discovery, development, and delivery of innovative new medicines.

Expanding the Patient Engagement Infrastructure Beyond FDA

PIPC acknowledges that the Food and Drug Administration (FDA) has made significant strides in its work to engage patients through its Patient-Focused Drug Development initiative. In describing systemic challenges at the FDA, the report stated, “Bringing the underperforming divisions up to the standards set by these more efficient divisions would greatly help patients with other diseases and conditions; however, determining the best manner in which to accomplish this a complex challenge that will require the FDA, Congress, sponsors, patients, and

researchers to work together.”¹ PIPC strongly agrees that collaboration, particularly with affected patients, will be critical in the development of new regulatory pathways. We cannot emphasize enough the value of the patient experience in the innovation process.

PIPC agrees with the report’s assertion that, “Identifying ways to encourage and facilitate enhanced collaboration between government and non-government entities to support important research will be critical to advancing novel therapies and ensuring America’s continued global leadership in medical innovation.”² We are also pleased to see that the report expresses the value of public-private partnerships, and referenced several examples. Yet, the report acknowledged, “It is not clear, however, who is accountable for ensuring that these partnerships are folded into systemic change.”³ Without accountability, partnership and collaboration have little meaning, and instead often represent token efforts to engage other stakeholders.

Meaningful patient engagement requires that the patient voice not just be heard, but that it actually informs decision-making. We urge that the HELP Committee consider how it can ensure that patient participation is meaningful, and does not represent what we would call “token” engagement where the patient voice is heard but later dismissed. By engaging patients at each step of the process—from driving basic research at NIH to shared decision-making at the point of care—we can design an infrastructure that 1) supports the generation of research that matters to patients, 2) spurs continued innovation of precision or personalized medicine, and 3) ensures delivery system and payment reforms are patient-centered and improve health outcomes. As an example, PCORI could provide the Committee with some insights gained as it has gone through a long process, in its first five years of implementation, to create policies that give the patient voice meaning in the development of patient-centered outcomes research.⁴

The report also references *delivery* of innovative treatments as a goal of its work. Therefore, we believe that an opportunity exists to utilize and improve upon the FDA’s Patient-Focused Drug Development initiative as a model for patient engagement throughout HHS and its agencies to ensure the development and implementation of patient-centered policies that affect the delivery of health care innovations. For example, there is a rare opportunity for HHS to work collaboratively with groups of patients and providers to identify outcomes that matter to patients, turn those outcomes into rigorously developed measures, apply those measures to research, and use those measures as the basis for assessing the impact of new delivery models on patients and patient care as they are being promoted by the Centers for Medicare and Medicaid (CMS). This work will not happen effectively with only a notice and comment period in the Federal Register. It requires a more proactive posture within government agencies to welcome the meaningful and timely input of patients—which is the goal of the FDA initiative—accompanied by the effective translation of the patient perspective into the delivery of public health programs.

¹ *id.* at 22

² *id.* at 5

³ *id.* at 26

⁴ Example at <http://www.pcori.org/assets/2014/02/PCORI-Patient-and-Family-Engagement-Rubric.pdf>

Therefore, PIPC urges the HELP Committee to require HHS to develop an infrastructure for meaningful patient engagement in all of its agencies, and to demonstrate to Congress how its engagement activities are making a difference in the management of its programs.

An Infrastructure for Measuring Value to the Patient

We recognize that the Office of Communication and Knowledge Transfer at the Agency for Healthcare Research and Quality (AHRQ) plays a vital role in the dissemination of information on the impact of innovative treatments. The office receives a substantial portion of the Patient-Centered Outcomes Research Trust Fund to support dissemination activities for comparative clinical effectiveness research. With the office's existing and explicit goal of affecting behavior change, we believe that AHRQ should be accountable for engaging patients and providers in assessing the value of the information for improving patient care, and in tailoring the communication of the information so that it can be used to support individualized care decisions. In this way, AHRQ could better support shared decision-making and the evolution of new payment and delivery models that embrace personalized medicine.

PIPC also believes that the National Institutes of Health (NIH) often plays a valuable role in the innovation process. Yet, we share the concern articulated in the report that, "It is difficult to quantify the value of NIH-supported research and track it through the entirety of the research continuum, as research findings could result in anything from generating broadly applicable new scientific knowledge, informing application of this knowledge in a clinical setting, or eventually resulting in an actual product used in patient care."⁵ PIPC encourages the HELP Committee to consider the experience of PCORI and its development of evaluation tools on the impact of research.

The need for a stronger and more effective infrastructure for patient engagement in assessing the value of innovation is apparent in the recent announcement by HHS Secretary Sylvia Burwell (Secretary) calling for new measurable goals intended to move the Medicare program further toward value-driven health care – "the first time in the history of the Medicare program that HHS has set explicit goals for alternative payment models [APMs] and value-based payments."⁶ PIPC recognizes that policymakers want to shift from health care payment based on volume to "value-based" models. As the Secretary seeks to develop and test new payment models, we also urge consideration of the significant implications these models will have on the transition to increasingly patient-centered healthcare, and the related issues of patient access and the physician-patient relationship. As part of our ongoing commitment to patient-centeredness in health care, PIPC recently developed a white paper to highlight some of the most important opportunities and issues that must be addressed in translating principles of patient-centeredness into value-based payment.⁷ It will be imperative for the Health Care Payment Learning and Action Network being created by HHS to promote value-based measurements to include the patient voice so that value *for the patient* is prioritized.

⁵ http://www.help.senate.gov/imo/media/Innovation_for_Healthier_Americans.pdf, at p.5

⁶ see <http://www.hhs.gov/news/press/2015pres/01/20150126a.html>

⁷ see <http://www.pipcpatients.org/PIPC-APM-White-Paper.pdf>

Without a strong infrastructure at HHS for patients to engage in defining value, an opportunity will be lost to ensure the effective incorporation of the patient perspective by all federal agencies—not just FDA—that are involved in the innovation cycle, particularly related to the identification and measurement of healthcare outcomes that matter to patients. PIPC strongly supports the development and use of patient experience data to inform the FDA’s regulatory pathways, and also to develop measures of quality of care being delivered. We urge the HELP Committee to recognize that the patient experience is also a valid consideration for the *delivery* of innovation, especially as access is potentially limited by new payment and delivery models that do not necessarily prioritize or even measure value *to the patient*.

Based on our experience in CER and the work of PCORI, we have noted the challenges of capturing outcomes that matter to patients in research due to the lack of existing rigorous methods for measuring those outcomes. Such challenges also seem to translate to capturing patient experiences in health care delivery. PIPC recently participated in a dialogue with Avalere Health, related to the development and use of patient-reported outcome (PRO) measures. We would urge the Committee to consider the following recommendations from the Avalere dialogue:

- Supplement existing PRO-related efforts by establishing a national measure development research agenda that reflects patient experience and patient engagement
- Continue to identify clinical areas where PRO measures can support high-quality, patient-centered care
- Refine and prioritize existing measures to establish their clinical practicality via testing and evaluation
- Invest in openly accessible tools that providers, payers, and patients can build into health information technology and clinical practice
- Create an interoperable, data-sharing mechanism that allows PRO data to be entered, used, and interpreted by every level of a care team (e.g., patient, caregiver, physician, nurse, physician’s assistant, post-acute care/long-term care provider)
- Support workforce development, training, and education to advance best practices for PRO data collection, interpretation, use, and evaluation
- Provisionally adopt PRO-based performance measures in pay-for-reporting and accreditation programs
- Gradually integrate PRO-based performance measures into provider practice transformation initiatives such as pay-for-performance, then into new payment and

delivery models⁸

In addition to the proactive development of PRO measures, we urge the HELP Committee to create clearly-defined pathways for patients to define the value of innovation. This includes patient engagement in the development of quality measures, in assessing the value of any information that is being disseminated from publicly-funded research, and in developing effective communications about research that support individualized assessments of the impact of treatments. Patients should play a leading role in assessing the value of innovation, and the development and use of measures that are the basis for determining value and quality.

In closing, thank you for this opportunity to submit our comments on the *Innovation for Healthier Americans* report. We look forward to working with you to advance a patient-centered health system.



Tony Coelho
Chairman, PIPC

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⁸ see <http://198.101.157.179/expertise/life-sciences/insights/avalere-white-paper-facilitating-a-transition-to-using-pros-to-measure-perf>