

**PIPC Comments on the 21st Century Cures Discussion Draft
February 10, 2015**

The Partnership to Improve Patient Care (PIPC) supports the goals of the 21st Century Cures Initiative of accelerating discovery, development and delivery of innovative treatments for many diseases that do not currently have treatment options. We applaud the House Energy and Commerce Committee for starting this discussion, and urge Chairman Fred Upton and Congresswoman Diana DeGette 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 are very pleased to see the Committee’s 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 would 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 the Committee’s analogy that “[t]he discovery, development, and delivery process is a cycle, meaning that data captured and analyzed on the delivery side informs new discoveries and better, more targeted solutions for patients.” On the upswing of that cycle, if being implemented in compliance with principles of patient-centeredness, is an informed and empowered patient being treated by providers with the 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

We applaud the Committee for explicitly requiring patient participation in the development of regulations to implement various components of the discussion draft. First, we agree that patients will be a vital voice in any workshop convened by the Department of Health and Human Services (HHS) to obtain input regarding methodologies for developing the guidance on patient experience data, which will facilitate the translation of this work to also support shared decision-making and the delivery of personalized medicine.¹ Second, we agree that patients are essential

¹ See page 13 of discussion draft, as it amends Section 505(y)(3)(B)(i) of the FDCA

in the development of evidentiary standards related to surrogate endpoints and the approval of breakthrough therapies.² Third, we are pleased to see patients represented on the proposed Expanded Access Task Force and Innovative Cures Consortium.³

PIPC also acknowledges that meaningful patient engagement requires that the patient voice not just be heard, but that it actually informs decision-making. We urge that the 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. 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 Committee’s discussion draft clearly seeks to build on the existing momentum to engage patients at the Food and Drug Administration (FDA). Yet, the Committee also includes “delivery of innovative treatments” as a goal of the discussion draft. 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. 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.

Therefore, PIPC urges the 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

The need for a stronger and more effective infrastructure for patient engagement 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

² See page 22 of discussion draft as it amends Section 507A(b)(2)(B)(III)(iii) of the FDCA

³ See page 87 of the discussion draft, Section 1124(b)(1)(C) (iii) and (iv) and (b)(1)(D)(iii) and (iv)

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

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.”⁵ Parallel to this announcement, the Secretary unveiled the launch of a newly-established “Health Care Payment Learning and Action Network [Network],” which she indicated will help “[t]o make these goals scalable beyond Medicare” and thus applicable to states (and state Medicaid programs) and consumers, as well as private partners – such as payers, employers, and providers.⁶ The first meeting of the Network convenes sometime in March 2015.

HHS sets out to have “85% of all Medicare fee-for-service [FFS] payments tied to quality or value by 2016, and 90% by 2018” – with a further goal of “tying 30% of [FFS] Medicare payments to quality or value through [APMs], such as Accountable Care Organizations [ACOs] or bundled payments by the end of 2016, and tying 50% of payments to these models by the end of 2018.”⁷ Regarding the former, HHS notes the role of the ongoing Hospital Value-Based Purchasing (VBP) and Hospital Readmissions Reduction programs as leverage in meeting these ambitious targets.

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.⁸ It will be imperative for the newly created Network to include the patient voice so that value *for the patient* is prioritized.

Without a strong infrastructure at HHS (particularly within CMS) 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. We urge the 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*.

Therefore, we urge the Committee to require patient participation on the newly created Health Care Payment Learning and Action Network. We also urge the Committee to require CMS to engage patients in determining the appropriate measures that should be applied as the basis for determining value and quality in its work to shift from health care payment based on volume to “value-based” models.

⁵ see <http://www.hhs.gov/news/press/2015pres/01/20150126a.html>

⁶ *id*

⁷ see <http://www.nejm.org/doi/full/10.1056/NEJMp1500445>

⁸ see <http://www.pipcpatients.org/PIPC-APM-White-Paper.pdf>

Patient Experience Data

PIPC strongly supports the development and use of patient experience data to enhance structured risk-benefit assessment frameworks at the FDA. As the Committee works through the complexities and details of these policies, we urge continued engagement of patients and providers, with an explicit goal of facilitating effective shared decision-making.

We are pleased that the discussion draft articulates the need for methodologies, standards, and potential experimental designs for patient-reported outcomes. 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. We 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 that 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 models⁹

In closing, thank you for this opportunity to submit our comments on the 21st Century Cures Discussion Document. We look forward to working with the committee and staff to advance a patient-centered health system.



Tony Coelho
Chairman, PIPC

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⁹ see <http://198.101.157.179/expertise/life-sciences/insights/avalere-white-paper-facilitating-a-transition-to-using-pros-to-measure-perf>