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for Health Policy & Economics

August 8, 2024

The Honorable Diana DeGette
The Honorable Larry Bucshon, M.D.
United States House of Representatives

Re: Response to Request for Information - additional reforms, support mechanisms, or incentives needed to enhance or improve the effectiveness of 21st Century Cures

Dear Representatives DeGette and Bucshon,

This letter responds to your request for input on the progress and future direction of the 21st Century Cures initiative. As experts in the field of patient preference research, we believe we may have a perspective on how you can improve upon the great strides taken through 21st Century Cures.[1]

The 21st Century Cures Act has made significant progress in advancing policies that promote patient access to innovative medical technologies. The Act has laid a strong foundation for incorporating patient perspectives into medical product development and regulatory decision-making. Specifically, Section 3002 of the Act addresses patient experience data and requires the FDA to issue guidance on methodologies for collecting such data, including approaches to identify what is most important to patients regarding disease burden, treatment burden, and benefits and risks in managing their condition. Furthermore, Section 3004 focuses on patient-focused drug development guidance, requiring the FDA to develop guidance on collecting patient experience data throughout drug development, methodologies to measure impacts on patients, and approaches to identify and develop methods to measure what is most important to patients.

Since the passage of the 21st Century Cures Act, there have been significant advances in the development of scientifically valid qualitative and quantitative methods in assessing patient perspectives through stated preference techniques The Medical Device Innovation Consortium (MDIC), in collaboration with U.S. FDA's Center for Devices and Radiological Health (CDRH), published a framework for assessing Patient-Centered Benefit Risks in 2016 (Ho et al. 2016), and the IMI PREFER project expanded and refined MDIC's original 14 methods of eliciting patient preferences to generate quantitative data that can be used in various stages of medical decision-making, including product development, clinical trials, regulatory approval, health technology assessment, and coverage and payment policies (IMI PREFER Consortium 2022).

Building on these advancements, a major structural reform that Congress could pursue is to further integrate patient preference information (PPI) into medical product development, regulatory decision-making, and Medicare coverage and payment. To further advance the use of PPI in product development and enhance patient access to new technologies, the following suggestions are proposed for your consideration:

[1] The opinions expressed in this document are solely those of the authors and do not necessarily reflect the views of the University of Southern California or the USC Schaeffer Center for Health Policy & Economics.

1. Expand and codify the application of PPI in regulatory and reimbursement decision-making.

The FDA's Center for Devices and Radiological Health (CDRH) has already prioritized the development and use of PPI to inform regulatory policy decisions, as evidenced by their patient preference-sensitive priority areas initiative (Webber et al. 2021). CDRH has already incorporated PPI into its guidance for breakthrough pathway designation for medical devices (CDRH/FDA 2023). Expanding a consistent approach to drugs and biologics and in other regulatory decision-making contexts would ensure consistency across all medical products. This could involve using PPI to demonstrate that a new therapy addresses an unmet medical need from the patient's perspective, or offers significant advantages over existing treatments in terms of outcomes that matter most to patients (Ho et al. 2015).

This expansion would build upon the Act's definition of "patient experience data" in Section 569C of the Federal Food, Drug, and Cosmetic Act, which includes information about patients' experiences with a disease or condition and its treatment, including patient preferences regarding treatment benefits and risks.

2. Develop a coordinated approach between FDA and CMS for evaluating endpoints and outcomes that matter most to patients, as identified through patient preference research.

A coordinated approach could involve joint scientific advice procedures between FDA and CMS, where PPI is used to inform the selection of clinically relevant endpoints and outcome measures. This would help ensure that the evidence generated during clinical trials is not only sufficient for regulatory approval but also meets the needs of payers for coverage decisions. The MDIC heart failure patient preference project, which involved multiple device manufacturers, demonstrated how PPI could be used to identify patient-relevant endpoints across various types of potential devices (Reed et al. 2022). MDIC's work into payer perspectives on patient preferences (MDIC 2022), as well as HTAi's research into the same topic (Hiligsmann et al. 2024) reveal that payers are interested in incorporating the patient perspective into their decision-making process. They need only the direction and approach to doing so, and Congress is in a unique position to help provide that direction.

This approach aligns with the Act's emphasis on patient-focused methodologies to measure impacts on patients and identify what is most important to them, as outlined in Section 3004 of the Cures Act.

3. Allocate financial support, potentially through PCORI, for patient organizations to conduct patient preference research.

Patient organizations are well-positioned to conduct preference studies due to their deep understanding of patient needs and their ability to engage diverse patient populations. However, many disease groups struggle to generate the financial support to provide the diverse range of patient support services that their constituencies need. As a result, many groups must turn to outside funding sources, which frequently come from industry. Due to the potential for perceived conflicts of interest, and the value that such data would provide to regulatory and reimbursement decision-makers, greater social value could be generated if patient groups could spearhead federally-funded preference research projects. Providing financial support would enable these organizations to conduct rigorous preference studies that could inform both product development and regulatory decision-making. Perhaps one vehicle to facilitate that funding would be to channel additional appropriations focused on patient preference data collection that could be used to assess and compare technologies through the Patient Centered Outcomes Research Institute (PCORI).

This initiative would complement the Act's promotion of data sharing in research, as outlined in Section 2014 of the Cures Act.

4. Utilize patient preferences to guide CMS policies, such as Coverage with Evidence Development (CED) or evaluation of drug value under the IRA drug price negotiation program.

Incorporating PPI into CMS policy decision-making could help ensure that coverage and payment decisions align with patient needs and perspectives. For example, in the context of CED, PPI could be used by CMS to:

a. Define the criteria to use when deciding when/whether to apply CED. For example, if questions or uncertainties that matter to patients (as measured in PPI) remain about that item or service, then CED could be justified. Otherwise, if PPI data indicate that patients are willing to tolerate the risks or uncertainties that might otherwise have been used to justify the application of CED, CMS would be restricted from applying that policy.

b. Inform the design of post-approval studies, ensuring that they focus on outcomes that are meaningful to patients (as measured in PPI). The CDRH-sponsored obesity weight-loss devices study demonstrated how PPI could be used to inform regulatory benefit-risk assessments and could serve as a model for integrating PPI into coverage decisions (Ho et al. 2015).

PPI could also be used by CMS in its drug price negotiation program to better understand what outcomes matter most to patients and the relative weights of those outcomes. Through this lens, CMS can better understand patients' priorities and the relative value of treatment alternatives. This application of PPI in coverage and payment decisions would extend the patient-focused approach beyond drug development and approval, as envisioned in the Act, to post-market decision-making processes.

By implementing these suggestions, Congress can build upon the successes of the 21st Century Cures Act and further ensure that medical product development and regulatory decisions align with patient needs and preferences. The IMI PREFER project has developed recommendations on how to assess and use PPI in medical product decision-making, which could serve as a valuable resource in implementing these proposals.

These recommendations aim to further the Act's goal of increasing the incorporation of patient perspectives and preferences into drug development and regulatory decision-making processes. They represent a continuation of the Act's emphasis on a more patient-centric approach to medical research and drug approval, recognizing the importance of understanding and considering patient experiences and preferences throughout the process.

Regards,

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