PIPC Partnership to Improve Patient Care

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Dear Dr. Chin:

The Partnership to Improve Patient Care (PIPC) appreciates this opportunity to comment on the Proposed Guidance Document for Coverage with Evidence Development (CED). We share the agency's concerns that a framework is needed for more predictable and transparent evidence development. We hope our comments are useful in finalizing a framework that is centered on achieving outcomes that matter to patients and people with disabilities.

Since its founding, the Partnership to Improve Patient Care (PIPC) has been at the forefront of applying principles of patient-centeredness to the nation's health care system – from the generation of comparative clinical effectiveness research at the Patient-Centered Outcomes Research Institute (PCORI), to the translation of evidence into patient care in a manner that achieves value to the patient. Having driven the concepts of patient-centeredness and patient engagement in the conduct of research, PIPC looks forward to bringing the voices of patients and people with disabilities to the discussion of how to advance patient-centered principles throughout an evolving health care system.

Our comments focus on the following concerns and recommendations:

- CED guidance should promote early and consistent engagement of patients and people with disabilities.
- Patient safeguards should promote health equity, not restrict access to care.
- CED should focus on real world data collection.
- CMS should establish standards for meeting evidence generation requirements.
- CED should clearly acknowledge and abide by the laws barring use of QALYs and similar measures.

CED guidance should promote early and consistent engagement of patients and people with disabilities.

Patients and people with disabilities should be engaged in CED determinations beyond written notice and comment opportunities. This includes AHRQ's prioritization of the needs and

priorities of the Medicare program, determinations of whether evidence is "insufficient" to determine "reasonable and necessary," and determinations of the CED measured outcomes that will address "specific evidentiary deficiencies." Early stakeholder engagement, including impacted patient communities, should first seek to understand the patient perspective related to whether a treatment is reasonable and necessary and whether it should be subject to CED. Once a determination is made to subject a treatment to CED, patient stakeholders should be engaged in identifying the specific evidentiary deficiencies, with ongoing engagement to identify the data to be collected to address such specific deficiencies.

PIPC had limited experience with the TVT Registry Project for TAVR in its early stages, in which patients were engaged but the goals of data collection were not particularly clear, leading to concerns about burdensome data collection requirements for providers without a clear understanding as to what data was required to be collected to achieve the "reasonable and necessary" standard. While patients had a seat at the table in the later stages of data collection for the TAVR CED, it was not clear how patients were engaged early in the process of selecting specific deficiencies on which data was to be collected, which would have made it much easier to then determine how to collect data and what information was important to be reported.

By updating its guidance, CMS can incorporate what has been learned from past CEDs to provide an improved framework for patient engagement. We would encourage CMS to build on the engagement best practices of entities such as the Patient-Centered Outcomes Research Institute (PCORI).[1] Similar to PCORI, CMS and sponsors of data collection activities should engage patients and people with disabilities and establish a predictable process for engagement in the CED process. This includes meaningful roles for patients and people with disabilities throughout the process, including in Medicare Evidence Development & Coverage Advisory Committee (MEDCAC) deliberations, in the implementation of data collection and the conclusion of a CED.

Patient safeguards should promote health equity, not restrict access to care.

We are concerned that CMS is advancing its goal of ensuring that systematic patient safeguards are in place by potentially advancing policies that encourage CED to restrict access to care for certain patients. Restricted access to care can have the unintended consequence of entrenching health disparities and work against the agency's goals of health equity by making it harder for treatments to reach the populations most in need. Once FDA has approved a treatment, it is important to recognize that safety and effectiveness is already established. At that point, patients should be safeguarded against restricted access to a treatment that FDA has deemed to have evidence of effectiveness, particularly patients with few other options.

[1] PCORI, "Engagement Rubric for Applicants," *Patient-Centered Outcomes Research Institute*, last modified June 6, 2016, https://www.pcori.org/sites/default/files/Engagement-Rubric.pdf.

Achieving broad knowledge of how a treatment impacts subgroups requires more, not less, access to the treatment on which data is being collected.

CED should focus on real world data collection and its use in shared decision-making.

We share concerns about representation in pivotal studies due to factors such as age and disability, and therefore strongly support subgroup analyses. For CED to generate real world evidence will require broad access to new treatments, consistent with the CMS principles seeking to expand access to medical technologies. Real world data collection will further support health equity by increasing the representation of subgroups in the evidence base and therefore expand information about a treatment's clinical benefit for those subgroups.

With real world evidence, CMS will be in a stronger position to advance its program for shared decision-making authorized by the Affordable Care Act to support preference sensitive care.[2] A 2018 letter strongly encouraged CMS to advance shared decision-making fundamentals for healthcare organizations, establish a measurement framework for shared decision-making, and then implement the "Drivers of Change" outlined by the National Quality Partners Playbook: Shared Decision-Making in Healthcare.[3] Advocacy organizations have recommended, "Providers whose reimbursement may be impacted by incentives to conduct shared decision-making should have appropriate guidance from CMS, and patients should have assurances that shared decision-making will empower them, not overwhelm them or steer them to a payer-preferred treatment."[4] While the Playbook is careful not to recommend shared decision-making as a requirement of CED, we urge CMS to recognize the significance of real world evidence to inform discussions between patients and providers and drive improved treatment decisions based on how treatments impact patients with similar needs, preferred outcomes and characteristics.[5] Real world evidence can address the need articulated by advocates for "specialized training to develop providers' person-centered communication capabilities, validation that patient decision aids meet the quality standards outlined in the Playbook and deference in coverage decisions to the outcome of a high-quality shared decision- making process."[6] CMS should focus its efforts on improving shared decision-making and supporting preference sensitive care, a core component of the statute establishing a Shared Decision-Making Program within CMS.

CMS should establish standards for meeting evidence generation requirements.

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[2] Patient Protection and Affordable Care Act. Section 936 [42 u. S. C. 299b-36] program to facilitate shared decision making. 2010.
[3] See http://www.pipcpatients.org/uploads/1/2/9/0/12902828/sdm-letter-to-cms-final.pdf
[4] See http://www.pipcpatients.org/uploads/1/2/9/0/12902828/sdm_comment_on_interoperabiity_final.pdf
[5] See http://www.pipcpatients.org/uploads/1/2/9/0/12902828/pipc_sdm_lung_cancer_ncd_final.pdf
[6] See http://www.pipcpatients.org/uploads/1/2/9/0/12902828/sdm_comment_on_interoperabiity_final.pdf

We are concerned that the standards for evidence generation to support coverage and data quality are not clearly defined, i.e. when evidence of health outcomes is determined to be sufficient. It is important to recognize that CMS sets the standard for some payers and that researchers will respond to CMS standards for high quality patient-centered evidence. For example, CMS indicates that some CEDs may require randomized clinical trials with placebo. Yet, upon FDA approval, we question whether a control group provided a placebo can be done ethically or morally when a treatment is already deemed safe and effective. Instead, we would urge generation of real-world evidence be the focus of CED, with the intent of extending more access to treatment, not less. In the draft guidance, it is also not clear how a CED is determined to have served its evidentiary purpose so that a treatment may go on to be fully covered. We are concerned that a CED could be misused to restrict access to certain treatments, particularly treatments for rare diseases and conditions or treatments for conditions that have few, if any, options. CMS should be clear in its guidance that CED is only intended to generate the evidence needed to meet the "reasonable and necessary" standard, with significant weight given to the patient and disability perspective as to whether that standard has been met.

CED should clearly acknowledge and abide by the laws barring use of QALYs and similar measures.

By law, CMS cannot reference measures of effectiveness that devalue disabled lives or discriminate to determine whether a treatment will be subject to CED.[7,8] We are concerned that the MEDCAC has referenced studies utilizing the quality-adjusted life year (QALY) in the past as part of its National Coverage Decision process, twice leading to a decision to subject a treatment to CED,[9,10] despite enactment of provisions in the Affordable Care Act (ACA) barring use of QALYs in Medicare coverage decisions.[11] In the final guidance, we urge CMS to acknowledge current nondiscrimination laws and the ban on using QALYs and similar measures to make Medicare decisions, including those related to coverage through CED.

In closing, we appreciate CMS' efforts to provide clarification of its CED authority through updated guidance. We hope that the final guidance will reflect policies that are centered on the

[7] 29 USC Sec 794, 2017.
[8] 42 USC Sec 12131, 2017.
[9] Tamra Syrek Jensen, et. al., "Chimeric Antigen Receptor (CAR) T-cell Therapy for Cancers," Medicare Coverage Database, Centers for Medicare and Medicaid Services, August 7, 2019, https://www.cms.gov/medicare-coverage-database/view/ncacal-decision-memo.aspx?proposed=N&NCAId=291. (Reference to ICER report)
[10] Tamra Syrek Jensen, et. al., "Monoclonal Antibodies Directed Against Amyloid for the Treatment of Alzheimer's Disease," Medicare Coverage Database, Centers for Medicare and Medicaid Services, January 11, 2022, https://www.cms.gov/medicare-coverage-database/view/ncacal-decision-memo.aspx?proposed=Y&NCAId=305. (Footnote to ICER report)
[11] House of Representatives, Congress. 42 U.S.C. 1320e - Comparative clinical effectiveness research. U.S. Government Publishing Office, https://www.govinfo.gov/app/details/USCODE-2010-title42/USCODE-2010-title42-chap7-subchapXI-partD-sec1320e

needs of patients and people with disabilities, especially those with few options for treatment that stand to benefit most from newly approved treatments by the FDA.

Sincerely,

Tony Coelho