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ASSISTANT SECRETARY FOR PLANNING AND EVALUATION

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PROJECT REPORT

U.S. Department of Health and Human Services Stakeholder Engagement Report Executive Summary

Analysis and Synthesis of Key Themes

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Acknowledgments

Executive Summary

High-quality data are necessary for patient-centered outcomes research (PCOR) to produce new knowledge that informs healthcare decisions and improves patient outcomes. PCOR studies are designed to rigorously evaluate the outcomes and effectiveness of healthcare interventions and generate evidence that is relevant to patients in consideration of diverse preferences and values. PCOR provides decision makers, patients, their families, and their healthcare providers with objective, scientific evidence on the effectiveness of treatments, services, and other healthcare interventions.[1] The many and varied sources of health data are the core building blocks of PCOR.

The Patient-Centered Outcomes Research Trust Fund (PCORTF), established in 2010, builds and enables data capacity and infrastructure for the conduct of PCOR studies. The PCORTF supports the work of the Patient-Centered Outcomes Research Institute, as well as related activities at the Agency for Healthcare Research and Quality and the Office of the Secretary (OS) of the Department of Health and Human Services (HHS). The OS-HHS carries out its work under the auspices of the Office of the Secretary Patient-Centered Outcomes Research Trust Fund (OS-PCORTF) portfolio. Under a delegation of authority by the Secretary, the Assistant Secretary for Planning and Evaluation (ASPE) collaborates with agency leaders to coordinate, manage, and delegate funding for new OS-PCORTF projects to build data capacity for PCOR.[2]

In December 2019, Congress reauthorized the PCORTF through 2029. With the reauthorization, intellectual and developmental disabilities (ID/DD) and maternal mortality were named as research priorities, and the scope of outcomes data was expanded to include the potential burdens and economic impacts of medical treatments, items, and services relevant to meeting the needs of different stakeholders and decision makers.[3] With the renewed and expanded scope and the advances in health information technology and tools for interoperability over the past decade, ASPE is working with internal stakeholders affiliated with HHS agencies and external stakeholders to update and modernize the OS-PCORTF Strategic Plan for the next decade.

This report provides a summary of the interviews conducted with key HHS stakeholders to inform the 2021–2029 OS-PCORTF Strategic Plan. The Strategic Plan will help chart the course for HHS and other federal health agencies to work collaboratively and with other entities to build data capacity for PCOR studies. These findings, in conjunction with input from internal and external stakeholders and public participation, will inform development of a strategy to guide the long-term direction of the OS-PCORTF portfolio. A well-informed and innovative strategy will support the OS-PCORTF vision of providing the coordination and collaboration necessary to build data capacity for PCOR and generate evidence to inform decision making to improve health outcomes for all Americans.

ASPE's OS-PCORTF portfolio engaged the Health federally funded research and development center (Health FFRDC), operated by The MITRE Corporation, to conduct interviews and subsequently analyze responses from the interviews. The HHS stakeholder engagement and interviews were designed in partnership with a group of agency representatives that are referred to as the ASPE's Strategic Planning Engagement Council. The main goals of the interviews were to understand (1) key agency priorities related to PCOR, (2) existing work that can be leveraged to improve data capacity, (3) gaps and opportunities, (4) research trends and legislative and policy drivers in the environment, and (5) how the current process for creating and funding OS-PCORTF projects could be improved within the context of an

evolving healthcare system and new priorities for PCOR. From March to May 2021, OS-PCORTF and the Health FFRDC conducted 32 interviews with 62 participants, including HHS agency leaders, leaders of OS-PCORTF projects, and agency data experts.

The Health FFRDC team completed the following steps for the qualitative analysis:

1. Entered interview findings and transcripts into RIGOR, a Health FFRDC-developed qualitative analysis software tool.
2. Developed a coding taxonomy based on keywords from interview questions and PCOR concepts discussed.
3. Summarized source language in RIGOR to arrive at 1,292 observations. An observation is defined as a brief, objective summary statement of a portion of the direct transcription.
4. Coded, sorted, and grouped like observations and labeled each with a conclusion statement to arrive at 134 conclusions. A conclusion is an objective summary statement of like observations.
5. Clustered like conclusions into similar groups to develop 12 common themes in five broad categories focused on ways to enhance data infrastructure for the OS-PCORTF. A theme is a summary statement of like conclusions, written in the form of a problem statement.

As shown in Table 1, the 12 themes were each categorized into one of the following topic areas: (1) Health Equity and Populations of Focus, (2) Data Infrastructure, (3) Collaboration, (4) OS-PCORTF Portfolio Management, and (5) Workforce Capacity.

Table 1: Themes Across Topic Areas

Twelve Themes Across Topic Areas
Health Equity and Populations of Focus
1. There is a need to apply a health disparities and equity lens across platforms, projects, and data sources/core datasets to address research needs for underserved, underrepresented, and at-risk groups and populations.   2. Data for PCOR are insufficient in quantity, quality, and linkages for many population segments (e.g., Coronavirus Disease 2019 [COVID-19], ID/DD, Maternal/Child Health, Opioid Use).
Data Infrastructure
3. PCOR research requires the OS-PCORTF to be at the leading edge of new technologies and methods (e.g., artificial intelligence/machine learning, natural language processing, advanced analytics) to address issues with data access, analysis, and sharing.   4. Strategies are needed for using real-world data for practical application for PCOR.   5. There is a need for efficient and sustainable/reusable data infrastructure that addresses standardization, liquidity, and transparency.   6. There is a continued need to invest in sustainable, robust data linkages to demonstrate intervention-associated outcomes.   7. There are limitations in the completeness, timeliness, access, cost, exchange, and granularity of data for effective PCOR.   8. There is a need to develop policy/policies policy and governance approaches to follow patients over time and across settings that protect privacy and identity.

Twelve Themes Across Topic Areas
Collaboration

	9. There is a need to improve ASPE's collaboration infrastructure to build, scale, and sustain data capacity for PCOR studies.

OS-PCORTF Portfolio Management

	10. The OS-PCORTF portfolio must respond quickly to shifting departmental priorities and policies by funding projects to meet emerging needs. 11. There are challenges with the OS-PCORTF funding application process that affect the quality of applications.

Workforce Capacity

	12. The current data science workforce across HHS limits the ability of agencies to engage in data analysis and sustain project outputs for PCOR.

Robust data capacity and infrastructure should support the OS-PCORTF core functionalities highlighted in the *Building Data Capacity for Patient-Centered Outcomes Research in HHS: A Formative Evaluation of 2012-2016 Projects* report. These functionalities serve as pillars in the OS-PCORTF Strategic Framework and are "core research functions and HHS focus areas for enhancing and improving data infrastructure."[4] However, the findings from the 2021 HHS stakeholder engagement interviews suggest expansion, refinement, and new foci. Table 2 summarizes each of the five functionalities and opportunities for enhancement or reconceptualization.

Table 2: OS-PCORTF Core Functionalities: Opportunities for Enhancement

OS-PCORTF Core Functionalities: Opportunities for Enhancement	
Functionality	**Opportunities for Enhancement**
Use of Clinical Data for Research: Stems from multiple sources of clinical data available for research (e.g., electronic health records [EHRs], administrative claims, data available via patient portals, registries); and efforts in this area focus on improving access and interoperability of clinical data for query and analysis.	• Develop tools and resources to support the use of EHR data for PCOR • Expand the collection and analysis of data beyond clinical data to include social determinants of health and measures of well-being
Standardized Collection of Standardized Clinical Data: Supports the use of common data elements to enable more effective, efficient linking and aggregating across data sources.	• Focus on the development and adoption of data standards

OS-PCORTF Core Functionalities: Opportunities for Enhancement	
Functionality	**Opportunities for Enhancement**
Linking Clinical and Other Data for Research: Allows researchers to collect longitudinal patient information and to link data sets with other relevant information for research.	• Expand focus on linking health and human services data to generate a richer understanding of patient outcomes • Address critical governance issues, including privacy and limitations on sharing linked data sets
Collection of Participant-Provided Information: Via new data collection technologies provides a means for collecting patient-generated information critical to PCOR.	• Grow this underdeveloped area by addressing (1) data standardization, (2) quality issues of participant-provided data, and (3) integration of patient-provided information into research and clinical care
Use of Enhanced Publicly Funded Data: Focuses on efforts to leverage current investments in federally available data and infrastructure to inform future infrastructure development.	• Focus on making HHS data assets findable, accessible, interoperable, and reusable (FAIR) • Build on COVID-19 collaborations and infrastructure initiated across HHS and through fiscal year 2021 COVID-19 OS-PCORTF funded projects

In addition to opportunities for enhancement of the core functionalities, the 2021 HHS stakeholder engagement interviews identified new ideas to enhance data infrastructure for PCOR stakeholders that are critical and currently not highly prioritized in the OS-PCORTF core five functionalities. New considerations to enhance data infrastructure include the following recommendations:

- Establish health equity as a foundational concept of the OS-PCORTF Strategic Framework, given the prioritization and prominence as a theme to improve PCOR and reduce disparities in health outcomes.
- Expand efforts to collect and link complete data for PCOR on populations of focus, including COVID-19, ID/DD, maternal and child health, and opioid use. The reauthorization of OS-PCORTF supports expanding PCOR for these priority areas as well.
- Continue leadership and investment in driving collaboration for PCOR across and beyond HHS.

The reauthorization of the OS-PCORTF, including named research priorities and expanded scope of outcome data, offers a unique collaborative opportunity to identify and address data capacity issues for the PCOR community. Looking ahead, the results from the HHS stakeholder interviews point to a number of considerations for strategic and implementation planning.

Set priorities and develop clear criteria to assess projects and outcomes:

- The FAIR principle offers useful guidance for assessment of HHS data assets to ensure that a wide range of researchers and policy makers can make best use of the government's data assets.
- A key HHS priority is to apply policy levers to drive the adoption of data standards to promote interoperability and improve the value and efficiency of HHS data investments.

Focus on the strategic management of the portfolio:

- Funding opportunities must continue to be responsive to changing Secretarial priorities. Balancing short-term use cases and longer-term activities of the portfolio will support advances in PCOR data capacity.
- Projects within the OS-PCORTF portfolio are often focused on a specific use case. In funding projects, the portfolio should continue to develop a mix of projects that both address specific policy challenges and build technical infrastructure across use cases.
- Periodic reassessment of the OS-PCORTF Strategic Framework and progress toward the five functionalities over the next decade would further illuminate challenges and help identify potential solutions emerging from the dynamic interplay of the different components of the framework.

Collaborate inclusively to support advancements in data capacity and PCOR:

- The new portfolio should continue to build collaboration structures that extend beyond the boundaries of individual projects to reduce fragmentation and produce innovative ideas and approaches. Most recent examples include the Maternal Health Consortium, a consortium among three projects focused on maternal-health-related issues, and Collaboration on Data for Evidence, a collaborative among seven COVID-19 projects funded in 2021.
- ASPE should share timelines and processes for developing and awarding projects, so that other agencies can integrate the process into their internal planning and operations.
- Over the course of the next decade, it would be beneficial for ASPE and HHS to find ways to sustain engagements to strengthen data capacity for PCOR.

Recognize potential limitations:

- Moving to the vision of open government data called for in the Foundations for Evidence Based Policymaking Act of 2018 (Evidence Act) will require technical and cultural changes in how agencies collect, use, and share data.
- Data science expertise may be a limiting factor in how quickly work can be accomplished.

These findings, in conjunction with input from internal and external stakeholders and public participation, will inform the development of an evidence-based strategy to guide the long-term direction of the OS-PCORTF portfolio. Other inputs to the Strategic Plan include a listening session with external stakeholders; the results of an environmental scan; interviews with clinical registries and health outcomes research data networks stakeholders; observations from ongoing COVID-19 projects; and conclusions from the National Academies of Sciences, Engineering, and Medicine consensus panel and three public workshops (pending). Findings across all these sources will be synthesized and analyzed to determine themes and support the development of mission, vision, guiding principles, goals, outcomes, and objectives for the Strategic Plan and future implementation planning.

The Strategic Plan is a key step in charting the course for the OS-PCORTF portfolio. The OS-PCORTF will engage the HHS agencies in collaboration to build data capacity for PCOR from 2021 through 2029. It will guide the operation and the long-term execution of the OS-PCORTF portfolio.

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