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Building Data Capacity for Patient-Centered Outcomes Research in HHS: A Formative Evaluation of 2012-2016 Projects

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SYNOPSIS

The purpose of this report is to present the findings of a formative evaluation to assess the progress of the OS PCORTF portfolio at a point in time and how the findings inform future efforts to build data capacity to support clinical comparative effectiveness research (CER) and patient-centered outcomes research (PCOR). This report describes a formative evaluation of OS-PCORTF projects that were active or completed between 2012 to 2016.

The projects were supported by the Patient-Centered Outcomes Research Trust Fund (PCORTF) and administered by the Office of the Assistant Secretary for Planning and Evaluation (ASPE) for the United States Department of Health and Human Services (HHS).

HHS has identified 4 components necessary to build data capacity to support PCOR:

1. Standards, or accepted specifications that ensure that the data used for PCOR are consistent and usable across different sources and for different uses,
2. Services, such as programming protocols and interfaces, that allow for the capture, storage, linkage, analysis, and exchange of clinical data or evidence,
3. Policies that address how data are used and to ensure that data are protected and secure, and
4. Governance structures to support data sharing among organizations.

The evaluation assessed the progress made by the OS PCORTF portfolio towards implementation of these 4 components and 5 specific research functionalities identified in the HHS Strategic Framework for Building Data Capacity. The evaluation also examined the perspectives of federal and nonfederal stakeholders to better understand the current status of the universe of data capacity-building projects, identify strengths and limitations of the current work, and to determine areas where further work is needed.

The evaluation found that:

- **Efforts to improve standards, services, policies, and governance have moved from planning to implementation.** The most significant progress has been made in standards, particularly standards for common data elements, and in services, which encompass the resources to capture, store, and exchange data. Further work is most needed to implement policies that oversee data use, security and privacy, and to create governance structures that support the efficient use of data.
- **Significant progress has been made toward the core functionalities identified in the HHS Strategic Framework, especially the use of clinical data for research and the standardized collection of standardized clinical data.**
- **Modest progress has been made on standards, services, policies and governance aimed at improving data quality.**

Future projects are needed to explore how to enhance data privacy and security, ensure data quality, address data governance, and operationalize related standards.

Potential specific areas for future research include:

- Developing technical services and standards for services that allow patient data to be securely linked to other data sources.
- Developing standards, services, and policies to assure data quality for research.
- Creating a policy framework that preserves security and privacy while improving the ability to access and query clinical data by researchers.
- Developing a better understanding and methods to address the socio-legal challenges that arise with using patient data for research.
- Dissemination efforts to promote greater awareness of OS-PCORTF initiatives and products among members of the research community.

EXECUTIVE SUMMARY

Overview of the PCORTF

The Patient-Centered Outcomes Research Trust Fund (PCORTF) was established as part of the 2010 Patient Protection and Affordable Care Act (ACA). It supports the work of 3 entities and areas of work (see **Exhibit ES-1**) essential to conduct, disseminate, and build capacity for PCOR. PCOR is defined as evidence-based research that helps people and their caregivers communicate and make informed health care decisions, allowing their voices to be heard in assessing the value of health care options.[1]

Exhibit ES-1. Three PCORTF-Supported Entities

Entity	Directive	Percentage of Funds
Patient-Centered Outcomes Research Institute (PCORI)	Conduct research to advance evidence on patient-centered health outcomes	80%
Agency for Healthcare Research and Quality (AHRQ)	Disseminate findings from PCOR, integrate findings into clinical practice, train researchers	16%
HHS Secretary	Build data capacity for comparative effectiveness research (CER)*	4%

The Office of the Assistant Secretary for Planning and Evaluation (ASPE) coordinates and administers HHS' efforts under the PCORTF. The ACA charged the secretary of HHS, to:

> *…provide for the coordination of relevant Federal health programs to build data capacity for comparative clinical effectiveness research,[*] including the development and use of clinical registries and health outcomes research networks, in order to develop and maintain a comprehensive, interoperable data network to collect, link, and analyze data on outcomes and effectiveness from multiple sources including electronic health records (EHRs).*[2]

PCOR is designed to inform health care decisions by providing evidence of the effectiveness, benefits, and harms of different treatment options. The evidence is generated from research studies that compare drugs, medical devices, tests, surgeries, or ways to deliver health care. PCOR also helps individuals and their caregivers communicate and make informed health care decisions, allowing their voices to be heard in assessing the value of health care options. Individual patients want to know whether the treatment their health care provider has prescribed will benefit them given their current health status, their concurrent treatments, and their age or other demographic status.

To answer these questions with a high degree of accuracy requires analysis of very large data sets. The data sets must be clinically rich, complete, and longitudinal to allow investigators to evaluate

[*] For consistency of terminology in this report, clinical comparative effectiveness research also refers to patient-centered outcomes research.

how particular treatments might benefit the individual patient over time, given the interaction among patients' current conditions, behavioral characteristics, and demographics. Such rich, large, population-based real-world data sets are rare for many reasons. Current data sets may have some, but not all, of the data needed to conduct PCOR.

Building the data capacity needed to conduct PCOR can be achieved in two main ways:[3]

1. Create more data (via creation of registries, new data networks, etc.); or,
2. Create what's needed to make existing and future electronic health data more usable or "liquid" for CER and PCOR purposes

For data to be used for PCOR, they must be consistent, valid, and capable of being linked across patients and data sets. In addition, research-quality data must be able to reflect patient health over time and include comprehensive information on patient demographics, diagnoses, procedures, and medications.

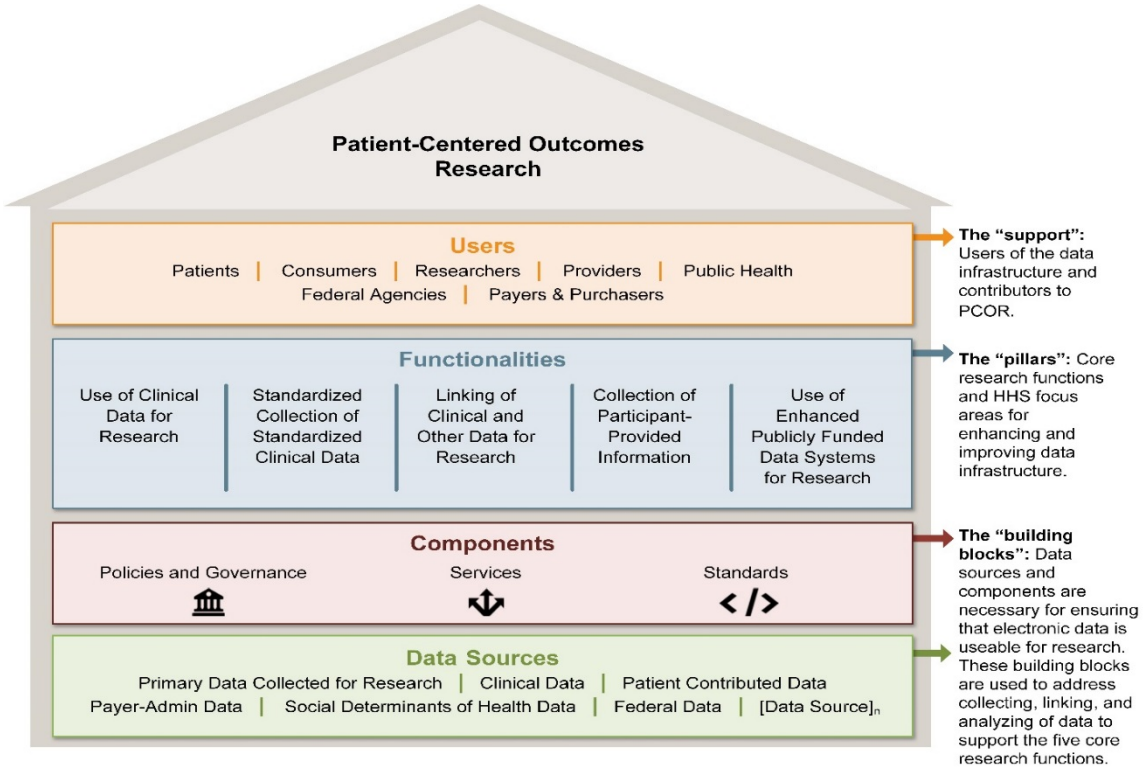
ASPE's Role in Creating a Framework for PCOR Data Infrastructure

In its role as a coordinator of HHS programs to build data capacity, ASPE convened the PCORTF Leadership Council, which collaborated with external researchers to develop the *HHS Strategic Roadmap for Building Data Capacity for Clinical Comparative Effectiveness Research (CER*). The Roadmap includes both a Strategic Framework to guide OS-PCORTF data-capacity investments and a series of milestones against which to measure progress.

The Strategic Framework is analogous to a 3-level structure where each level enables the creation, enhancement, and usability of data for PCOR (see **Exhibit ES-2**). At the foundation are the data sources needed to support PCOR. The next level represents the components, or "building blocks" needed to ensure that data are usable for PCOR. The components include the following elements.

1. **Standards** are nationally accepted specifications that have been widely approved and adopted because of market forces, community consensus, or regulatory requirements.
2. **Services** are resources that researchers can use to capture, store, link, analyze, or exchange data or evidence.
3. **Policies** are federal rules or guidelines that need to be established to ensure, for example, that identity checking and security and privacy rules are followed; patient data are protected; and other established standards and services are followed.
4. **Governance structures** support the efficient use of the data infrastructure for research across individual and organizations' boundaries of control and ownership

Exhibit ES-2. HHS Strategic Framework for PCOR Data Infrastructure



Within each component, specific examples are referred to as "developmental components." For example, the standards component includes a developmental component called *Standards for electronic health records to interact with forms*. In the HHS Strategic Framework, 42 specific developmental components are identified within the 3 components, and are used to gauge progress in this evaluation.

The components provide essential support for the next level, the pillars, or "functionalities," needed to enhance and improve data infrastructure:

1. ***Use of Clinical Data for Research*** stems from multiple sources of clinical data available for research (e.g., 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.

2. ***Standardized Collection of Standardized Clinical Data*** supports the use of common data elements to enable more effective, efficient linking and aggregating across data sources.

3. ***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.

4. ***Collection of Participant-Provided Information (PPI)*** via new data collection technologies provides means for collecting patient-generated information critical to PCOR.

5. ***Use of Enhanced Publicly Funded Data Systems for Research*** focuses on efforts to leverage current investments in federally available data and infrastructure to inform future infrastructure development.

Developmental Components and Milestones

The HHS Strategic Framework also identified specific milestones to be achieved to assess progress toward the functionalities. Milestones are defined in terms of developmental components that must be implemented for the milestones to be attained. **Exhibit ES-3** shows an example for one functionality, ***Standardized Collection of Standardized Clinical Data***, a milestone, and the developmental components required to achieve the milestone. **Exhibit ES-4** displays the full list of developmental components aligned with their component categories. Note that the milestones identified in the Roadmap and Strategic framework are all targeted at times in the future (mainly 2018 and 2019), and there are no milestones that apply to the period of time that this evaluation examined. ***Appendix A*** provides a glossary of terms. The milestones for each of the functionalities are shown in ***Appendix B*****.**

Exhibit ES-3. Milestones to Assess Progress Toward the Functionality of *Standardized Collection of Standardized Clinical Data*

Functionality	Milestones	Developmental Components
Standardized Collection of Standardized Clinical Data	By 2018, support the development of repositories/portals for CDEs, standards for utilizing CDEs for research, and services to allow researchers to easily utilize standardized components.	▪ Services for contribution and/or harmonization of CDEs ▪ Service(s) that code elements and questions used to collect data for research to common clinical standards (e.g., SNOMED CT) ▪ CDE representation standards ▪ Standards for forms using CDEs ▪ Standards for EHRs to interact with forms and forms libraries

Abbreviations: CDE = common data element; SNOMED-CT = Systematized Nomenclature of Medicine-Clinical Terms.

Methodology

RTI International conducted a mixed-methods evaluation to assess the progress of the OS-PCORTF project portfolio in building data capacity for PCOR and to assess where further work is needed. The evaluation examined the collective progress of 26 OS-PCORTF projects that were active or completed between 2012 and 2016 toward meeting milestones in support of the functionalities needed to enhance and improve PCOR data capacity. The evaluation also examined insights gleaned from interviews with 45 key stakeholders to gauge the progress of federal data capacity-building efforts, identify strengths and limitations, and determine future areas of activity.

The evaluation addressed 3 overarching research questions:

1. What contributions has the OS-PCORTF portfolio of projects made to strengthening the components (or building blocks) of standards, services, policies, and governance needed to effectively conduct PCOR?
2. To what extent has the OS-PCORTF portfolio of projects enabled the research functionalities (or pillars) outlined in the Strategic Framework to improve data capacity, including use of clinical data for research, standardized collection of standardized clinical data, linking of clinical data, collection of PPI, and use of publicly funded data systems?
3. Is the Roadmap and Strategic Framework sufficiently comprehensive to build clinical data capacity for PCOR and advance researchers' ability to capture, store, access, link, exchange, and analyze data securely and efficiently?

Evaluating OS-PCORTF Project Activities

The evaluation assessed overall progress made by the OS-PCORTF portfolio of projects conducted between 2012 and 2016—specifically, progress of the project portfolio toward reaching the milestones outlined in HHS' Roadmap. For example, "by 2018, support the development of a set of research common data elements (CDEs) in specific gap areas and support development of a governance structure for CDE harmonization" is a key milestone for fulfilling the functionality ***Standardized Collection of Standardized Clinical Data***. Progress toward the milestone depends on implementation of the developmental components in that milestone. In this example, project activities to attain the milestone must address developing services for contribution and/or harmonization of common data elements, services that code elements and questions used to collect data for research to common clinical standards (such as SNOMED CT), common data element representation standards, and standards for forms using common data elements.

The Technical Expert Panel proposed that RTI adopt a "maturity schema" to help guide the evaluation. The Capability Maturity Model (CMM) is a process framework initially developed to guide organizations with software development processes by assigning "maturity levels" for various stages of process improvement.[4] The 5 core functionalities identified as critical to building data capacity can be viewed as process goals or capabilities to be enabled. In this evaluation, the CMM is used to assess progress toward enabling the functionalities. Each level of enablement signifies a stage of achievement relative to process maturity and is a foundation for successive levels. The maturity schema provides the lens through which this evaluation views, assesses, and interprets progress on the various evaluation outcomes discussed in more detail below. Using the CMM, progress toward implementation of the developmental components and enablement of the functionalities was assessed along a continuum ranging from "no implementation" to "full implementation" for the developmental components, and "not enabled" to "fully enabled" for the core functionalities. The CMM also recognizes that improving data capacity is an ongoing process. As medicine advances, there will always be new data and data types to structure, collect, link, and analyze—so that even if a functionality is determined to be "fully enabled" at a point in time, it

does not mean the work is complete. It is important to note that the findings of this evaluation reflect a point in time.

Evaluating Stakeholder Input

To evaluate progress toward building data capacity, 45 stakeholders within HHS agencies and from other public and private organizations were interviewed to understand their impressions of OS-PCORTF investments to build data capacity for PCOR. They shared their impressions of HHS' efforts to date and helped identify future opportunities and investments to build data capacity. In addition to addressing the 3 overarching questions, input was solicited on the following 4 topics:

1. Understand key stakeholders' views of the core research functions needed to address gaps in data capacity for PCOR.
2. Evaluate whether and how various key stakeholders used the products from the OS-PCORTF portfolio of projects to enable core research functions.
3. Assess how OS-PCORTF portfolio of projects informed and contributed to key federal stakeholders' research needs, helped to avoid duplication, and fostered coordination across HHS.
4. Understand how OS-PCORTF projects and products are perceived to have addressed the research needs of federally and privately funded research stakeholders.

Interviews were conducted by telephone with participants from 5 stakeholder groups. Two groups came from HHS and consisted of federal agency leaders and HHS project leads. The 3 remaining groups consisted of groups involved in data capacity initiatives including research network leaders, health care delivery system and payer representatives, and patient advocates.

Results

OS-PCORTF Project Portfolio Evaluation Results

The results of the evaluation of the portfolio's project activities are organized by the 3 main research questions.

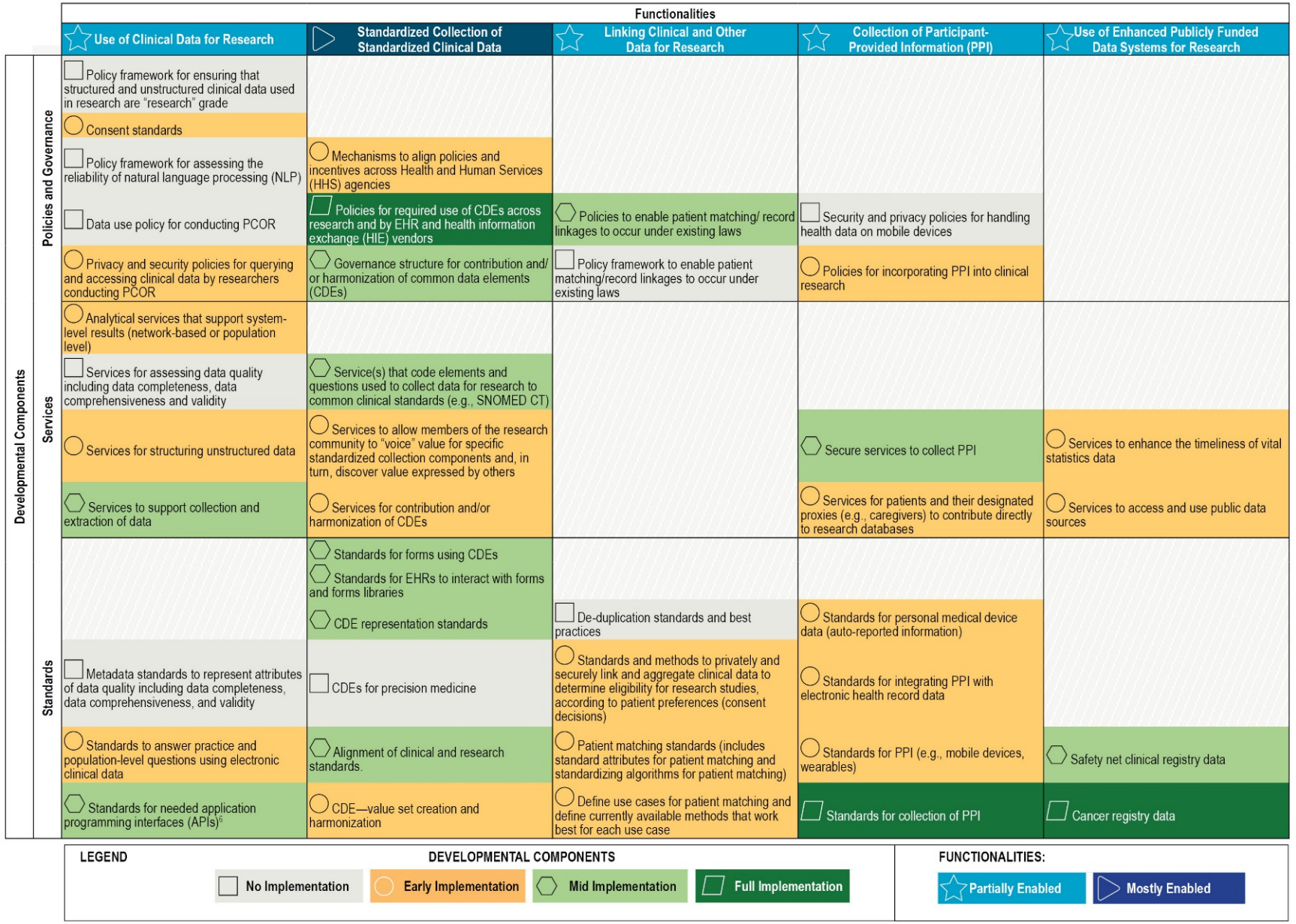
Question 1: What contributions has the OS-PCORTF portfolio of projects made to strengthening the standards, services, policies, and governance needed to effectively conduct PCOR?

The activities conducted by the OS-PCORTF portfolio of projects supported 33 of the 42 developmental components. The largest number of developmental components supported by project activities were in the area of development of standards (17), followed by the development of services (10), and the development of policies and governance (6). Nine developmental components were not addressed by any of the projects in the portfolio (standards n=3, services n=1, and policies and governance n=5).

The evaluation found that:

- The developmental component *standards for application programming interfaces (APIs)* was addressed by the largest number of projects (13). Almost as many projects (12) addressed the developmental component *services to support data collection and extraction*. One project, "Creating the Foundation Building Blocks for the Learning Healthcare System: Data Access Standards for Electronic Health Records (EHRs)," led by the Office of the National Coordinator for Health Information Technology (ONC), laid the groundwork for APIs and other services needed to access EHR data. These capabilities are critical to successful collection, linkage, and analysis of standardized data contained in EHRs.
- Across all projects, the greatest effort was targeted at standards, services, and policies for common data elements. The ONC project, "Creating the Foundational Building Blocks for the Learning Health System: Structured Data Capture" was a key contributor, addressing standards for common data elements, creation of common data elements for certain chronic conditions, and creation of a registry for those elements in collaboration with the National Library of Medicine. Standardized common data elements are essential to link and analyze clinical data across EHR platforms.
- The majority of developmental components were in the early implementation phase. Three were fully implemented, 11 were in mid-implementation, 19 were in early implementation, and 9 developmental components were not supported by any project activities (no implementation. The Heat Map (**Exhibit ES-4**) shows implementation levels for each developmental component.

Exhibit ES-4. Heat Map of Developmental Components Needed to Enable Functionalities



Question 2: To what extent have the OS-PCORTF projects enabled the functionalities (or pillars) that improve data capacity, including use of clinical data for research, standardizing collection of clinical data, linking of clinical data, collection of PPI, and use of publicly funded data systems?

Progress toward the functionalities was measured on a continuum ranging from 'not enabled' to 'fully enabled.' The evaluation found that one of the functionalities was mostly enabled and the others were partially enabled by the work of the portfolio.

The most progress was made toward ***Standardized Collection of Standardized Clinical Data***, which was scored as "mostly enabled" (level 3 of 4) on the 4-step continuum. Eight projects included activities that contributed to developmental components that led to milestone achievement for this functionality. In addition, the developmental components for this functionality were collectively farther along in implementation than for other functionalities. One developmental component, *Policies for the use of CDEs across research and by EHR and health information exchange (HIE) vendors,* was scored as fully implemented. Of the projects contributing to this functionality, 2 ONC projects, "Structured Data Capture" and the "Data Access Framework" were instrumental to enabling the functionality. The most progress was made toward milestones for the development of CDEs and policies to promote the adoption and use of standardized collection components and services.

The ***Use of Clinical Data for Research*** was scored as partially enabled (level 2 of 4), although it had the largest number of OS-PCORTF projects (15) supporting it. Four developmental components for the functionality, mainly relating to policy development and assessing data quality, were not addressed by any project, which resulted in less progress toward the milestones for this functionality.

Use of Enhanced Publicly Funded Data Systems for Research and ***Collection of Participant-Provided Information (PPI)*** were supported by 7 projects and 4 projects, respectively. Consequently, less progress was made toward the implementation of the developmental components and attainment of milestones for these functionalities.

Although ***Linking Clinical and Other Data for Research***, was partially enabled, it had only 3 projects with activities supporting the developmental components and milestones for the functionality, and is thus the least supported of the 5 high-priority functionalities.

Question 3: Is the OS PCORTF Strategic Framework sufficiently comprehensive to build clinical data capacity for PCOR and advance researchers' ability to capture, store, access, link, exchange, and analyze data securely and efficiently?

The project portfolio analysis portion of the evaluation primarily assessed progress toward the components and functionalities set forth in the Strategic Framework. There were no results that suggested that the 5 high-priority functionalities were inadequate for building

data capacity. However, activities in 2 projects suggested that the current developmental components were inadequate to document and measure the full scope of work needed to enable the 5 functionalities. Specifically, the ONC project, "Security and Privacy Standards for Patient Matching, Linking and Aggregation" includes project activities to create necessary services for secure, private linkage of data. However, no developmental component currently exists for this activity in the current HHS framework. This suggests that a developmental component may be missing, and this additional developmental component is proposed: *Services to securely and privately link data,* to help document and measure progress toward enablement of the functionality ***Linking Clinical and Other Data for Research***.

Similarly, the activities conducted in the ONC project, "PCOR Privacy and Security Blueprint Legal Analysis and Ethics Framework for Data Use and Use of Technology for Privacy," suggest that an additional developmental component may be needed: *Policy framework for privacy-preserving access and querying of clinical data by researchers* to measure progress toward the functionality ***Use of Clinical Data for Research***.

Stakeholder Evaluation Results

In addition to the OS-PCORTF portfolio review, 45 key stakeholders were interviewed to better understand the progress of federal and private sector data capacity-building efforts, the strengths and limitations of current initiatives, and needed future areas of focus. Overall, those interviewed observed that the OS-PCORTF project portfolio represents a substantial body of work, that the core research functions provide sufficient strategic markers for building data capacity for PCOR, and that the projects have addressed core research functions. Roughly 75% of the research network representatives who were interviewed were familiar with the OS-PCORTF projects and products, and of those, one-third confirmed that they had knowingly used the products developed. They suggested that HHS identify ways to share the knowledge gained from these projects more broadly.

A majority of stakeholders suggested that as the landscape evolves, the project portfolio would benefit from increased focus in 5 strategic areas, including:

- **Implementing Standards**: Develop best practices to develop, implement, and maintain data standards so that health care and research institutions can reduce the time and costs incurred when implementing and updating standards.
- **Enhancing Data Governance**: Additional effort is needed to address ongoing barriers to increased data capacity. Although this issue remains challenging, it is critical to the efficient use of the research-oriented data infrastructure across individual and organizations' boundaries of control and ownership.
- **Improving Data Quality**: Promote focus on data quality and increase the quantity and accessibility of electronic health data to improve the efficiency and effectiveness of PCOR; also support core functions and improvements in data interoperability.

- **Balancing Access with Enhancing Privacy and Security**: Spur strategies that enhance privacy and security and inform how research and health care entities can better balance data access with security. Strategies include employing innovative technologies that offer researchers access to data, securely and privately, as well as educating the public about the benefits of making available their anonymous health care data.
- **Disseminating Research Findings:** Improve mechanisms for dissemination of OS-PCORTF-sponsored research so that stakeholders within and outside of HHS can better gauge federal efforts to build data capacity for PCOR.

The stakeholders also noted the need to reduce barriers and costs of obtaining federal data, particularly from the Medicare, Medicaid, and National Death Index programs. The federal stakeholders also discussed concerns about the sustainability plans for OS-PCORTF-funded projects. Some stakeholders suggested that further consideration be given to whether certain functionalities should take priority over others to increase the quantity, quality, and accessibility of data for PCOR. Finally, stakeholders commented on the positive role ASPE plays in promoting cross-agency coordination and collaboration to build data capacity.

Discussion

This formative evaluation found that the 20 portfolio projects evaluated, which were either active or completed between 2012 through 2016, made significant advances toward building data capacity for PCOR, with measurable progress toward implementing the components and enabling all the core research functionalities. Reasonable progress has been made toward the core high-priority research functionalities, with notable progress toward ***Standardized Collection of Standardized Clinical Data***. Both federal and nonfederal stakeholders generally agreed that the 5 functionalities detailed in the Strategic Framework provide sufficient guideposts for building data capacity within PCOR.

The portfolio analysis revealed a need for additional efforts in targeting data linkage. The stakeholder input supported this finding: stakeholders remarked that even though investments have been made to promote and achieve data linkages, more can still be achieved. To make an effective difference in PCOR, clinical and health services researchers require effective means for linking clinical data to data from claims, EHRs, registries, and vital statistics, including but limited to the National Death Index. Both the portfolio and stakeholder analyses suggested that forward-facing efforts should focus on policies, governance, and data quality.

Finally, because of the advances across HHS and through the private sector in developing data capacity, it is challenging to disentangle the singular effect of the OS-PCORTF investments. Further, OS-PCORTF standards efforts may not be broadly adopted because of the rapidly changing pace in the area. More recent initiatives, such as fast health care

interoperability resources (FHIR)-based methods of exchange and interoperability, as well as vendor-initiated efforts, may supersede approaches advanced in earlier portfolio projects.

Recommendations for Future Directions

Based on the findings of the evaluation, HHS may wish to consider the following points (organized by applicable functionality) when developing future priorities and action plans.

Use of Clinical Data

Data quality has been a key part of OS-PCORTF efforts to date, but future work is necessary to focus the community on strategies for addressing data quality, particularly for building data capacity in PCOR. Such efforts could include discussions with stakeholders to define and describe the specific challenges for data quality, then compare and contrast those findings with other efforts that have tackled this issue (past and current). Organizations to consider for outreach include American Health Information Management Association (AHIMA), the American Society for Testing and Materials International, and the International Organization for Standards (ISO).

Additionally, EHRs and other standard data sources may not always capture the data needed for research. An initiative exploring data needed for research, but not typically captured as a byproduct of normal clinical care, might provide insight into new mechanisms for data capture needed to make data maximally useful for researchers.

Standardized Collection of Standardized Clinical Data

Additional effort could include work to demonstrate monetary value of standards or examine standards' impact on the efficiency and effectiveness of PCOR. A key piece is building a body of evidence around cost-effective standards implementation strategies within real-world settings, and effectively disseminating those standards in ways that meet the needs of organizations tasked with contributing standardized data for PCOR. Some of this work is already occurring as part of FDA's Sentinel and PCORnet, both of which have common data models for ease of analyzing data from distributed networks.

Efforts might also include exploring more rigorous policies that insist contractors and grantees use certain standards and prove that they used those standards.

Finally, future attention could include greater support to clinical end users tasked with collecting data for PCOR: e.g., physicians and nurses. Although stakeholders generally agreed that clinical researchers are the key end users, clinical researchers are the appropriate *distal* key end users. However, HHS may want to consider frontline clinicians as the *proximal* users, given that frontline staff are tasked with collecting clinical data for research, and may impact the quality of those data. Investments in improved health IT usability and implementation are well warranted.

Linking Clinical and Other Data for Research

Future work is needed on efforts to link clinical and other data for research both at the individual and aggregate level. Alternatives may include but are not limited to additional work to explore and refine deterministic and probabilistic linkage methods.[5] This work would entail outreach to ethical, legal, technical, and social leaders to inform strategy as to how new approaches and evolving research needs may make necessary a refresh on how HHS considers this sensitive topic. Other institutions have dealt with privacy issues related to linking of sensitive data. It may be worthwhile to have cross-disciplinary discussions with financial institutions and others who have grappled with privacy and security of confidential data.

Collection of PPI

There is great interest in the ability for patient-provided information to contribute PCOR. However, not enough work to date on PPI, or its more generally known term, patient-generated health data (PGHD) has demonstrated the reliability and validity of device-based data. In particular, evidence of benefit and data quality from the consumer wearables market is lacking. Future work could focus on this area and seek to build public-private partnerships around strategies, standards, and even certifications for making PGHD into research-grade data. Additionally, support for initiatives exploring new and better ways to collect patient data, e.g. wireless devices that auto-populate EHRs with structured data could prove valuable.

Use of Enhanced Publicly Funded Data Systems for Research

Future efforts should continue to find ways to link and make more accessible the data in publicly enhanced repositories, particularly Medicare and Medicaid data, possibly through the Chronic Conditions Warehouse, the Healthcare Cost and Utilization Project (HCUP), data from the Veteran's Administration and Indian Health Service, and the NDI. Efforts could include exploring business models that increase access to these valuable data at reduced costs to clinical researchers and/or the general public.

Fostering Better Awareness of Initiatives to Build Data Capacity

In asking nonfederal stakeholders whether they were aware of the projects and products reviewed for this evaluation, a subset indicated they had not heard about the projects and their products. Those stakeholders commented it would be valuable to understand federal efforts for building data capacity. Thus, additional work is needed to foster better awareness of initiatives to build data capacity for PCOR. Efforts could include updating stakeholders about program developments, and disseminating updates when standards and data are available. A potential next step is to identify a curator that aggregates and disseminates updates and points users to existing (and potential) data and vocabulary standards. Other

fields leverage open-source communities continuously by disseminating resources, rules libraries, and mock data for experimentation.

Conclusion

This formative evaluation, completed in mid-2017, documented progress over a slice in time from 2012 through 2016. It demonstrates that data capacity for PCOR has advanced through structuring, linking, and sharing of e-health data across patient groups and repositories throughout the health care ecosystem. Work continues, and will undoubtedly lead to additional improvements. Further, as previously mentioned, it is important to remember that improving data capacity is a process which will never be truly complete. Not only as medicine advances, but technology as well, there will always be new data and data types to structure, collect, link, and analyze.

As the amount of data grows, efforts should focus on continually improving data quality to promote consistency across data sets that ultimately improve the reliability and validity of research results. Those efforts should further the work done to date in promoting effective governance mechanisms among agencies, research entities, and health systems. Furthermore, stakeholders who generate the bulk of e-health data (systems and users) would benefit from greater assistance as to how they can economically and efficiently integrate the standards that the OS-PCORTF project portfolio generates. This task is not easy; stakeholder interviews revealed that these areas are the most difficult challenges in building data capacity for PCOR.

New interagency initiatives just getting under way may enhance or create synergies with the OS-PCORTF aims to build data capacity. For example, the 21st Century Cures Act[6] includes several provisions that could create synergies on data sharing by requiring National Institutes of Health grant recipients to share their data (Section 2014), developing recommendations for a formal policy to enhance rigor and reproducibility of scientific research (Section 2039), accessing sharing and use of health data for research purposes (Section 2063), addressing and expediting interoperability of data among EHRs (Section 4003), and promoting policies ensuring that patients have access to their electronic data (Section 4006). In particular, the Act addresses the implementation of policies and mechanisms for secure and private data sharing of identifiable and sensitive data from federally funded research. The Precision Medicine Initiative (Now the All of Us Initiative) to promote individualized care has developed Privacy and Trust Principles to guide activities regarding governance; transparency; participant engagement and preferences; data sharing, access, and use; and data quality and integrity.[7] Each of these new initiatives hold the promise of enriching or extending existing OS-PCORTF investments.

Investigators conducting PCOR are working in an exciting time—abundant clinical data are available and many research questions need to be answered. This evaluation suggests that

the OS-PCORTF investments are increasing data capacity and helping to create a robust infrastructure for future research.

1. INTRODUCTION

The Patient-Centered Outcomes Research Trust Fund (PCORTF) was established as part of the 2010 Patient Protection and Affordable Care Act (ACA) to help build the data capacity needed to conduct patient-centered outcomes research (PCOR). The PCORTF supports 3 entities and areas of work (see **Exhibit 1**) essential to conducting, disseminating, and building capacity for PCOR. The collective aim of these efforts is to help patients, providers, and caregivers to make more informed health care decisions.

Exhibit 1. Three PCORTF-Supported Entities

Entity	Directive	Percentage of Funds
Patient-Centered Outcomes Research Institute (PCORI)	Conduct research to advance evidence on patient-centered health outcomes	80%
Agency for Healthcare Research and Quality (AHRQ)	Disseminate findings from PCOR, integrate findings into clinical practice, train researchers	16%
HHS Secretary	Build data capacity for clinical comparative effectiveness research (CER)*	4%

* For consistency of terminology in this report, clinical comparative effectiveness research also refers to patient-centered outcomes research.

The Office of the Assistant Secretary for Planning and Evaluation (ASPE) coordinates and administers HHS' efforts under the PCORTF. The charge for the secretary of HHS was as follows:

> *…provide for the coordination of relevant Federal health programs to build data capacity for comparative clinical effectiveness research,[1] including the development and use of clinical registries and health outcomes research networks, in order to develop and maintain a comprehensive, interoperable data network to collect, link, and analyze data on outcomes and effectiveness from multiple sources including electronic health records (EHRs).[2]*

HHS is uniquely positioned to coordinate data capacity-building initiatives across the federal sector because its agencies are involved in developing the data resources, regulating medical products involved in research activities, and using the data produced by various agencies. This report describes an independent evaluation of the PCORTF-supported activities conducted between 2012 and 2016.

[1] For consistency of terminology in this report, comparative effectiveness research also refers to patient-centered outcomes research.

1.1 Need to Increase Data Capacity for Clinical Comparative Effectiveness Research and Patient-Centered Outcomes Research

The evidence generated by PCOR[8,9] helps patients, their caregivers, and providers make informed health care decisions about the benefits and harms of different health care treatments and services. In many cases, little or no scientific evidence is available about the outcomes that result from the health care treatments and services offered to patients. This is particularly true when taking into account factors such as health status, concurrent treatments, age, gender, and other demographics. PCOR studies generate the evidence[10,11] needed to inform these decisions.

The data generated from patients' interactions with the health care delivery system are potentially valuable for PCOR because they can be used to link health care interventions with health outcomes. These data include billing claims, electronic health record data(EHR), and patient registry data. To be used for research, these data must be standardized.so that they are consistent, valid, and linkable across data sets. In addition, researchers need to be able to validate the clinical accuracy of certain data elements (e.g., the validity of being billed for an ICD-10-CM code versus the clinical meaning of that code). These data sets must also be longitudinal and contain information on patient demographics, diagnoses, procedures, and medications to allow researchers to evaluate how particular treatments might benefit similar groups of patients over time. Such rich, large, population-based, real-world data sets are becoming increasingly available to researchers, but substantial work remains to make these data usable for PCOR. Thus, the ACA authorized HHS, and the Secretary charged ASPE with coordinating relevant federal health programs to build data capacity for PCOR.

1.2 HHS'S Leadership for Building Data Capacity for PCOR

The ACA, through the PCORTF, enhanced the data capacity-building work initiated under the American Recovery and Reinvestment Act (ARRA), which expanded researchers' ability to perform PCOR by making significant investments in building capacity for the meaningful use of electronic clinical data. Some PCORTF projects extended work funded under ARRA to begin developing new or enhancing existing data resources and establishing health information technology (IT) standards to leverage EHRs for PCOR.

HHS works with its PCORTF Leadership Council—principals or their designees from the following agencies and offices—to develop priorities for using PCORTF funds to build data capacity for PCOR:

- Assistant Secretary for Planning and Evaluation (ASPE)
- Agency for Healthcare Research and Quality (AHRQ)
- Assistant Secretary for Preparedness and Response (ASPR)

- Centers for Disease Control and Prevention (CDC)
- Centers for Medicare and Medicaid Services (CMS)
- Chief Technology Officer (CTO)
- Food and Drug Administration (FDA)
- Health Resources and Services Administration (HRSA)
- National Institutes of Health (NIH)
- Office of the National Coordinator for Health Information Technology (ONC)

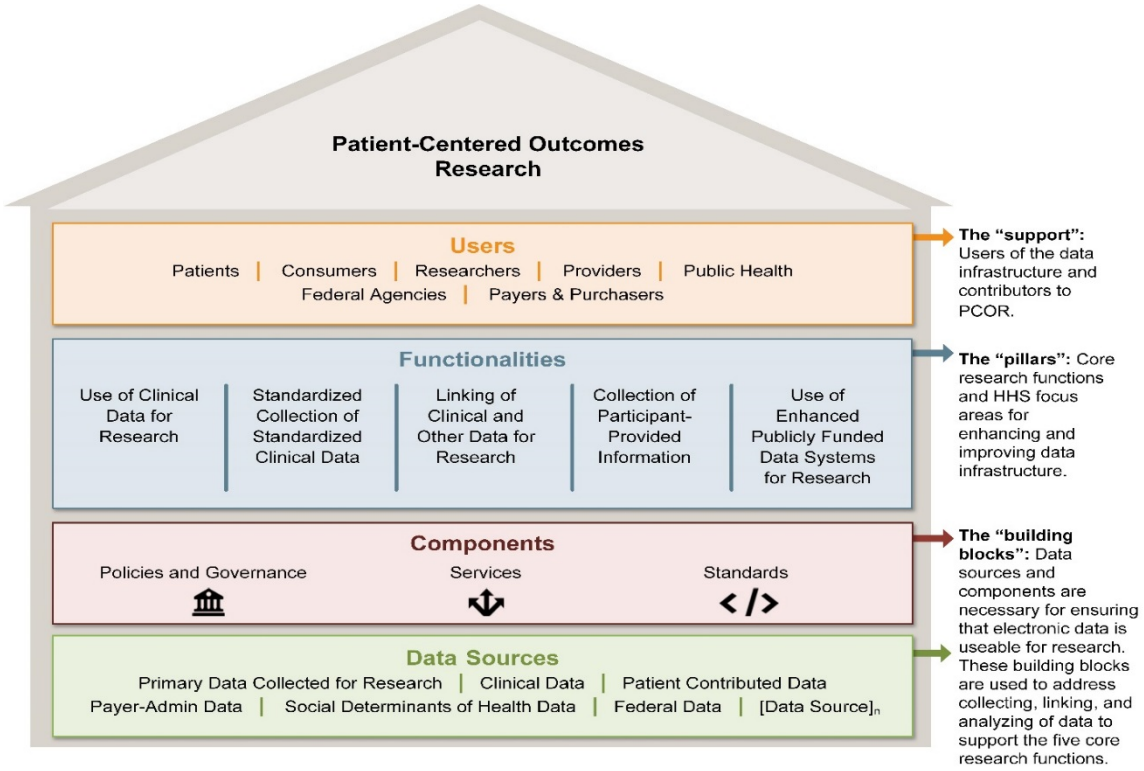
Using the vision for PCOR data infrastructure in **Exhibit 2**, the PCORTF Leadership Council, an HHS Interagency Workgroup, and external researchers endorsed a strategic framework and roadmap to guide PCORTF data infrastructure investments[12] for addressing the core research functions "to collect, link, and analyze data on outcomes and effectiveness from multiple sources including EHRs"[13] for conducting PCOR. Exhibit 2 includes 5 functionalities and four components, defined in Section 1.3, that HHS anticipates will enable the achievement of the overarching PCORTF goal.

The research questions developed for this evaluation were derived from recommendations made in a Government Accounting Office (GAO) report published in March 2015. One of GAO's recommendations was for ASPE to further develop a strategic roadmap to identify and guide PCORTF data capacity-building investments in 2014 and beyond.[14,15] In particular, GAO's final recommendation report states that:

> *… through ASPE, [HHS intends] to further develop the roadmap by specifying milestones with corresponding time frames. HHS will also develop specific performance indicators for its portfolio of data capacity investments. Consistent with our findings and conclusions, HHS's comments also stated that its data capacity investments need to coincide with other key HHS policy initiatives and be responsive to the needs of CER data networks, including PCORI's PCORnet."*[15, p. 26]

In the Roadmap and Strategic Framework, building data capacity for research includes the development and use of clinical registries and health outcomes research networks.[16] Creating more electronic health data using disease registries has the potential to improve the comprehensiveness, completeness, and quantity of data needed to conduct PCOR. The functionalities in Exhibit 2 are the core research functions or capabilities to increase data capacity for PCOR. The components in the Strategic Framework are key infrastructure requirements (in the case of standards and services) and environmental elements (policy and governance) that must be in place to enable the functionalities. Building the requisite component types in Exhibit 2 ensures that electronic data are usable for carrying out PCOR. The definitions for the functionalities and components, as described in the draft *HHS Strategic Roadmap for Building Data Capacity for Clinical Comparative Effectiveness Research*, are listed in Section 1.3.1.[3]

Exhibit 2. HHS Strategic Framework for PCOR Data Infrastructure



1.3 Definitions of Functionalities and Components Used in HHS's PCORTF Strategic Framework

HHS's roadmap and strategic framework were developed to guide decisions about priorities for the portfolio for achieving the portfolio's overarching investment goal: to develop and maintain a comprehensive, interoperable data network to collect, link, and analyze data on outcomes and effectiveness from multiple sources for PCOR. The roadmap and framework are intended to be dynamic so that they can accommodate changes in research needs over time. Components

The four component types that HHS and the PCORTF Leadership Council identified as necessary for building research data capacity are as follows:[14]

1. **Standards** are nationally accepted specifications that have been widely approved and adopted because of market forces, community consensus, or regulatory requirements.
2. **Services** are resources that researchers can use to capture, store, link, analyze, or exchange data or evidence.
3. **Policies** are federal rules or guidelines that need to be established to ensure, for example, that identity checking and security and privacy rules are followed; patient data are protected; and other established standards and services are followed.

4. **Governance structures** support the efficient use of the data infrastructure for research across individual and organizations' boundaries of control and ownership.

To measure progress, incremental steps within each of the four components are referred to as *developmental components*. For example, *Standards for electronic health records to interact with forms* is one step to achieve the "standards" needed. Contained within the four main components are 42 specific developmental components.

1.3.1 Functionalities

HHS's Roadmap and Strategic Framework identified 5 critical functionalities necessary for building data capacity to address the core research areas required for collecting, linking, and analyzing electronic data from different data sources, including EHRs, administrative claims data, registries, vital statistics, biospecimens, surveillance data,[14] and patient-generated health data.

1. ***Use of Clinical Data for Research*** refers to the multiple sources of clinical data available for research (e.g., EHRs, administrative claims, data available via patient portals, registries); and efforts in this area are focused on improving access and interoperability of clinical data for query and analysis.
2. ***Standardized Collection of Standardized Clinical Data*** supports the use of common data elements to enable more effective and efficient linking and aggregating across data sources.
3. ***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.
4. ***Collection of Participant-Provided Information*** (PPI) via new data collection technologies provides means for collecting patient-generated information critical to PCOR.
5. ***Use of Enhanced Publicly Funded Data Systems for Research*** focuses on efforts to leverage current investments in federally available data and infrastructure to inform future infrastructure development.

1.3.2 Milestones

The HHS Roadmap and Strategic Framework identified specific milestones to be achieved to assess progress toward the functionalities. Milestones are based upon developmental components that must be implemented in order for the milestones to be attained. **Exhibit 3** shows the relationship between one functionality, ***Standardized Collection of Standardized Clinical Data***, its milestones, and the developmental components required to achieve the milestones. ***Appendix B*** contains the milestones and developmental components for all of the functionalities.

Exhibit 3. Milestones to Assess Progress Toward a Functionality

Functionality	Milestones	Developmental Components
Standardized Collection of Standardized Clinical Data	1. By 2018, support the development of a set of research CDEs in specific gap areas and support development of a governance structure for CDE harmonization.	▪ CDE—Value set harmonization ▪ Alignment of clinical and research standards. ▪ Governance structure for CDE development and harmonization ▪ CDEs for Precision Medicine Initiative
	2. By 2018, support the development of repositories/portals for CDEs, standards for utilizing CDEs for research, and services to allow researchers to easily utilize standardized components.	▪ Services for contribution and/or harmonization of CDEs ▪ Service(s) that codes elements and questions used to collect data for research to common clinical standards (e.g., SNOMED CT) ▪ CDE representation standards ▪ Standards for forms using CDEs ▪ Standards for EHRs to interact with forms and forms libraries
	3. By 2018, support research and/or crowdsourced methods to determine which standardized collection components and services are most valuable.	▪ Services to allow members of the research community to "voice" value for specific standardized collection components and, in turn, discover value expressed by others
	4. Beginning in 2019, create policies to promote the adoption and use of valuable standardized collection components and services.	▪ Policies for required use of CDEs across research and by EHR and HIE vendors ▪ Align policies and incentives across HHS agencies

Abbreviations: CDE = common data element; EHRs = electronic health records; HHS = Department of Health and Human Services; HIE = health information exchange; SNOMED-CT = Systematized Nomenclature of Medicine-Clinical Terms.

1.4 Evaluation Approach

The evaluation was conducted in 2 parts. To directly assess progress on the project portfolio, the OS-PCORTF portfolio of projects was reviewed to measure progress toward achieving the functionalities and components described in the Strategic Framework. The second part of the evaluation sought comments from stakeholders on the contributions of the OS-PCORTF projects and their products toward building data capacity for conducting PCOR.

The portfolio portion of the evaluation aimed to answer the following overarching research questions:

1. What contributions has the OS-PCORTF portfolio of projects made to strengthening the components (or building blocks) of standards, services, policies, and governance needed to effectively conduct PCOR?

2. To what extent has the OS-PCORTF portfolio of projects enabled the research functionalities (or pillars) outlined in the Strategic Framework to improve data capacity, including use of clinical data for research, standardizing collection of clinical data, linking of clinical data, collection of PPI, and use of publicly funded data systems?

3. Is the Roadmap and Strategic Framework sufficiently comprehensive to build clinical data capacity for PCOR and advance researchers' ability to capture, store, access, link, exchange, and analyze data securely and efficiently?

The stakeholder portion of the evaluation sought to address the 3 overarching research questions, and added four additional aims:

1. Understand key stakeholders' views of the core research functions needed to address gaps in data capacity for PCOR.
2. Evaluate whether and how various key stakeholders used the products from the OS-PCORTF portfolio of projects to enable core research functions.
3. Assess how OS-PCORTF portfolio of projects informed and contributed to key federal stakeholders' research needs, helped to avoid duplication, and fostered coordination across HHS.
4. Understand how OS-PCORTF projects and products are perceived to have addressed the research needs of federally and privately funded research stakeholders.

2. THE PORTFOLIO EVALUATION

2.1 Introduction

The portfolio portion of the evaluation assessed how 26 projects funded between 2012 and 2016 supported the components and functionalities described in the HHS Roadmap and Strategic Framework to expand data capacity for PCOR. For example, did the projects facilitate the conduct of PCOR by improving the accuracy and ease of collecting patient-provided data? The portfolio portion of the evaluation is by its nature formative and assessed only a portion of the overall portfolio (currently 35 projects). The activities conducted within each project were expected to support the implementation standards, services, policies and governance required for conducting PCOR, and ultimately, to make progress toward building data capacity and advancing researchers' ability to capture, store, access, link, exchange, and analyze data securely and efficiently by enabling the 5 functionalities described in the Strategic Framework.

2.1.1 Design of the Portfolio Evaluation

The evaluation design was originally developed by conducting an environmental scan, assembling a technical expert panel (TEP), and conducting interviews to understand the limitations of evaluating a portfolio that was undertaken in a very dynamic space. To reaffirm the evaluation design, the TEP was reconvened to review the measurement strategy again at the start of the evaluation. Panel members included individuals with PCOR, informatics, clinical, and evaluation expertise, and they provided health system and patient perspectives. As shown in ***Appendix C***, the TEP included individuals representing the academic, commercial, and nonprofit sectors.

The TEP proposed using the Capability Maturity Model (CMM) to evaluate the portfolio.[4] The CMM is a process framework initially developed to guide organizations with software development processes by assigning "maturity levels" for various stages of process improvement.[4] The 5 core functionalities identified as critical for building data capacity can be viewed as process goals or capabilities to be enabled. In this evaluation, the CMM was used to assess progress toward enabling the functionalities. Each level of enablement signified a stage of achievement relative to process maturity and served as a foundation for successive levels. A maturity schema was developed to provide the lens through which this evaluation viewed, assessed, and interpreted progress toward enabling the 5 functionalities.

Development of the maturity schema for the evaluation began with the components and milestones ***(Appendix B)*** from the *HHS Strategic Roadmap for Building Data Capacity for Clinical Comparative Effectiveness Research*.[3] RTI identified, and the TEP subsequently approved a set of 4 incremental levels of maturity for the developmental components. A similar set of maturity levels for the functionalities were also identified. The specific maturity

levels and how they are assigned in the during the evaluation process are detailed in Section 2.2.3., The Coding Process.

Exhibit 4 displays these 42 developmental components arranged by increasing level of complexity. It also aligns the components with the functionalities they enable. Using the Strategic Framework as the organizing scheme for Exhibit 4, the developmental components are arranged by component category (standards, services, policies and governance). Exhibit 4 vertically orders the developmental components under each functionality by level of sophistication or complexity, from bottom to top within each component category. In other words, the developmental components listed higher in each component row (i.e., standards, services, and policies/governance) are associated with a higher level of maturity under the CMM model. The developmental components that fall lower in the series require less work to accomplish and are sometimes prerequisites for developmental components higher in the column. Note that this organization reflects "expected" outcomes, and may not reflect the actual way that the developmental components were addressed. After the model in Exhibit 4 was developed, the TEP reviewed it to ensure that it was complete and that the proposed alignment was accurate.

Exhibit 4. Developmental Components Underlying the Functionalities Using a Maturity Schema

	Use of Clinical Data for Research	Standardized Collection of Standardized Clinical Data	Linking of Clinical and Other Data for Research	Collection of Participant-Provided Information	Use of Enhances Publicly Funded Data Systems for Research
Policies and Governance (Increasing Maturity)	• Policy framework for ensuring that structured and unstructured clinical data used in research are "research" grade • Consent standards (RTI assumes these refer to policies) • Policy framework for assessing the reliability of natural language processing • Data use policy for conducting PCOR • Privacy and security policies for querying and accessing clinical data by researchers conducting PCOR	• Mechanisms to align policies and incentives across HHS agencies • Policies for required use of CDEs across research and by EHR and HIE vendors • Governance structure for common data elements development and harmonization	• Policies to enable patient matching/ record linkages to occur under existing laws • Policy framework to enable patient matching/record linkages to occur under existing laws	• Security and privacy policies for handling health data on mobile devices • Policies for incorporating participant-provided information into clinical research	• None
Services (Increasing Maturity)	• Analytical services that support system level results (network-based or population level) • Services for assessing data quality including data completeness, data comprehensiveness and validity • Services for structuring unstructured data (RTI assumes this refers to data that are not being addressed by other initiatives such as ONC's Data Access Framework) • Services to support collection and extraction of data	• Service(s) that code elements and questions used to collect data for research on common clinical standards (e.g., SNOMED CT) • Services to allow members of the research community to "voice" value for specific standardized collection components and, in turn, discover value expressed by others • Services for contribution and/or harmonization of common data elements	• None	• Secure services to collect participant-provided information • Services for patients and their designated proxies (e.g., caregiv-ers) to contribute directly to research databases	• Services to enhance the timeliness of vital statistics data • Services to access and use public data sources
Standards* (Increasing Maturity)	• Metadata standards to represent attributes of data quality including data completeness, data compre-hensiveness and validity • Standards for needed application programming interfaces (APIs) • Standards to answer practice and population level questions using electronic clinical data	• Standards for forms using common data elements • Standards for EHRs to interact with forms and forms libraries • Common data elements representation standards • Common data elements for Precision Medicine • Alignment of clinical and research standards • Common data elements -Value Set Creation and Harmonization	• De-duplication standards and best practices • Standards and methods to privately and securely link and aggregate clinical data to determine eligibility for research studies, according to patient preferences (consent decisions) • Patient matching standards (includes standard attributes for patient matching and standardizing algorithms for patient matching) • Define use cases for patient matching and define currently available methods that work best for each use case	• Standards for personal medical device data (auto-reported information) • Standards for integrating participant-provided information with electronic health record data • Standards for participant-provided information (e.g., mobile devices, wearables etc) • Standards for collection of participant-provided information	• Safety-net clinical registry data • Cancer registry data

* Note: Standards are of mixed types, with largest portion being data standards
Blank spaces in columns do not indicate that anything is missing or lacking; but indicate that area is not relevant to the enhancement of that particular functionality

* Note: Standards are of mixed types, with largest portion being data standards. ** The TEP did not identify components for this area.

2.2 Portfolio Analysis Methods

2.2.1 Document Acquisition

For the project portfolio evaluation, documents provided by ASPE were the primary means by which the projects were evaluated. ASPE staff provided RTI with documentation for 30 OS-PCORTF projects awarded from FY2012 through FY2016. These projects varied in funding amount, size (e.g., number of goals and/or activities), duration, and scope. Specifically, statements of work were received from interagency agreements, progress reports, written communications between ASPE and the awarded agency, and deliverables (including manuscripts and presentations) via a secure FTP site. The documentation varied among the 30 projects, depending on the project's period of performance.

Projects Not Included in the Evaluation

Documentation was provided for 30 projects. A minimum documentation threshold was established for including a project in the evaluation: a statement of work and a minimum of 3 progress reports. Because of this threshold, four projects early in their period of performance were not included when the evaluation concluded:

- Source Data Capture from EHRs: Using Standardized Clinical Research Data (16-004)
- Use of ADAPTABLE Trial to Strengthen Methods to Collect and Integrate Patient-reported Information with Other Data Sets (16-005)
- Standardization of Querying and Data Quality Metrics and Characteristics of Electronic Health Data (16-006)
- Harmonization of Clinical Data Element Definitions for Outcomes Measures in Registries (16-007)

One project, Maintenance and Support of the Chronic Conditions Warehouse for CER (12-003) was merged into another project, Maintenance and Support of the Chronic Conditions Warehouse for Comparative Effectiveness Research (13–002), and was not separately evaluated.

Finally, 3 projects conducted early in the evaluation period were not targeted toward implementation of developmental components and, therefore, could not materially influence the evaluation findings:

- Multi-Payer Claims Database Beta Test (12-004)
- CER Inventory (12-008)
- Strategic Opportunities for Building Data Infrastructure for CER (13-008)

Thus, 22 projects remained to be fully evaluated. This number was reduced to 20 when 2 sets of projects were merged (creating 2 projects from the initial four). Upon receipt of project documentation, all project documentation was imported into NVivo qualitative

analysis databases (Version 11, www.qsrinternational.com) to be distributed to the coding team for review and coding.

2.2.2 Document Review

Summative projects were designated as those with periods of performance ending before October 1, 2016 as. Those that were still in progress at the time of final document review, and that met the minimum documentation threshold, were designated as formative projects.

Table 1 presents the list of the 30 projects for which documentation was received; this table identifies the subset of 26 projects for which documentation was reviewed and coded, as well as the 20 projects that ultimately included in the analysis. Also included are the projects' awarding agencies, periods of performance, and formative versus summative designation.

Table 1. Projects Included in Document Review and Coding

REMIS Number	Project Title	Agency	Period of Performance	Summative (S) vs Formative (F) Project	Included in Document Review	Included in Analysis
12-003	Maintenance and Support of the Chronic Condition Warehouse for CER	CMS	07/11/12- 06/30/13	S	✓	
12-004	Multi-Payer Claims Database Beta Test	CMS	9/24/12–9/15/13	S	✓	
12-007 and 13-004	Infrastructure for Use of EHRs in Comparative Effectiveness Research Data Infrastructure for Use in EHRs in Comparative Effectiveness Research (CER)	NIH/ National Library of Medicine	6/1/12–9/30/13 7/20/13–9/30/14	S S	✓ ✓	✓*
12-007 and 13-004	Infrastructure for Use of EHRs in Comparative Effectiveness Research Development of Data Infrastructure for Use in EHRs in Comparative Effectiveness Research (CER): Development of Meaningful Use Standards for CER Data Elements	ONC	8/29/12–9/30/13 7/12/13–9/30/14	S S	✓ ✓	✓*
12-008	CER Inventory	ASPE	9/30/12–9/29/13	S	✓	
13-002	Maintenance and Support of the Chronic Condition Warehouse for Comparative Effectiveness Research	CMS	9/30/13–9/29/14	S	✓	✓
13-003	Creating the Foundational Blocks for the Learning Health Care System: Structured Data Capture	ONC	1/27/14–9/30/17	F	✓	✓

(continued)

Table 1. Projects Included in Document Review and Coding (continued)

REMIS Number	Project Title	Agency	Period of Performance	Summative (S) vs Formative (F) Project	Included in Document Review	Included in Analysis
13-006	Expanding Data Collection Infrastructure of the National Program of Cancer Registries for Comparative Effectiveness Research	CDC	4/8/13–8/31/15	S	✓	✓
13-008	Strategic Opportunities for Building Data Infrastructure for Comparative Effectiveness Research	ONC	2/15/13–6/15/15	S	✓	
14-009	Strengthening and Expanding Community Health Applied Research Network (CHARN) Registry to Conduct Patient-Centered Outcomes Research	HRSA	8/4/14–8/30/15	S	✓	✓
14-012	Creating the Foundational Blocks for the Learning Health Care System: Data Access Standards for Electronic Health Records (EHRs)	ONC	11/1/13–9/30/16	S	✓	✓
15-003	Improving Beneficiary Access to Health Information "Blue Button" to Enable a 'Data-as Service' Platform	CMS	10/1/14–6/30/16	S	✓	✓
15-012	Improving the Mortality Data Infrastructure for Patient-Centered Outcomes	CDC	4/3/15– 9/22/17	F	✓	✓
15-013	Utilizing Data from Various Data Partners in a Distributed Manner	FDA	7/15/15–6/30/18	F	✓	✓
15-014	Cross-Network Directory Service	FDA	7/15/15–6/30/18	F	✓	✓
15-015	Collection of Patient-Provided Information through a Mobile Device Application for Use in Comparative Effectiveness and Drug Safety Research	FDA	7/15/15–6/30/18	F	✓	✓
15-016	Conceptualizing a Data Infrastructure for the Capture and Use of Patient-Generated Health Data	ONC	6/16/15–6/30/18	F	✓	✓
15-018	Security and Privacy Standards for Patient Matching, Linking and Aggregation	ONC	6/16/15–9/30/18	F	✓	✓
15-019	PCOR: Privacy and Security Blueprint, Legal Analysis and Ethics Framework for Data Use, & Use of Technology for Privacy	ONC	6/16/15–9/30/18	F	✓	✓
15-019	PCOR: Privacy and Security Blueprint, Legal Analysis and Ethics Framework for Data Use, & Use of Technology for Privacy	Developmental component	7/10/15–9/30/17	F	✓	✓
16-002	Improving Beneficiary Access to their Health Information through an Enhanced Blue Button Service	CMS	4/30/16–4/29/18	F	✓	✓

(continued)

Table 1. Projects Included in Document Review and Coding (continued)

REMIS Number	Project Title	Agency	Period of Performance	Summative (S) vs Formative (F) Project	Included in Document Review	Included in Analysis
16-003	Development of a Natural Language Processing (NLP) Web Service for Public Health Use	CDC	6/01/16–5/31/18	F	✓	✓
16-003	Development of a Natural Language Processing (NLP) Web Service for Public Health Use	FDA	4/01/16–9/30/21	F	✓	✓
16-004	Source Data Capture from EHRs: Using Standardized Clinical Research Data	FDA	8/25/16–9/30/18	F		
16-005	Use of ADAPTABLE Trial to Strengthen Methods to Collect and Integrate Patient-reported Information with other Data Sets	NIH	8/31/16–9/30/17	F		
16-006	Standardization and Querying and Data Quality Metrics and Characteristics of Electronic Health Data	FDA	8/20/16–9/30/18	F		
16-007	Harmonization of Clinical Data Element Definitions for Outcomes Measures in Registries	AHRQ	4/29/16–4/28/16	F		
16-008	Creation of LOINC Equivalence Classes	NIH	9/23/16–TBD	F	✓	✓
TOTAL					26	20

Preparing to Code Project Documentation

A team of individuals with health informatics expertise coded project documentation. To prepare for the formal document review and coding process, a training session was conducted with the coders, covering the evaluation design, coding schemes, database structure, review processes and the coding process. In a pilot activity conducted after training, all coders independently reviewed and coded the same documentation for one project (Creating the Foundational Blocks for the Learning Health Care System: Structured Data Capture). After conducting the pilot, the full team convened to compare their coding efforts and discuss their reasoning for applying the codes they chose to ensure that all analysts had a shared understanding of code definitions and could apply them in a consistent, replicable manner. To ensure consistency in coding, standard operating procedures (SOPs) (see ***Appendix D***) were developed to define database management and coding workflows specific to the project. All coding decisions were documented for the coding team's reference.

2.2.3 The Coding Process

After achieving an acceptable consistency across coders, the project-specific coding process began using NVivo, a qualitative data analysis software that facilitates coding of textual data from documents and interviews. Statements of work and available progress notes for each project were uploaded into individual project-specific NVivo databases that were prepopulated with the project-specific coding scheme. Coders independently analyzed and coded the documentation for their assigned projects. Coding assignments were released in small batches. The team used the time between batches to discuss high-level findings and lessons learned and to clarify definitions and processes as needed.

In some cases, the coder, the review committee, or both parties determined that additional project documentation or clarifying information was needed from ASPE or the federal agency project lead to determine or finalize coding the status of a project's activities or to map to the developmental components. Gaps in information were documented in the NVivo database and the missing information was requested from the project leads. Upon receiving clarifying information from the project leads, the coding team imported the responses into the NVivo database so that the evidence supporting the selected status or mapping could be coded. Project documentation was also supplemented by conducting project-specific literature searches to glean additional information about the projects and their outputs. Specifically, a literature search was conducted using the internal project unique identifier [Resources Management Information Systems (REMIS) numbers], project titles, contract numbers, and keywords against 6 resources: (1) PubMed, (2) PubMed Central, (3) Google, (4) Google Scholar, (5) MEDLINE, and (6) PCORI Web site. The resulting references (e.g., manuscripts, conference presentation slides, white papers) were imported into the corresponding project's NVivo database for the coder to review and code as appropriate. Finally, ASPE provided drafts of 2 documents compiled by a third party[17,18] to corroborate project information. These drafts were not directly coded in the NVivo database, but were useful for confirming information found in the project documentation.

Coders reviewed the statement of work for each project, coding selected text from the background, primary goals, and activities listed in the statement of work. They mapped each project activity to developmental components that the activity helped implement. **Exhibit 5** illustrates the coding process. Activities are work units within a project to achieve the project's intended goals (e.g., create data sets, expand EHR reporting capacity). Activities equate most directly to the tasks for a project; however, for an activity to be listed as such, it must be operationally possible to assess progress toward completing the activity. When coders encountered project activities that did not map to any of the developmental components in this schema, they applied one of 2 codes: "Does not map to existing developmental components- administrative" or "Does not map to existing developmental components- possible new developmental component". The "administrative" tag indicated that the activity has an administrative or operational role in the project, and therefore, is

not intended to support a developmental component. After recording goals, activities and mappings to developmental components in the NVivo database, reviewers assigned an "activity status" for each activity in the NVivo database.

Table 2 illustrates the possible activity statuses for each activity.

Upon completing the initial NVivo coding of a project's documentation, the coder met with 2 senior members of the team who served as a review committee. The committee and coder reviewed the coding together and documented any questions or issues (e.g., inadequate information in documentation to determine the status of an activity). The coder then revised the coding as needed to reflect decisions made during the review committee meeting.

Exhibit 5. Mapping Activities to Developmental Components

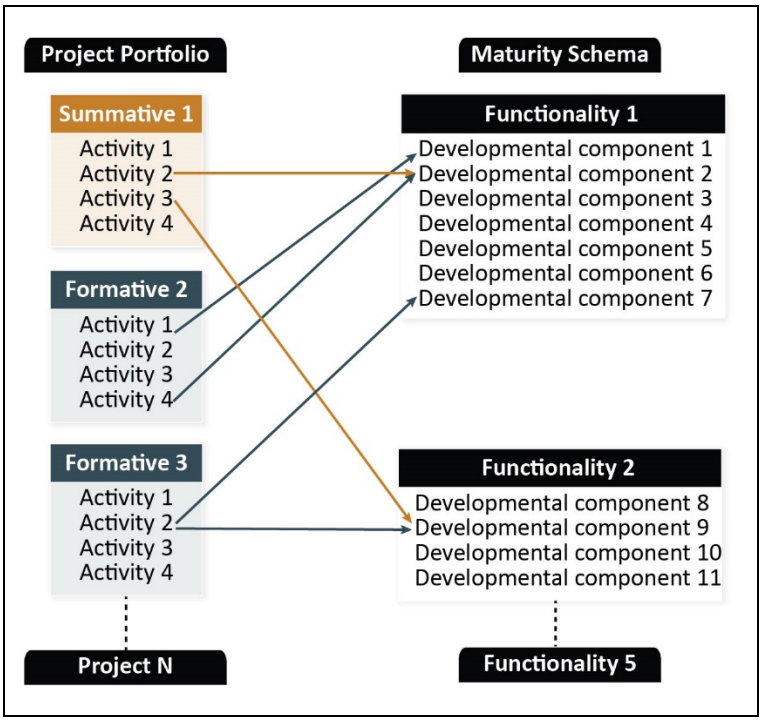


Table 2. Coding Scheme for Categorizing Progress on Activities Contributing to Achievement of Each Developmental Component

Activity Status	Code Definition
Not Started	This developmental component was mentioned in the project's goals, objectives, or plans, but could not document evidence that work had begun.
Partially Achieved	Documentation is available that work toward this developmental component is currently under way.
Fully Achieved	Documentation is available that shows project work toward this developmental component was completed fully, whether the project's period of performance is ongoing or the project has ended.

After coding all project-specific documentation, including supplemental information received from project leads and the literature, the coders refined and finalized their coding by resolving questions that had been documented earlier in the process. They also reviewed project activities that were not mapped to any existing developmental components or that

had been assigned a status of "Partially Achieved." At this point, coders met with the review committee once again to close out the review of each project

Coding Developmental Components and Functionalities

Once all of the project activities had been reviewed, an implementation code for each developmental component was calculated according to the coding scheme in **Table 3.**

Table 3. Coding Scheme for Assessing the Level of Implementation for Each Developmental Component

Code for developmental component	Code Definition
No Implementation	No activities contributed to the developmental component, or activities contributing to the developmental component are in planning and have not started on a majority* of relevant projects (i.e., *Not Started* code in Table 2).
Early Implementation	Activities contributing to developmental component are under way on a majority* of relevant projects (i.e., *Partially Achieved* code in Table 2).
Mid-to-Late Implementation	Activities contributing developmental component are complete or nearing completion on a majority* of relevant projects (i.e., *Fully Achieved* code in Table 2).
Full Implementation	Activities contributing to developmental component are complete or nearing completion on ALL relevant projects, ** and the focus has shifted to optimization (i.e., *Fully Achieved* code in Table 2).

Note: *When ties occurred between statuses, because there was no majority (i.e., 2 contributing activities were coded as "partially achieved" and 2 contributing activities were coded as "fully achieved"), the lower status was applied. **If only a single project was relevant, the lower "Mid-to-Late Implementation" status was applied in lieu of the "Full Implementation" status.

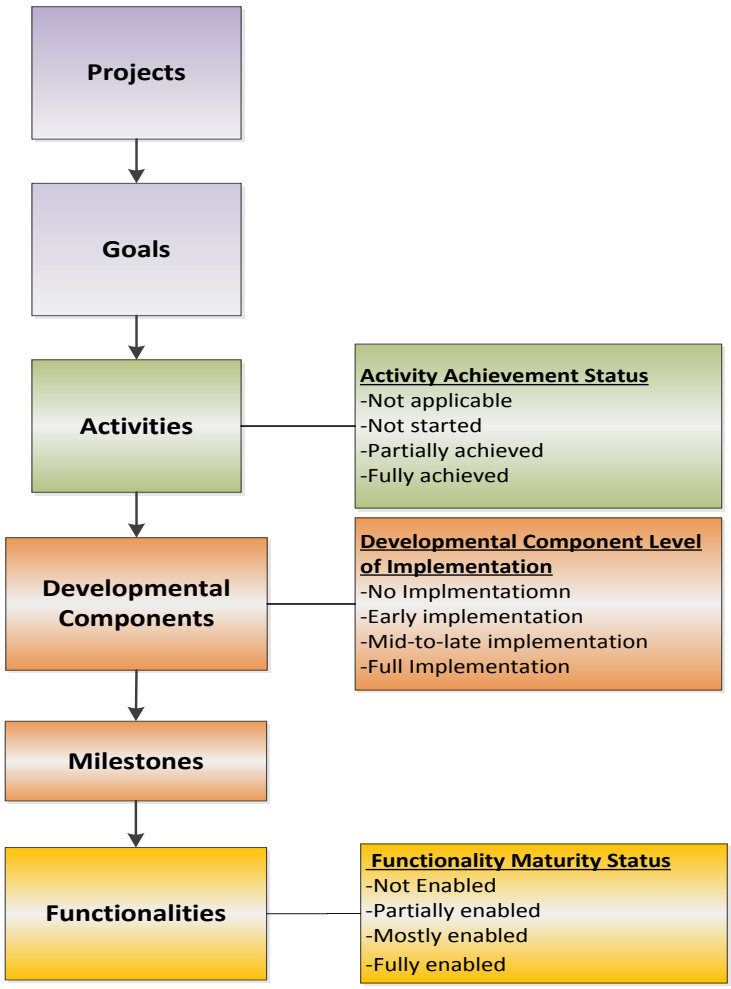
To determine the progress made by the portfolio toward enabling the functionalities, the implementation codes assigned to the developmental components in Table 3 were used to assess the extent to which the functionalities they support were enabled. (see **Table 4**).

Table 4. Determining How Well Functionalities Were Enabled by Developmental Components

Code for Functionality	Code Definition
Not Enabled by Portfolio	Majority of applicable Developmental components coded as 'No Implementation' in Developmental Component Maturity Coding Scheme (Table 3). This code would also be applied if no projects in the portfolio deal with Developmental components relevant to a functionality.
Partially Enabled by Portfolio	Majority of applicable Developmental components coded as 'Early Implementation' in Developmental Component Maturity Coding Scheme (Table 3).
Mostly Enabled by Portfolio	Majority of applicable Developmental components coded as 'Mid-to-Late Implementation' in Developmental Component Maturity Coding Scheme (Table 3).
Fully Enabled by Portfolio	Majority of applicable Developmental components coded as 'Optimizing' in Developmental Component Maturity Coding Scheme (Table 3).

Exhibit 6 provides a high-level graphic summary of the steps in the coding process, starting with project reviews to assess project goals, activities, and activity achievement statuses, through coding of the developmental components and ultimately functionalities.

Exhibit 6. Portfolio Evaluation Process



2.3 Project Portfolio Evaluation Results

This section describes the results of the mixed-methods formative evaluation of 20 projects begun between 2012 and 2016. Results are provided at the project, component, and functionality levels, and the limitations of this evaluation approach are discussed.

2.3.1 Project Analysis Results

Table 5 lists projects included in the document review by type, with the number of number of goals and activities identified, the number of developmental components each project mapped to. As shown in the last column of the table, four projects did not contribute to any of the developmental components, and were excluded from the analysis.

Table 5. Projects Included in the Portfolio Analysis by Type and Number of Goals, Activities, and Developmental Components Applied

REMIS Num-ber	Project Title	Type	Number of Goals	Number of Activities	Developmental Components Mapped to By Project Activities
12-003	Maintenance and Support of the Chronic Condition Warehouse for CER	Summative	1	1	N/A
12-004	Multi-Payer Claims Database Beta Test	Summative	1	3	N/A
12-007/ 13-004 NIH/ NLM	Infrastructure for Use of EHRs in Comparative Effectiveness Research Data Infrastructure for Use in EHRs in Comparative Effectiveness Research (CER)	Summative	3	7	11
12-007/ 13-004 ONC	Infrastructure for Use of EHRs in Comparative Effectiveness Research Development of Data Infrastructure for Use in EHRs in Comparative Effectiveness Research (CER): Development of Meaningful Use Standards for CER Data Elements	Summative	2	5	8
12-008	CER Inventory	Summative	1	2	N/A
13-002	Maintenance and Support of the Chronic Condition Warehouse for Comparative Effectiveness Research	Summative	1	3	1
13-003	Creating the Foundational Blocks for the Learning Health Care System: Structured Data Capture	Formative	1	4	6
13-006	Expanding Data Collection Infrastructure of the National Program of Cancer Registries for Comparative Effectiveness Research	Summative	1	2	1
13-008	Strategic Opportunities for Building Data Infrastructure for CER	Summative	N/A	N/A	N/A
14-009	Strengthening and Expanding Community Health Applied Research Network (CHARN) Registry to Conduct Patient-Centered Outcomes Research	Summative	1	3	3
14-012	Creating the Foundational Blocks for the Learning Health Care System: Data Access Standards for Electronic Health Records (EHRs)	Summative	3	9	2
15-003	Improving Beneficiary Access to Health Information "Blue Button" to Enable a 'Data-as Service' Platform	Summative	1	5	4
15-012	Improving the Mortality Data Infrastructure for Patient-Centered Outcomes.	Formative	1	3	4
15-013	Utilizing Data from Various Data Partners in a Distributed Manner	Formative	1	4	1
15-014	Cross-Network Directory Service	Formative	1	3	2

(continued)

Table 5. Projects Included in the Portfolio Analysis by Type and Number of Goals, Activities, and Developmental Components Applied (continued)

REMIS Num-ber	Project Title	Type	Number of Goals	Number of Activities	Developmental Components Mapped to By Project Activities
15-015	Collection of Patient-Provided Information through a Mobile Device Application for Use in Comparative Effectiveness and Drug Safety Research	Formative	1	4	2
15-016	Conceptualizing a Data Infrastructure for the Capture and Use of Patient-Generated Health Data	Formative	1	2	3
15-018	Security and Privacy Standards for Patient Matching, Linking and Aggregation	Formative	5	13	7
15-019	PCOR: Privacy and Security Blueprint, Legal Analysis and Ethics Framework for Data Use, & Use of Technology for Privacy	Formative	1	1	1
15-019	PCOR: Privacy and Security Blueprint, Legal Analysis and Ethics Framework for Data Use, & Use of Technology for Privacy	Formative	1	4	3
16-002	Improving Beneficiary Access to their Health Information through an Enhanced Blue Button Service	Formative	1	5	5
16-003	Development of a Natural Language Processing (NLP) Web Service for Public Health Use	Formative	1	1	3
16-003	Development of a Natural Language Processing (NLP) Web Service for Public Health Use	Formative	1	1	3
16-008	Creation of LOINC Equivalence Classes	Formative	1	3	3

N/A = not applicable.

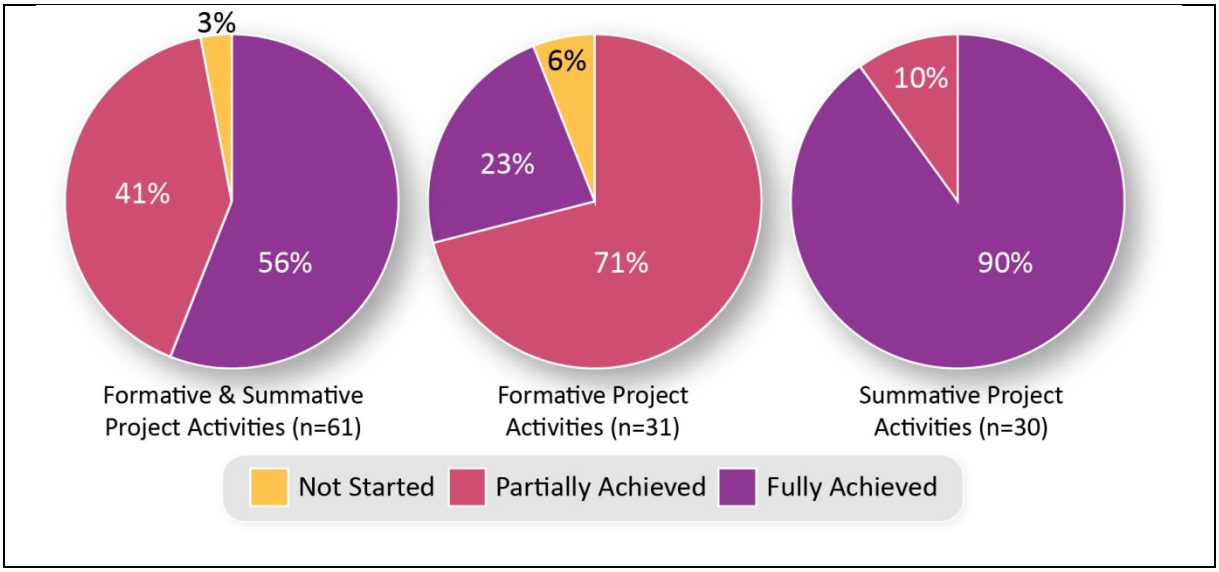
Thirteen projects were formative (ongoing) and 7 were summative (complete). Most projects (n=16) had one goal, one project had 2 goals, 2 projects had 3 goals, and another project had 5 goals. On average, each project had four activities across the goals, and four applicable developmental components. Projects were not equal in terms of funding, size, or scope, which may account for the variability in the number of goals, activities, and developmental components across the projects.

For full details of the goals, activities, activity achievement status, and applicable developmental components, see ***Appendix E*****.** A detailed description of all the projects in the portfolio (35 at the time this evaluation was submitted) can be found in ***Appendix F***.

Exhibit 7 shows the percentage of activities by achievement status for formative, summative, and all projects. Across all projects included in the analysis, more than half (56%) of the activities were fully achieved, less than half (41%) were partially achieved, and less than 5 percent were not started. Among the formative projects, less than one-fourth (23%) were fully achieved, with more than two-thirds (71%) partially achieved, and

less than 10 percent not yet started. For the summative projects, most activities (90%) were fully achieved, with the remaining activities partially achieved.

Exhibit 7. Percentage of Activities Across the Portfolio by Achievement Status



The partially achieved activities in the summative projects are attributable to one project, "Data Infrastructure for Use of EHRs in Comparative Effectiveness Research (CER)." In that project, activities related to the creation and contribution of common data elements and electronic case review forms (eCRFs) were only partially complete at the end of the project, and it was noted that the activities would continue in the context of other projects and initiatives. Thus, all but one of the summative projects fully achieved all their activities during the period of performance. Although the formative projects were still in progress, most of their activities were at least partially achieved; only a small percentage had not yet been started.

2.3.2 Results Supporting Implementation of Developmental Components and Achievement of Milestones

This section addresses findings related to the 42 developmental components. **Exhibit 8** shows the 33 developmental components with the number and achievement status of the activities mapped to them. Exhibit 8 does not include the 9 developmental components with no project activities mapping to them. The developmental components, *Services to support collection and extraction of data* and *Standards for needed application programming interfaces (APIs,)* had the greatest number of activities (n=12 and 13, respectively). In addition, most activities for these 2 developmental components are fully achieved.

Exhibit 8. Number of Project Activities Achievement Status for Developmental Components

Across the 20 projects, activities were mapped to 33 of the 42 existing developmental components. **Exhibit 9** displays the 33 existing developmental components that had activities mapping to them and adds the 2 "Does not map developmental components" with the counts of formative and summative projects attributed to each developmental component. The developmental component *Services to support collection and extraction of data* had the most projects mapped to it.

Level of Implementation Attained by the Developmental Components

Once all projects were reviewed, activities supporting each developmental component were tallied so that the developmental components could be scored and assigned implementation levels. **Exhibit 10** shows the implementation level attained for all of the 33 developmental components that had project activities mapped to them (at least one project).

Nine of the 42 developmental components were not supported by any of the project activities included as part of the evaluated portfolio. **Table 6** shows these developmental components. The majority are related to the development of policies. The remainder of the developmental components (33) are supported by one or more project activities. **Exhibit 10** shows the 33 developmental components that were supported by project activities and their implementation level. None of the developmental components were in the planning phase. More than half (n=19) were in early implementation. Eleven were in mid-to-late implementation, and 3 were full implementation. The developmental components and their level of implementation can also be shown aligned with the milestones they support. ***Appendix B*** displays the milestones, their associated developmental components, and the level of achievement for each developmental component for each of the functionalities.

2.3.3 Results Supporting Enablement of Functionalities (Scoring)

Exhibit 11 shows the number of projects supporting each of the 5 core functionalities. Most formative projects (n=11) and many summative projects (n=4) were relevant to ***Use of Clinical Data for Research***. None of the summative projects were relevant to ***Linking Clinical and Other Data for Research***.

Exhibit 9. Number of Formative and Summative Projects with Activities Mapped to Developmental Components

Functionality	Development Component	Number of Projects
Use of Clinical Data for Research	01 – Privacy and security policies for querying and accessing clinical data by researchers conducting PCOR	● ●
	04 – Consent standards	●
	06 – Services to support collection and extraction of data	● ● ● ● ● ○ ○ ○
	07 – Services for structuring unstructured data	● ● ○
	09 – Analytical services that support system-level results (network-based or population level)	● ●
	11 – Standards to answer practice and population-level questions using electronic clinical data	● ● ○
	12 – Standards for needed application programming interfaces (APIs)	● ● ○
Standardized Collection of Standardized Clinical Data	13 – Governance structure for contribution and/or harmonization of common data elements (CDEs)	○
	14 – Policies for required use of CDEs across research and by EHR and health information exchange (HIE) vendors	○ ○
	15 – Mechanisms to align policies and incentives across Health and Human Services (HHS) agencies	● ○ ○
	16 – Services for contribution and-or harmonization of CDEs	○
	17 – Services to allow members of the research community to "voice" value for specific standardized collection components and, in turn, discover value expressed by others	●
	18 – Service(s) that code elements and questions used to collect data for research to common clinical standards (e.g., SNOMED CT)	○
	19 – CDE—value set creation and harmonization	● ○ ○
	20 – Alignment of clinical and research standards	● ○ ○
	22 – CDE representation standards	● ● ● ○
	23 – Standards for EHRs to interact with forms and forms libraries	● ○ ○
	24 – Standards for forms using CDEs	● ○ ○
Linkage of Clinical and Other Data for Research	26 – Policies to enable patient matching - record linkages to occur under existing laws	●
	27 – Define use cases for patient matching and define currently available methods that work best for each use case	●
	28 – Patient matching standards (includes standard attributes for patient matching and standardizing algorithms for patient matching)	● ●
	29 – Standards and methods to privately and securely link and aggregate clinical data to determine eligibility for research studies, according to patient preferences (consent decisions)	●
Collection of Participant Provided Data (PPI)	31 – Policies for incorporating PPI into clinical research	●
	33 – Services for patients and their designated proxies (e.g., caregivers) to contribute directly to research databasesatching)	● ●
	34 – Secure services to collect PPI	● ● ○
	35 – Standards for collection of PPI	● ○
	36 – Standards for PPI	●
	37 – Standards for integrating PPI with electronic health data	●
	38 – Standards for personal medical device data	●
Use of Enhanced Publicly Funded Data Systems for Research	39 – Services to access and use public data sources	● ● ● ○
	40 – Services to enhance the timeliness of vital statistics data	●
	41 – Cancer registry data	● ○
	42 – Safety-net clinical registry data	○
	43 – Does not map to existing DCs - Administrative	● ● ● ● ● ● ○ ○
	44 – Does not map to existing DCs - Possible DC	● ● ●

Notes. ● Formative Projects ○ Summative Projects

Exhibit 10. Level of Implementation by Developmental Component

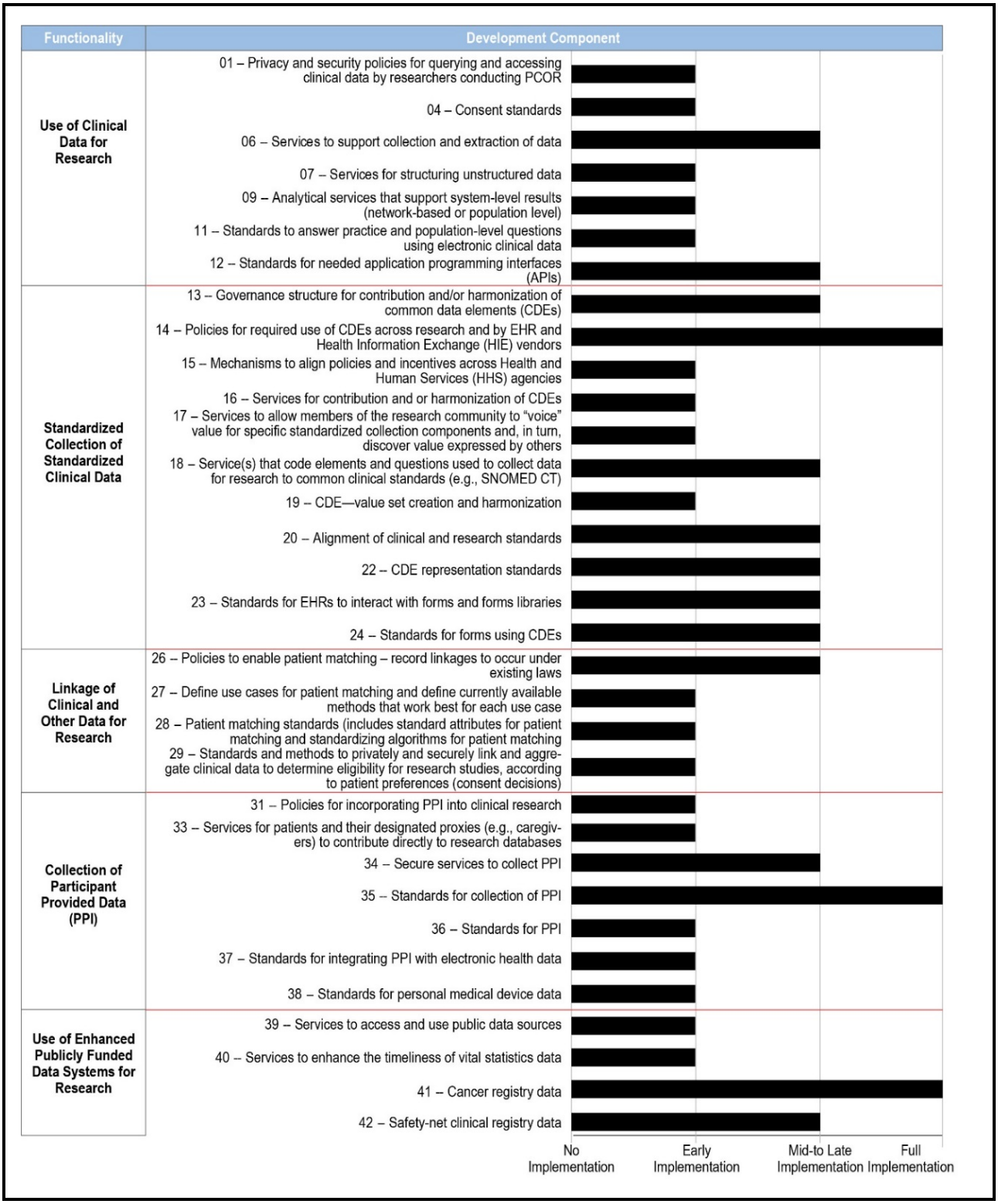


Table 6. Milestone-Building Developmental Components Not Supported by Project Activities

	Unsupported Development Components
Policies and Governance	Security and privacy policies for handling health data on mobile devices
	Policy framework to enable patient matching/record linkages to occur under existing laws
	Data use policy for conducting PCOR
	Policy framework for assessing the reliability of natural language processing
	Policy framework for ensuring that structured and unstructured clinical data used in research are "research" grade
Services	Services for assessing data quality including data completeness, data comprehensiveness, and validity
Standards	Metadata standards to represent attributes of data quality including data completeness, data comprehensiveness, and validity
	Common data elements for precision medicine
	De-duplication standards and best practices

Exhibit 11. Number of Projects Supporting Each Core Functionality

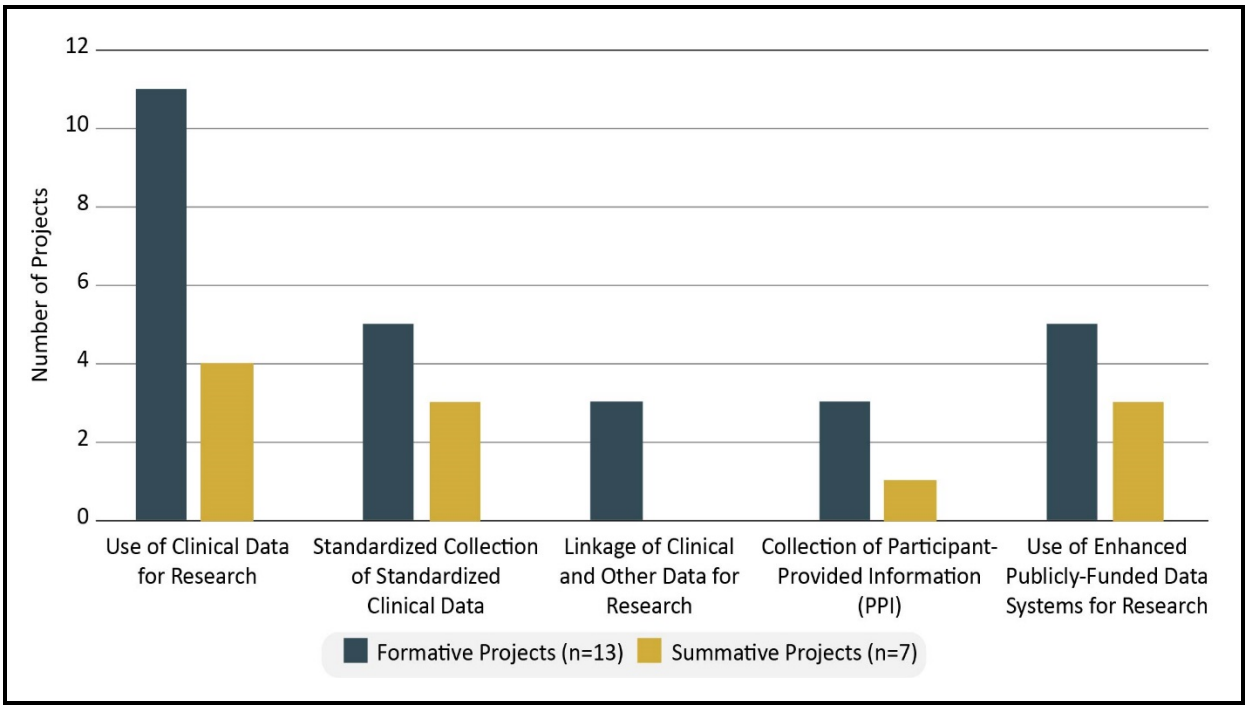


Exhibit 12 shows the titles of the specific projects that contributed to the enablement of the 5 functionalities**.**

Exhibit 12. Projects Supporting Enablement of the Functionalities

Project	Use of Clinical Data for Research	Standardized Collection of Standardized Clinical Data	Linking Clinical and Other Data for Research	Collection of Participant Provided Information (PPI)	Use of Enhanced Publicly Funded Data Systems for Research
12-007/13-004-NLM - Infrastructure for Use of EHRs in CER + Meaningful Use Standards for CER data		X			
12-007+13-004-ONC - Infrastructure for Use of EHRs in CER + Meaningful Use Standards for CER data	X	X			
13-002-CMS - Maintenance and Support of the CCW for CER					X
13-003 - Structured Data Capture	X	X	X		X
13-006 - Expanding Data Collection Infrastructure of National Program of Cancer Registries for CER					X
14-009 - Strengthening & Expanding CHARN Registry for PCOR	X	X			X
14-012 - Data Access Standards for EHRs	X				
15-003 - Improving Beneficiary Access to Health Information to Enable 'Data as Service Platform	X			X	
15-012 - Improving Mortality Infrastructure for PCOs	X	X	X		X
15-013 - Utilizing Data from Partners in a Distributed Manner	X				
15-014 - Cross-Network Directory Service	X				X
15-015 - Collection of PPI through Mobile Device App for Use in CER & Drug Safety Research				X	
15-016 - Data Infrastructure for Capture & Use of PGHD				X	
15-018 - Security & Privacy Standards for Patient Matching, Linking, & Aggregation	X	X	X		
15-019-Cdevelopmental component - Privacy & Security Blueprint	X	X			

(continued)

Exhibit 12. Projects Supporting Enablement of the Functionalities (continued)

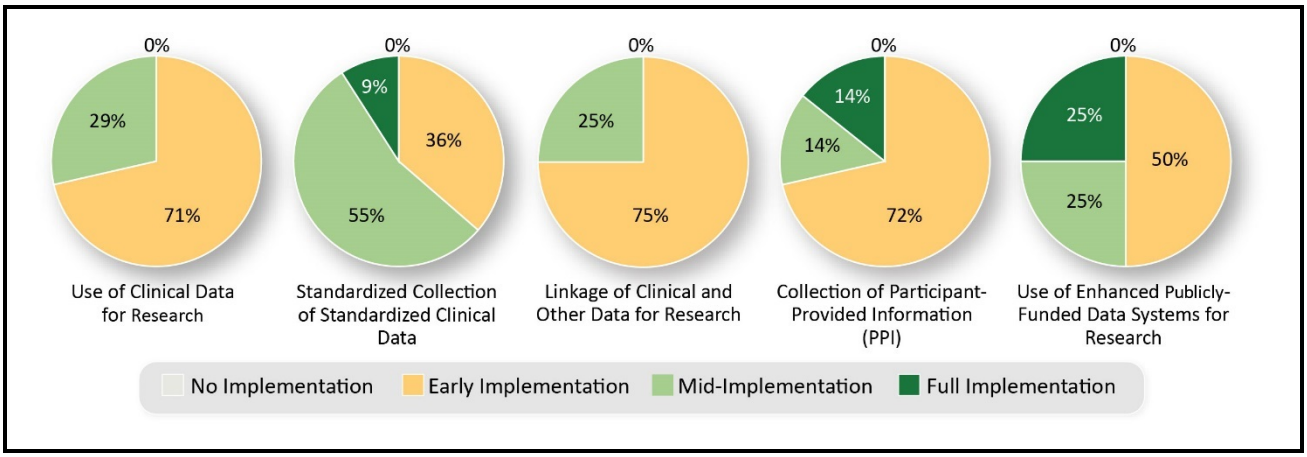
Project	Use of Clinical Data for Research	Standardized Collection of Standardized Clinical Data	Linking Clinical and Other Data for Research	Collection of Participant Provided Information (PPI)	Use of Enhanced Publicly Funded Data Systems for Research
15-019-ONC - Privacy & Security Blueprint	X				
16-002 - Improving Beneficiary Access to Health Information through Enhanced BlueButton	X			X	
16-003-Cdevelopmental component - Development of NLP Web Service for Public Health	X				X
16-003-FDA - Development of NLP Web Service for Public Health	X				X
16-008 - Precision Medicine Informatics	X	X			

Formative Projects Summative Projects

Exhibit 13 shows the percentage of developmental components by their level of implementation within each functionality. Notable findings are as follows:

Use of Clinical Data for Research has no developmental components that are fully implemented, and the majority of developmental components (71%) are partially implemented. ***Standardized Collection of Standardized Clinical Data*** has the most developmental components at higher levels of implementation, with 55% in mid-implementation, and 9% fully implemented. ***Linking Clinical and Other Data for Research*** has the lowest overall level of implementation with 75% percent of developmental components partially implemented, 25% of developmental components in mid-implementation, and no developmental components fully implemented. Finally, ***Use of Enhanced Publicly Funded Data Systems for Research*** is notable in that 25% of its developmental components are fully implemented.

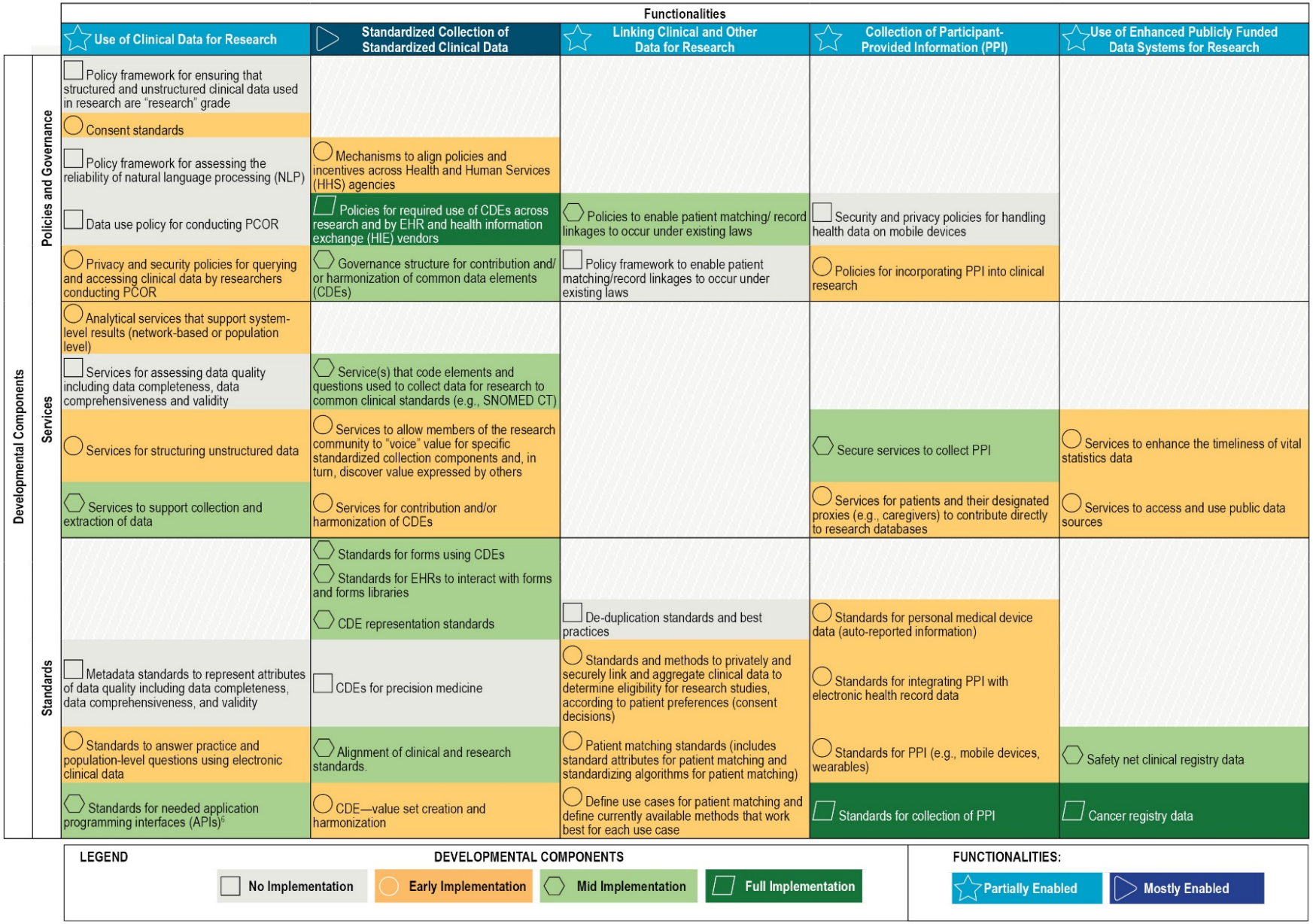
Exhibit 13. Percentage of Developmental Components by Level of Implementation for Each Functionality



A heat map was developed to present a comprehensive view of the status of both the developmental components and functionalities. **Exhibit 14** represents the implementation level of all developmental components and the level support for the 5 core functionalities across the projects included in the portfolio analysis. The columns represent the functionalities and the rows represent the 3 component categories (i.e., standards, services, and policies/governance). The cells represent each of the 42 developmental components, which are color-coded based on the level of their implementation. Nine cells (21%)are shaded in grey, indicating no projects mapped to those developmental components. The 19 yellow cells (58%) represent developmental components in early implementation. The 11 light green cells (33%) represent developmental components in mid-to-late implementation, and the 3 dark green cells (9%) are at the full implementation stage.

The functionalities along the top row are also colored coded based on their maturity status. As noted previously, four of the 5 functionalities are partially enabled. The ***Standardized Collection of Standardized Clinical Data*** functionality had many developmental components in the mid-to-late implementation level, and therefore, was the best supported functionality with a maturity level of mostly enabled. Among the components, standards, had 3 developmental components for which there were no relevant project activities, services had one developmental component for which there were no relevant project activities. Policies and Governance was the least well supported component, containing 5 developmental components with no relevant project activities.

Exhibit 14. Heat Map of Developmental Components Needed to Enable the 5 Functionalities



2.4 Portfolio Discussion

The document analysis provided important insights into how OS-PCORTF projects are advancing needed standards, services, policies and governance, and are enabling the functionalities. The impact of these findings within the context of the 3 global evaluation questions is also discussed.

2.4.1 Overarching Question 1: What contributions were made by the projects funded by the OS-PCORTF to help advance needed standards, services, policies, and governance?

The 20 projects analyzed in the portfolio evaluation made important contributions toward advancing needed standards, services, policies, and governance. Across the 20 projects, activities mapped to most of the developmental components. Interestingly, the formative projects had activities that mapped to a greater number of developmental components than did the summative projects. This may reflect the fact that some earlier summative projects were conceived based upon the earlier ARRA mandates, before the Roadmap and Strategic Framework were developed.

The policies and governance component had the lowest level of project support. Policies are particularly important because specifying conditions of use (in policies) is often a prerequisite to the development of both standards and services. Other areas with limited support included developmental components for data quality and linkage, and privacy and security. Specific developmental components without support included *Policies for research-grade clinical data*, *Natural language processing, Data use policies for conducting PCOR, Handling health data on mobile devices, Standards for metadata CDEs in precision medicine* and *De-duplication of standards and best practices.*

Project Activities that Did Not Map to Existing Developmental Components

Three projects had activities that did not map to any of the 42 existing developmental components, but had tangible products or deliverables resulting from the activities (see **Table 7**).

Table 7. Activities Assigned a "Does Not Map – Possible New Developmental Component" Code

Project	Goal	Activity	Proposed developmental component
13-003 -- Creating the Foundational Blocks for the Learning Health Care System - Structured Data Capture	Goal 1 -- Identify, Develop, Pilot, and Ballot standards for CDEs	Activity 1.2 -- Create use case document to guide structured data capture efforts	None proposed. However, these activities do support the stakeholders' recommendation to focus on the implementation of standards
		Activity 1.3 -- Develop draft implementation guide	
15-018 -- Security and Privacy Standards for Patient Matching, Linking and Aggregation	Goal 2 -- Create an open-source visual tool for patient matching and aggregation	Activity 2.1 -- Tool Specifications Document - Design and Component Development v.1	Services to securely and privately link data
		Activity 2.2 -- Deliver integration with PS API and NPPES v.2	
		Activity 2.3 -- Distribute Version 2 to CHARN and other partners	
		Activity 2.4 – Pilots	
15-019-ONC -- PCOR Privacy and Security Blueprint Legal Analysis and Ethics Framework for Data Use and Use of Technology for Privacy	Goal 1 -- Develop a privacy and security data infrastructure blueprint and legal analysis	Activity 1.1 -- Prioritization of PCOR use cases, data flows, and type and purpose of data	Policy framework for privacy-preserving access and querying of clinical data by researchers

The review committee examined each of these activities and determined that 2 of the projects had activities warranting the addition of a new developmental component. Activities 2.1 through 2.4 for the project "Security and Privacy Standards for Patient Matching, Linking and Aggregation" suggest the need for a new developmental component. The proposed developmental component, *Services to securely and privately link data*, would fall within the Services component in support of the ***Linking Clinical and Other Data for Research*** functionality.

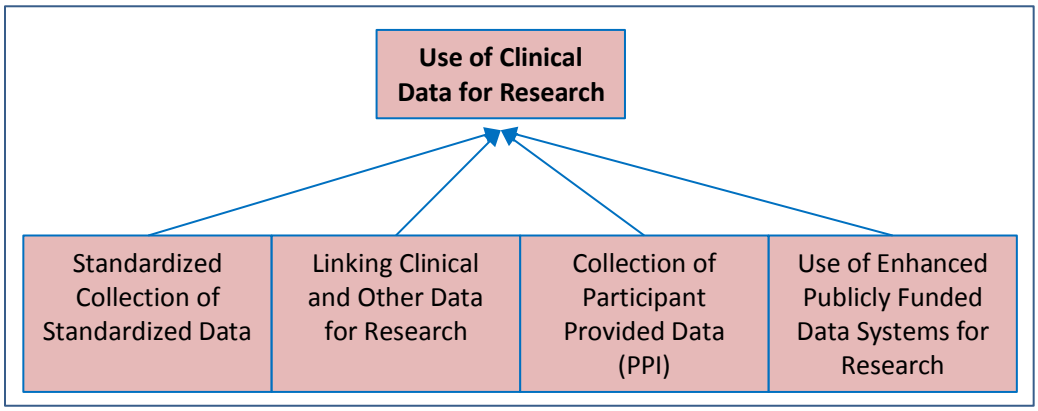
Activity 1.1 for the project "PCOR Privacy and Security Blueprint Legal Analysis and Ethics Framework for Data Use and Use of Technology for Privacy" also suggested the need for a new developmental component. Relevant activities have more to do with developing a policy framework than the policies themselves. The proposed new developmental component, *Policy framework for privacy-preserving access and querying of clinical data by researchers*, would fall within the Policies and Governance component supporting the ***Use of Clinical Data for Research*** functionality.

2.4.2 Overarching Question 2: To what degree has the OS-PCORTF portfolio of projects enabled the functionalities specified in HHS' Strategic Framework?

The portfolio of projects made important contributions toward enabling the 5 functionalities specified in HHS' Strategic Framework. All the functionalities were at least partially enabled, and one functionality, ***Standardized Collection of Standardized Clinical Data***, was mostly enabled. Entities may have recognized that standards are a necessary early step in the process to enable all the functionalities. None of the functionalities remain in the planning stage; however, none have been fully enabled either.

The 2 functionalities with the most unaddressed developmental components were ***Use of Clinical Data for Research*** and to a lesser degree, ***Linking Clinical and Other Data for Research***. ***Use of Clinical Data for Research*** is a broad area and depends in part on the level of enablement of the other four functionalities. For instance, ***Use of Clinical Data for Research*** may depend on the advances in ***Standardized Collection of Standardized Clinical Data***) and ***Linking Clinical and Other Data for Research.*** **Exhibit 15** graphically depicts this implied relationship among functionalities.

Exhibit 15. Implied Relationship Among Functionalities



Developmental components for the ***Collection of PPI*** may be lacking because projects were added more recently for this functionality than for others. Finally, the findings suggest that the functionality ***Linking Clinical and Other Data for Research*** is missing one or more developmental components for services to link data that, when added, will benefit both the ***Linking Clinical and Other Data for Research*** and the ***Use of Clinical Data for Research*** functionalities.

Finally, it is important to note that if the full portfolio of projects could have been included in the evaluation, the results may have shown greater levels of enablement for each functionality. Four recently initiated projects were excluded from the evaluation because

they did not meet the threshold of progress reports at the time of the evaluation. However, based on the information available (primarily in the statement of work) for each of the four projects, they may enable some functionalities once the projects are completed. Three of the four projects are expected to impact the ***Use of Clinical Data for Research*** and ***Standardized Collection of Standardized Clinical Data***. One project is anticipated to affect ***Linking Clinical and Other Data for Research*** and another project is expected to impact ***Collection of PPI***. The exact impact on the developmental components, and degree of additional enablement of the functionalities cannot be predicted now, but will clearly add to the current level of functionality enablement. **Table 8** illustrates the functionalities these projects may impact.

Table 8. Possible Functionality Impact of Unevaluated Formative Projects

Project	Use of Clinical Data for Research	Standardized Collection of Standardized Clinical Data	Linking Clinical and Other Data for Research	Collection of Participant Provided Information (PPI)	Use of Enhanced Publicly Funded Data Systems for Research
16-004 – Source Data Capture from EHRs: Using Standardized Clinical Research Data	●	●	●		
16-005 - Use of ADAPTABLE Trial to Strengthen Methods to Collect and Integrate Patient-reported Information with other Data Sets	●			●	
16-006 - Standardization and Querying of Data Quality Metrics and Characteristics of Electronic Health Data	●	●			
16-007 - Harmonization of Clinical Data Element Definitions for Outcomes Measures in Registries		●			

2.4.3 Overarching Question 3: Has HHS specified the Roadmap and Strategic Framework comprehensively in order to build clinical PCOR data capacity and advance researchers' ability to capture, store, access, link, exchange, and analyze data securely and efficiently?

The Roadmap and Strategic Framework appear to comprehensively address the need to build PCOR data capacity and advance researchers' ability to capture, store, access, link, exchange, and analyze data securely and efficiently. The evaluation did not identify the need for any additional functionalities. However, the design of the portfolio evaluation seems better-suited for identifying gaps within the current Strategic Framework than identifying opportunities for new functionalities that fall outside of the scope of the existing

functionalities. Only 20 projects out of a current portfolio of at least 35 projects were evaluated (see ***Appendix F*** for the full list of current projects as of the time this evaluation was submitted). Therefore, as new projects are supported based upon newly identified research needs, or as a result of this evaluation, the Strategic Framework should be reexamined periodically to address the potential need for new functionalities.

2.4.4 Limitations

The portfolio evaluation has several limitations. A primary limitation relates to the documentation reviewed to code the developmental components and determine the status of the project activities. The statements of work used to identify the project goals and activities focused on the work the agency intended to do, not necessarily what was completed. The progress reports the agencies provided to ASPE were primarily for oversight and monitoring work status rather than explaining the work that was done in any detail. The documentation used to code the projects also changed over the years. The initial format was an open-ended summary by goal, which then changed to a version that included the functionalities and components with additional text. Neither of the formats were ideally suited for purposes of the evaluation because they were geared toward reporting progress on deliverables and provided little detail on actual project activities. In addition, the quality and quantity of the progress reports varied across the projects evaluated. Specifically, some of the older projects were not as well documented as more recent projects. Apart from this quality issue, it was sometimes hard to discern from progress reports of older projects which achievements were entirely funded by OS-PCORTF, versus being built by or drawing on earlier ARRA-funded efforts. Some limitations were addressed by supplementing the documentation with clarifying correspondence with the project leads and referral to other publicly available information such as published literature and project descriptions available on contracting agency web sites. However, ultimately, the findings are only as good as the information available regarding each project, and the quantity and quality of information varied across projects.

Due to the timing of the evaluation project within the OS-PCORTF funding cycle, evaluation was conducted only for subset of projects funded early enough to have accumulated at least 3 progress reports by the time of this final document review. Thus, four of the projects funded in FY 2016 and all projects funded in FY 2017 were excluded from this analysis, although when completed, they may contribute significantly to enabling the functionalities.

3. THE STAKEHOLDER EVALUATION

3.1 Introduction

The stakeholder evaluation was developed to obtain the perspective and insights of a broad array of stakeholders regarding the OS-PCORTF efforts to build data capacity for PCOR. Qualitative methods were used to collect the views of stakeholders including agency principals, project leads, and end users, on the progress made toward building data capacity to support PCOR. The research aims for the stakeholder evaluation were developed with input from ASPE and the TEP. The four aims are as follows:

1. Understand key stakeholders' views of the appropriateness of the core functionalities to address gaps in data capacity for PCOR.
2. Evaluate whether and how various key stakeholders used the products from the OS-PCORTF projects to enable the core functionalities.
3. Assess how the OS-PCORTF projects informed and contributed to key federal stakeholders' research needs, helped to avoid duplication, and fostered coordination across HHS.
4. Assess how OS-PCORTF projects and products are perceived to have addressed the research needs of federally and privately funded research network stakeholders.

3.2 Methods

3.2.1 Data Collection

Sampling

Five stakeholder groups were identified to represent key perspectives on the development of data capacity for PCOR in both federally and privately funded environments. The 5 stakeholder groups include:

1. Federal agency leaders whose agencies received OS-PCORTF funds.
2. Project leads from HHS agencies who led OS-PCORTF-funded projects (formative and/or summative).
3. Research network representatives who were involved in building data capacity but whose work may or may not have been funded from the OS-PCORTF.
4. Health care delivery systems and payers with experience building data capacity, either for their own organization's needs or for conducting PCOR.
5. Patient advocates.

Forty-five stakeholders were recruited across the 5 groups to ensure maximum variability in responses. The design adhered to the Office of Management and Budget (OMB) regulations[19] that limit any one question being asked of no more than 9 nonfederally-employed stakeholders.

Interview Guides

Semi-structured interview guides were developed in collaboration with ASPE staff and TEP members for all 5 stakeholder groups. The questions in each guide mapped to the four research aims and they were tailored to each stakeholder group. For example, the interview guide developed for federal agency leaders included questions on the sustainability of data capacity-building efforts, a subject not included in the other four guides. The guides are included in ***Appendix G***.

Identifying and Recruiting Stakeholders

Multiple approaches were used to select potential stakeholders. ASPE staff recruited federal agency leaders within HHS. To initiate the stakeholder interview phase, ASPE hosted a webinar that included a presentation introducing the goals and methods of the evaluation. After the webinar, ASPE e-mailed leadership at each relevant federal agency to explain the purpose of the stakeholder evaluation and invite one or more agency leaders to participate in a single telephone interview. The interviews were scheduled with agency leads based on their availability. HHS project leads were selected using the inclusion and exclusion criteria in **Exhibit 16)**. ASPE invited the selected HHS project leads to participate. Those who agreed were then scheduled for interviews.

Exhibit 16. Inclusion and Exclusion Criteria for Project Selection

Inclusion criteria:

- Mix of agencies (preferably at least one project from each agency)
- Mix of formative and summative projects
- Summative projects with all activities fully achieved
- Formative projects with activities partially or fully achieved
- Formative projects with 3 or more progress reports by June 30, 2017
- Projects that represent a spectrum of developmental components

Exclusion criteria:

- Projects that were not fully executed
- Foundational projects that were not evaluated
- A project that, for whatever reason, has only "Does not map" developmental components

Representatives of the remaining 3 stakeholder groups—research networks, health organizations and payers, and patient advocates—were recruited based on recommendations by ASPE and the TEP by snowball sampling, and through recommendations from professional connections. Interviewed agency and project leads were asked to identify stakeholders from among the 3 remaining groups who may provide valuable information for the evaluation. The project team also used their professional networks to identify additional stakeholders.

Conducting Interviews

Forty-five in-depth, semi-structured telephone interviews were conducted from July 5 through August 18, 2017, after first conducting a pilot interview with an ASPE

representative. Participants were not remunerated for their participation. Counts and characteristics of participants from each stakeholder group are provided in Exhibit 7.

Prior to each scheduled interview, participating stakeholders were emailed a 2-page overview document. The overview described the stakeholder evaluation's four aims, the 5 core functionalities, and the list of OS-PCORTF projects evaluated as part of this project. The 2-page overview document is provided in ***Appendix H***.

Interviews were conducted by an experienced researcher with health IT and qualitative research expertise. A notetaker accompanied the lead interviewer to take notes. All stakeholders gave verbal consent and permission to be audio recorded. In some cases, more than one interviewee participated on the same call.

3.2.2 Data Analysis

Preparing for Data Analysis

Notes from the 45 interviews were imported into an NVivo database for qualitative analysis. Using a coding schema appropriate for the stakeholder evaluation, each set of interview notes was coded with key information about the interviewee (e.g., stakeholder group, organization name, job title, years of relevant experience) and interview (e.g., interviewer, interview date and time). The coding scheme for organizing stakeholders' responses focused on the four research aims.

Content Analysis

Two evaluators conducted the interviews, one of whom also analyzed the content. The coder reviewed the interview notes and applied codes from the stakeholder coding scheme to the responses. As patterns emerged related to the research questions, the coder identified new subtopics which were added to the coding scheme. The evaluation team compared and contrasted themes within and across stakeholder groups. They also reviewed the results and identified themes that addressed each research question and key concept. These coding meetings were used to compare, contrast, and synthesize high-level findings from the stakeholder evaluation with those of the portfolio evaluation.

3.2.3 Recruiting and Interview Statistics

In total, 68 individuals were invited to participant and 45 individuals agreed to participate (81% participation rate, see **Table 9**). Most interviews were conducted individually but some interviews were conducted in groups. Those individuals who did not participate were either unavailable during the data collection time window, declined to participate, or did not respond to emails and phone calls. There were not any notable differences between those who participated and those who did not based on job titles and job descriptions. Of the 10 federal government project leads identified for participation, 8 led their OS-PCORTF-funded

project for the entire duration of the project. The telephone interviews ranged between 16 and 59 minutes; the average interview duration was 36 minutes.

Table 9. Count of Stakeholders, Interviews, and Invitations by Role

Roles	Status	Interviews	Stakeholders	Invitations Sent
Federal Agency Leader	Federal	8	14	14
Federal Government Project Lead	Federal	8	10	10
Research Network Representative	Nonfederal	19	21	32
Patient Advocate	Nonfederal	6	6	6
Health Care Organization or Payer Representative	Nonfederal	4	4	6
Total		45	55	68

3.3 Stakeholder Evaluation Results

The results for the stakeholder evaluation are organized by the four research aims for the Stakeholder evaluation. To distinguish between overall findings and comments from stakeholders from these notes, *italicized text* indicates verbatim quotes from the recordings. Regular text indicates comments or paraphrases. Findings with stakeholder role and unique identification number are also reported so that readers have greater context and can see findings from a variety of stakeholders.

3.3.1 Research Aim 1: Understand key stakeholders' views of the appropriateness of the core functionalities to address gaps in data capacity for PCOR.

Four of the 5 stakeholder groups were asked specifically to comment on whether the 5 core functionalities adequately addressed the data capacity needs to support PCOR (see Section 1.3.1 for the 5 core functionalities).

Stakeholders (particularly nonfederal stakeholders) noted that the core functionalities tended to focus on the technical aspects of data capacity building instead of the factors that facilitated use of data for PCOR. The stakeholders specified four potential gaps in the core functionalities.

First, stakeholders suggested that there are gaps related to governance and data provenance:

- *What's missing is the [emphasis on the] policy infrastructure, which is harder than the technology. The governance of who gets [access to data] or who doesn't get it.* (Research Network Representative)

- *What's missing from the 5 core elements is ethics, privacy, human subjects research, HIPAA, Common Rule, authorization, data ownership. Maybe it's buried inside linkage to some degree but that is the absolute greatest challenge.* (HCO/Payer)

Second, stakeholders also recommended that improving data quality based on its intended use ("fit for purpose") should be a core functionality:

- *These are good general areas, but quality and completeness of the data are crucial and should be highlighted and woven into each of these elements. It's good to focus on these [current core functionalities] but without quality data, each of the efforts will fall apart.* (Project Lead)
- *…evaluation of data quality in determining fit for purpose is important when you want to start using clinical data for research (or any data collected outside of the traditional research process).* (Research Network Representative)

Third, stakeholders recommended including data privacy and security as a core functionality:

- *…if the goal is to avoid breaches, a privacy preserving component should be under [functionality] number 3.* (Research Network Representative)
- *The challenges to clinical data for research are patient privacy issues…* (2 Project Leads)

Finally, stakeholders recommended including the development of novel methods for analyzing PCOR data as a core functionality.

- *…if you have the data you need to share research methodology. Share the next step and how to get from data to actionable insight.* (Research Network Representative)
- *… would like to see efforts to consolidate data collection; … there's a lot of data collection that goes on that's redundant because of the way programs are funded (siloed funding that results in siloed data).* (Project Lead)
- *Most researchers looking to reuse data are those who are trying to reproduce and enhance upon previous analyses, or creating new algorithms. And even though no analyses may be performed, researchers need a data set of a certain type.* (Research Network Representative)
- *There are knowledge gaps in how you go from data to analytic data sets.* (Research Network Representative)

Assessing Individual Core Functionalities

Core Functionality 1: Use of Clinical Data for Research

Clinical data, including those collected in EHRs and from patient self-reports, are key to PCOR. However, stakeholders expressed concerns about quality of data collected for clinical and other purposes and appreciated the significant efforts required to transform those data into research-grade data.

- Federal perspective

- *…using clinical data for research…it gets a little fuzzy when data is in the record [due to inconsistencies between data fields in EHRs, structured vs unstructured fields, etc.].* (Project Lead).

- Nonfederal perspectives
  - *Functionality 1 is extremely important because there's a lot of data out there but if we can't use it, they have nothing to work with or do research on* (Research Network Representative)
  - *…this has been an issue in health care for decades. Even organizations that use EHRs--there's no fundamental interoperability between EHR vendors. Progress in that area would be huge for the kinds of things the OS-PCORTF is interested in.* (HCO/Payer)

Core Functionality 2: Standardized Collection of Standardized Clinical Data

Stakeholders tended to agree on the importance of standardizing data collection and yet expressed differing opinions on how to execute a federal effort effectively to facilitate this functionality. One criticism offered by stakeholders was an underappreciation of the difficulty getting organizations to agree on standards and standardized data collection.

- Federal perspective
  - *…collect the data once and use many times is important to reduce burden and redundancy. In case of their programs, for them to collect the same set of data multiple times over multiple programs in different ways is not a good thing.* (Agency Leader)
- Nonfederal perspective
  - *…it's not going to happen. It doesn't matter how precisely you define a value set, nobody is being paid to collect it and collecting data according to a value set doesn't impact care. These data will always fall short of the research needs.* (Research Network Representative)

Core Functionality 3: Linking Clinical and Other Data for Research

Stakeholders broadly supported this functionality and agreed on the need to improve linkages between, and interoperability of, all types of data that can be used for PCOR.

- Federal perspective
  - Linking clinical data with claims data will enhance research, because not only will users have the ability to see clinical data but they will see how the claims data plays into it. This is an extremely good use of PCOR funding and will help patients. (Project Lead)
- Nonfederal perspective
  - Without linking clinical data, it is not possible to have a robust national data infrastructure that can be used for clinical research. The linking and

standardized collection are building blocks to get towards enabling a national infrastructure for research. (Research Network Representative)

Core Functionality 4: Collection of Participant-Provided Information (PPI)

Stakeholders differed in their opinions about whether PPI should be added as a core functionality, even though there was broad agreement that this functionality would become more important over time.

- Federal perspective
    - *For the fourth one, that's the strongest. This is a new type of technology; it's mobile devices being used in a new way.* (Project Lead)
- Nonfederal perspective
    - *…it's a good idea, and there's a lot of value there.* (Research Network Representative)

Core Functionality 5: Use of Enhanced Publicly Funded Data Systems for Research

Of the 5 core functionalities, this one was the hardest for stakeholders to respond to and. therefore, it generated the fewest responses. Stakeholders lacked familiarity with the term, despite reviewing a summary of all of the OS-PCORTF projects included in the evaluation.

- Federal perspective
    - *For enhancing publicly funded data systems for research, I don't know if there are any projects dealing with this aspect*. (Agency Leader)
- Nonfederal perspective
    - *…I need education on what the funded data systems are*. (Research Network Representative)

3.3.2 Research Aim 2: Evaluate whether and how various key stakeholders have used the products from the OS-PCORTF projects to enable the core functionalities.

To address this research aim, stakeholders who were involved with the PCORTF portfolio or work were asked how the products of their work may have been used to enable the core functionalities to support PCOR. Those stakeholders who did not participate in a OS-PCORTF project (e.g., Research Network Representatives) were asked how their data capacity-building efforts aligned with or advanced the core functionalities. Those stakeholders in federal roles who worked closely with the projects were understandably better able to relate their project deliverables to the functionalities than those outside the federal government. Therefore, this evaluation reports only federal perspectives on the products from the projects by individual functionalities.

Overall Assessment of Products by Core Functionalities

Overall stakeholders had some difficulty specifying how their projects' end products mapped to the core functionalities. This was especially true of stakeholders from federal agencies.

Those stakeholders from the private sector commented that their organizations' efforts aligned with the core functionalities, even if they did not directly map to the functionalities. Thirteen of 17 nonfederal stakeholders who were asked about their familiarity with the OS-PCORTF portfolio conveyed some level of familiarity with one or more of the projects. However, only 4 of the 13 could state they knowingly used any products or outputs from any portfolio projects.

- Federal perspectives
    - One of the project leads mentioned not knowing if there's an answer to how products would deliver value to the end user given that the project was complex. (Project Lead)
    - *In terms of the functionalities, they are pretty well reflected in structured data capture and the data access framework.* (Project Lead)
    - *…a lot of the projects that were funded… were so complicated and so technical, it's hard to comment on their impact…* (Agency Lead)
- Nonfederal perspectives
    - *In terms of the end products, some of these are a little more difficult to use in practice… The [structured data capture] project is in its infancy. That's always a question, at what point are [end products] ready for use?* (Research Network Representative)

Assessing Products from OS-PCORTF Projects Related to Individual Functionalities

Products for Core Functionality 1: Use of Clinical Data for Research

Federal stakeholders commented that efforts to create implementation guides and metadata standards, and use of natural language processing (NLP) techniques to classify unstructured clinical text are good examples of work that advances the use of clinical data for research functionality. However, the projects did not produce specific products that could be used by others to advance their work. Those outside the federal government (e.g., payers, etc.) were unfamiliar with the OS-PCORTF projects supporting this core functionality.

- *…there is a lot of work that has created implementation guides that can then populate metadata sources, which could be a common data element library.* (Agency Leader)
- *This project is collaborative with the FDA and what we're developing has the potential to not just be for public health, it could be clinical across the board. They are working toward some general functionality that could be implemented for other health care domains. It's applicable to the broader clinical care (not just public health).* (Project Lead)
- *NLP does sound very useful but I just didn't know about these [OS-PCORTF efforts].* (Research Network Representative)
- *NLP is definitely a methodology we would like to explore but I was not aware of this work.* (Research Network Representative)

Products for Core Functionality 2: Standardized Collection of Standardized Clinical Data

Stakeholders recognized the challenges to ***Standardized Collection of Standardized Clinical Data***, and explained how they could benefit from access to one or more resources that point them toward the use of common standards and standardized data.

- *The CHARN project was a game changer in some ways, but not in others. It's had some sustainability issues. At the end of the day, if (federally-qualified) health centers have to shift resources it will always go toward actual patient care.* (Agency Leader)
- *[Standardized clinical data] is also applicable, since a lot of the tools and instruments they are working with are actually used in clinical care as well.* (Project Lead)
- *[We] ran into reimbursement issues. CMS isn't paying for physicians to collect clinical data so it doesn't exist yet*. (Project Lead)
- *…another project created a website [Cross Network Delivery Service] that allows access to information about data standards. "I think that's been very useful", both as the start to having a place to look for core element definitions and in clarifying the magnitude of the variability.* (Agency Leader)
- *…develop a directory of all of the data partners. If a new data partner wants to be added, then they can provide the types of data they have. With a goal of cross network directory services, new data partners can register their services and then (depending on what data is needed for research) others can reach out to them.* (Agency Leader)

Products for Core Functionality 3: Linking Clinical and Other Data for Research

Stakeholders commented on the considerable work being conducted by the OS PCORTF portfolio as well as additional ongoing work to improve linkage among data sources. The general consensus was that important progress is being made to advance this specific functionality. Stakeholder noted that this functionality draws from all of the components—services, standards, policies, and governance structures.

- *The multi-payer claims database is a big deal…. if you're just looking at Medicare and Medicaid data, you will get a very different perception for decision making.* (Agency Leader)
- *The OS-PCORTF money is also helping with an evaluation, done by another contractor, to help develop a business model that will decrease the cost of data linking for researchers.* (Agency Leader)
- *[Work is still going on] to build the API that will allow clinicians to connect their data. The 5 core functionalities should enhance the linking of claims data with the collection of standardized clinical data. Focus on number 3 since that allows a better stakeholder patient view*. (Project Lead)
- *Linking Clinical and Other Data for Research is one of the most important ones. For instance, there are some disorders where there's not a lot of clinical data that's generated. There's a lot of care that's happening outside the medical system (like at school or at home), that data will be missing in the EHR. If you can link the EHR data*

with the data that's reported directly by patients or by care providers, that will be a huge advantage. (Patient Advocate)

Products for Core Functionality 4: Collection of Participant-Provided Information (PPI)

Although stakeholders did not pinpoint specific PPI-related end products from the portfolio, they expressed great interest in the collection of PPI and strongly advocated for ongoing research in this area.

- *Developmental component has done some work around the Blue Button. They've used that with some of the infectious disease projects. More collaboration there might be good.* (Project Lead)
- *[Collection of PPI] is relevant because it's the claims information that beneficiaries will individually agree to share with a research entity, and possibly number 2 as well.* (Project Lead)
- *[Collection of PPI] is huge… it is going to be really important, probably more important than core functionality number 2 [standardization of clinical data].* (Research Network Representative)
- *…how can we make some of the things that have been developed (blue button, for example) available to our grantees and researchers? How can we make something from this effort available to the research community at large?* (Agency Leader)
- *To be able to look at groups of data that might give you, from a retrospective perspective, it might give you some kind of an idea.* (Patient Advocate)
- *A lot of times, people are asked to report things and it goes into a black hole.* (Patient Advocate)

Products for Core Functionality 5: Use of Enhanced Publicly Funded Data Systems for Research

Stakeholders generally had difficulty discussing what constituted publicly funded data systems. However, multiple stakeholders mentioned the value of greater and more economical access to National Death Index data.

- *Funding for the OS-PCORTF NDI project has been about contributing to the improvement of mortality reporting but also the OS-PCORTF is helping with taking the data and more effectively getting it into the NDI.* (Agency Leader)
- *When researchers match against the preliminary file, they can be sure 90% of the deaths in the country are in the file. Further, if they use the preliminary file, they can pick up the extra 10% in the November/December time frame when it becomes available. This has really been something that has picked up and that researchers have liked.* (Project Lead)
- *The mortality data (not having better access) to the NDI, has been a long-standing frustration for people doing this kind of research*. (Research Network Representative)
- *… [Enhanced Publicly Funded Data Systems for Research] are more of an emerging area and will be more important when the framework exists*. (Project Lead)

3.3.3 Research Aim 3: Assess how funded projects informed and contributed to key federal stakeholders' initiatives, helped to avoid duplication, and fostered coordination across HHS.

Stakeholders reported that the OS PCORTF portfolio of projects has had an impact on data capacity. Neither the federal or nonfederal stakeholders commented on issues related to duplication of effort across projects. The overall body of work was viewed as valuable to advancing the cause of building data capacity to support PCOR.

Cross-Agency Data Capacity

Stakeholders perceived that cross-agency data building capacity was increasing, particularly through better collaboration and knowledge sharing among those at federal agencies.

- *No question, that it's steadily getting better. Believe this investment is incredibly important. It's complex and hard to oversee. Big data pulled from real-world care in answering questions is coming along and while it's only one piece, it's an important part of the federal investment.* (Agency Leader)
- *Even though they are only talking about the part of the OS-PCORTF [managed] by the Office of the Secretary, the PCORTF is much bigger and this is a component of a larger fund. OS-PCORTF has been working with PCORI and PCORnet. PCORnet has come a long way and they have put together a robust data infrastructure, which can perform queries, the kinds of which they couldn't have thought of 5-7 years ago. They are in a better spot now than before.* (Agency Leader)

Coordination and Collaboration

Coordination and collaboration among federal agencies are key components to the OS-PCORTF effort. Federal stakeholders appreciated partnering across agencies and they appreciated ASPE's role in facilitating partnerships. However, federal stakeholders indicated they were less aware of OS-PCORTF funded projects if their agency was not involved. Nonfederal stakeholders described frequent efforts to collaborate on projects to build data capacity.

- *The [OS-PCORTF] projects have built better bridges between HHS agencies, the National Institutes of Health, and Food and Drug Administration*. (Agency Leader)
- *They are working with multiple agencies (FDA, CMS, HRSA, NIA, NCI) to increase the impact of their work and it has been a productive collaboration… They also recently engaged with NCI closely to harmonize measures for lung cancer. Their work is more efficient when there is collaboration across agencies.* (Agency Leader)
- *There is a strong interest in trying to collaborate and money helps remove some barriers to collaboration. That being said, money is not enough. ASPE's involvement in trying to promote knowledge and understanding of these projects is a critical component of their success*. (Project Lead)
- *…our organization partners with local health care organizations, all benefit from learning about each other's data systems and patient-centered outcomes. We also partner with a number of universities and community partnerships, researching*

things that range from obesity mapping, food insecurity, to healthy eating and active living, to outcomes bariatric surgery. (Research Network Representatives)

- *The biggest barriers are time and money… They've done research on data sharing and they find that people express an interest in broad data sharing, especially if it's not going to financially enrich people, but it's seen as data sharing in the interest of public good… (Research Network Representatives)*
- *The barriers [to coordination and collaboration] include patient privacy and regulatory…. They've pushed the envelope on software security and secure networks.* (Research Network Representatives)

3.3.4 Research Aim 4: Assess how funded projects and products are perceived to have addressed the research needs of federally funded and privately funded research network stakeholders.

Identifying End Users

Both federal and nonfederal stakeholders identified similar types of end users of data capacity-building work. Both project leads and research network representatives view "the researcher" as the primary end user, followed by a variety of secondary end users (see **Table 10**).

Table 10. Examples of Identified End Users

Project Lead	Research Network Representative
They can be clinical researchers, federal researchers, state agencies (for instance - central cancer registries), and other developmental component programs. *They have a wide array of users in the federal sector and outside the federal sector.*	*It's faculty, student, and staff investigators.* *On one hand, it's going to be faculty and students doing research projects.* *On another hand, it's going to be a health system.* *Increasingly, it's also external investigators to help advance broader more generalized research.*

Generating value for users in PCOR would come from increased linkages between different types of data resources particularly linking Medicare/Medicaid claims data to EHR data, to better track the course of health, disease severity and changes to disease course and severity over time, as well as the quality of patient care across settings. End users would also gain additional value from a centralized resource that points researchers, patients, and other interested users to data resources that are available and the requirement for accessing these data taking privacy and security needs into account.

- *…the only way (right now) to find out if there are enough patient lives for a particular study is if you know people, who know people, who know people (which is really clumsy). If they could get owners of data sources to enroll in [a central system] and share meta-data about their data sources, they would have a hundredfold/thousand-fold more effective way for researchers to find data sources, and to find other researchers with expertise they might be looking for*. (HCO/Payer)

3.3.5 HHS Agency Leaders' Strategic Visions for Building Sustainable Data Capacity for PCOR Strategic Vision

ASPE asked that federal agency leaders be asked about their strategic visions for the OS-PCORTF Portfolio and building data capacity for PCOR more generally. Agency leaders offered a broad vision *to standardize and structure data* (2 Agency Leaders), as well as *understand and facilitate the capacity for participation in registries, information sharing, and outcomes research*. (Agency Leader) They highlighted the goal to increase volumes of data, availability of information, and access to various types of data for PCOR. They uniformly perceived that the OS-PCORTF *directly enables* that vision and goal: *Within the 21st Century Cures Act, FDA was mandated to leverage more data for clinical trials, OS-PCORTF directly enables that.* (Agency Leader).

Leaders recognized the need to develop new opportunities for using data and new techniques for leveraging the data for PCOR and discussed the challenges of data collection, particularly clinical data. Looking ahead, agency leaders noted ample opportunities for continuing work in building data capacity for PCOR.

Potential weaknesses included an inability for these leaders to *"get a good sense of what the gaps are around data infrastructure"* (Agency Leader) and align agency norms such as resolving differences between contract and grant funding mechanisms for projects. Also of note, the topic of data quality did not arise in these interviews as much as the topics of generating and providing greater access to data and information. This finding is notable given comments from non-agency leads and is addressed in further detail in this report.

3.3.6 Sustainability of OS-PCORTF Projects

Agency leaders were asked about sustainability of the work to get their viewpoints on strategy and perceived long-term impact, but sustainability arose organically in interviews with non-agency leaders too. Sustainability is key to long-term success yet stakeholders seemed unclear how sustainability will be achieved. Recommendations included connecting the OS-PCORTF investment more explicitly to homeland security and disease surveillance efforts.

- *…the promise for the OS-PCORTF investment was that a durable infrastructure was being created… if [projects] do have impact and can then be scaled to do other things that would be a good investment.* (Agency Leader)
- *The work that's ongoing is critical and will help support PCOR down the line; however, the challenge is that dissemination, uptake, and sustainability weren't baked into the project. (*Agency Leader*)*
- *The projects weren't designed to address the sustainability of data infrastructure.* (Agency Leader)
- *It's premature to say what effect it's had on sustainability. There is a lot of work left to be done.* (Agency Leader)

- *it's not clear who ASPE hands [the projects] off to… it would be good to hand it off to* PCORI (Agency Leader)
- *The path to market requires building and sustaining that infrastructure that can be re-used for other things (homeland security and disease surveillance) that's going to be the balance necessary to provide a business case. … At one time (referencing the change in administration) there was interest in coming up with a sustainability model for this effort. This could be really valuable, but [researchers] need to know about it. There is a lot of good work, but you need to maintain them and determine who is going to govern the rules of the infrastructure.* (Agency Leader)

3.3.7 Generating Value for Federal and Nonfederal Researchers

Stakeholders were asked what areas of work they would like to see carried out in the future should funding be made available.

Governance

Stakeholders highlighted the need for further developments and improvements in governance to enable access to data. And though governance is perhaps the most important and pressing, this issue may be the most difficult to overcome when building data capacity.

Data Quality

Assuming access to data is available, stakeholders highlighted greater attention toward the completeness and accuracy of the data and how to reassure researchers that the data they are using are valid and reliable. Given the many pitfalls to collecting data (poor EHR user interfaces, inefficient clinical workflows, insufficient metadata), poor data quality increases costs, impacts interoperability, and may even jeopardize research conclusions. After governance, data quality may be the most challenging area to address.

Privacy and Security

One interviewee commented, *"issues around governance and data provenance are the most difficult [to solve] and may be the most necessary to enable access to data and, as a result, increase data capacity."* Furthermore, developing the policy infrastructure for data sharing and data privacy and security is harder than developing the technologies. Some stakeholders noted that privacy and security were not listed as a core functionality. They noted that needs in this area come from primarily policy guidance and technological solutions.

Dissemination

Stakeholders expressed a need to better understand the OS-PCORTF efforts taking place in building data capacity for PCOR. This need included not only updating stakeholders about program and/or funding developments, but also being aware of new standards and available data. Federal and nonfederal stakeholders would benefit from well-publicized processes and mechanisms that help people make sense of the OS-PCORTF's data building efforts.

Adoption and Use

Developing and making available various standards does not ensure that health care entities (health care organizations, research shops, etc.) will use and implement those standards. Stakeholders noted the difficulties and costs with putting any standards into effect ("last mile problem"). Future work in implementation science and human factors research may be able to inform organizations on how to more efficiently and cost-effectively implement data standards for making PCOR a reality. Activities in the portfolio are already under way to address this recommendation such as, but not limited to, ONC's "Structured Data Capture" project, which included creating use cases documents to guide structured data capture efforts and developing draft implementation guides.

Data and Data Element Products

Similar to the "Dissemination" area above, stakeholders highlighted value in having a central point of access to current data and vocabulary standards as well as data sets. Other fields leverage open-source communities to make libraries of rules and data available for access, updates, and even for experimenting with data (i.e., software platforms such as jQuery, R, Kaggle, Python). Note that this need does not run counter to the goal of developing a distributed data architecture; users (and potential users) expressed value in having a hub from which tools, resources, news and updates can be found.

Mobile Health and PPI

Mobile health (mHealth) and collecting patient-provided information (PPI) is one of ASPE's core functionalities and is a rapidly growing area of interest. However, many questions (and concerns) remain about the validity and reliability of these data, as well as how exactly those data are to be incorporated into PCOR specifically, and health care in general. Some stakeholders questioned why PPI was considered important and mature enough to be one of ASPE's 5 core functionalities. Yet an agency leader stressed that focus needs to be placed on data infrastructure that supports mobile, wearables, and the Internet of Things.

Workforce and Education

As the sources and volumes of data for PCOR grow and diversify, new methods and skill sets will be necessary to make those data meaningful. Interviewee responses demonstrate that the need extends beyond clinical researchers and informaticians to public health researchers, regulatory agencies, and even patients themselves.

3.3.8 Stakeholder Research Aim 1: Understand key stakeholders' views of the appropriateness of the functionalities to address gaps in data capacity for PCOR.

Stakeholders generally agreed that the 5 functionalities are appropriate and laudable goals to build nationwide data capacity for enabling PCOR. The functionalities represent well-known needs, which include common data models, common data elements, interoperability-

supporting standards, and structured data capture. Most stakeholders agreed that the functionalities addressed the needs for building data capacity for PCOR. However, a subset of stakeholders noted pivotal areas of focus that are not explicitly addressed in the current set of functionalities. More specifically, they stated that the 5 functionalities primarily focused on technical aspects for building data capacity, while not addressing factors that are essential (and most difficult to achieve) for improving PCOR: a) enhanced data governance and provenance; b) data quality; c) data privacy and security; d) efficient and economic translation of standards into clinical care; and e) application of novel methods that better account for the amounts and variability in data types routinely encountered in PCOR. While acknowledging the need for better data quality, some might argue that data quality is a "local" issue to be addressed by the health care organization. The best a federal effort might undertake is to determine policies, standards (e.g., metadata standards to describe data quality), or governance for data quality, which would be reflected in the functionalities and components without requiring a new functionality. This may be a possible route for data governance, privacy, and security.

The 5 functionalities are necessary for achieving effective data capacity for PCOR, but others might be considered. A recurring theme among a subset of stakeholders was that the functionalities need to explicitly address data quality and strategize around the means for improving quality. Stakeholders also suggested that ASPE explicitly incorporate data governance and quality as functionalities, particularly ways they may enhance data privacy and security, implement standards, and promote novel analytics. An important caveat is that the stakeholders did not see the developmental components that were developed for the portfolio evaluation, so they were not aware that some of them addressed data quality.

3.3.9 Stakeholder Research Aim 2: Evaluate whether and how various key stakeholders have used the products from the OS-PCORTF projects to enable the functionalities.

Stakeholders across roles were knowledgeable about use and linkage of the NDI to other data resources, FDA's Sentinel initiative for prospective drug safety surveillance, and the Medicare and Medicaid data as they related to the 5 functionalities. Stakeholders from the federal perspective appeared less able to specify how end products mapped to functionalities than they were able to comment on the value of the functionalities themselves. Those from the nonfederal perspective said that their organization's project efforts aligned with the functionalities although many were unaware of the OS-PCORTF projects and their final products.

This evaluation was unable determine if and how stakeholders use the products from the 20 OS-PCORTF projects to achieve the 5 functionalities. Although federal and nonfederal stakeholders agreed in general consensus about the utility of the functionalities, they can benefit from further guidance on how their products map to the functionalities. Stakeholders

will benefit from further guidance and education about how they can conceptualize and report how their products benchmark against the functionalities and also how those products: a) reduce costs to access, b) improve accessibility and analysis efficiency; and c) improve data quality. A significant portion of nonfederal stakeholders were unaware of, and commented on, the potential value of the OS-PCORTF portfolio of projects to their own research. ASPE would do well to strategically plan and execute the ways it disseminates funded efforts to federal and nonfederal stakeholders.

3.3.10 Stakeholder Research Aim 3: Assess how funded projects informed and contributed to key federal stakeholders' initiatives, helped to avoid duplication, and fostered coordination across HHS.

Stakeholders from the federal perspective broadly agreed that the OS-PCORTF projects increased cross-agency data capacity. Multiple stakeholders attributed the increase to improved cross-agency collaboration and knowledge sharing among federal agencies. They pointed to ASPE as a driver of those collaborations. Stakeholders also praised the OS-PCORTF for promoting coordination, and even fostering partnerships, among federal agencies. They reported those efforts as key components toward developing data capacity in PCOR.

Both the federal and nonfederal stakeholders interviewed expressed a desire to better avail themselves of past, ongoing, and upcoming OS-PCORTF efforts but stressed that they needed to be made aware of these efforts. They suggested that HHS provide opportunities allowing them to better track efforts on building data capacity for PCOR. In addition, stakeholders (including agency leaders) were uncertain how OS-PCORTF efforts to date will be sustained for the long term. Stakeholders were unable to identify OS-PCORTF projects that have duplicated efforts with other HHS or private initiatives focused on data capacity building, but rather how the OS PCORTF projects have been synergistic to agency efforts.

ASPE plays a pivotal role in identifying critical needs for building the nation’s data capacity as well as coordinating multiple funded projects across agencies and promoting collaborations among them. Federal stakeholders welcomed opportunities to work across agencies to tackle common challenges and identify common solutions that could result in efficient and cost-effective end results. Agency representatives will also benefit from tools that enable them to be aware of interagency projects. Given this, effort might include a “one-stop shop” that has updated policies and tools, facilitating identification of novel opportunities. Such an effort may promote cross-agency projects and allow alignment with nonfederal efforts. Stakeholders also expressed concern that ASPE work with agencies to better define and plan for the sustainability of portfolio projects.

3.3.11 Stakeholder Research Aim 4: Assess how funded projects and products are perceived to have addressed the research needs of federally funded and privately funded research network stakeholders.

Both federal and nonfederal stakeholders uniformly noted that clinical researchers are the key end users for OS-PCORTF products. They believe the portfolio is generating value for clinical researchers through increased linkages between different types of data resources, particularly linking Medicare and/or Medicaid claims data and EHR data, to better track the course of health, disease severity and changes to disease course and severity over time. Further value can be derived from using these data to assess whether quality of care is improving over time and across care settings.

Whereas the current slate of OS-PCORTF projects target researchers' needs, further focus on governance, privacy and security, and data quality is still needed. Stakeholders repeatedly noted that clinical researchers have needs that are often misaligned with those who are stewards and curators of the data. Clinicians work with the data in real-time and recognize they are valuable but imperfect for PCOR whereas informaticians stress the need for standards to create consistent data for linkage across health systems and payers.

To make a truly effective difference in PCOR, clinical and health services researchers require cost-effective, reliable means for accessing and linking clinical data to one or more of the following: data from claims, EHR, registry, and to the NDI. Future investments are likely to include work on governance for data sources, privacy and security, data quality, and operationalizing related standards.

3.4 Limitations

This stakeholder evaluation has the following limitations. First, although leader representatives from all HHS agencies overseeing OS-PCORTF projects were contacted, due to time and resource constraints, project leads for a subset of 9 projects were interviewed. The small samples of interviewed participants from health care systems, payers, and patient advocates are not representative of all perspectives. Thus, the findings reflect the perspectives of these specific stakeholders and the topics asked about during the interviews.

In addition, the study results should be interpreted with the potential effects of selection and response biases in mind. Specifically, the snowball sampling technique may have led to stakeholders who were more likely to offer positive appraisals of the OS-PCORTF projects. As shown in Table 9 in Section 3.4, multiple interviews included more than one interviewee. Stakeholders may have responded differently with a colleague present than they would have if interviewed alone.

Finally, participants' familiarity with the portfolio varied and it is possible that some responses accounted for accomplishments of other data capacity initiatives that predated this funding or that were conducted concurrently, but were not funded through OS-PCORTF.

4. SYNTHESIS OF THE PORTFOLIO AND STAKEHOLDER EVALUATIONS

This synthesis brings together the discussions from the portfolio and stakeholder evaluations organized around the 3 global research questions and discusses possible areas for future work.

Question 1: What contributions were made by the projects funded by the OS-PCORTF to help advance needed standards, services, policies and governance?

Question 1 focuses on the progress made toward advancing the components that form the critical infrastructure for data capacity to support PCOR. Findings in both the portfolio and stakeholder analyses agree that progress has been made toward implementation of the developmental components. Many OS-PCORTF projects supported implementation of developmental components pertaining to standards and services. This is not surprising, given that standards and services are early steps in the process toward enabling the 5 functionalities. Stakeholders tended to agree on the importance of standardizing data collection, yet expressed differing opinions on how execution efforts. One criticism offered by several stakeholders was an underappreciation of the difficulty in getting organizations to agree on standards and standardized data collection. Some nonfederal end-users also felt that the value of standardized data did not offset the burden of collection. A clear takeaway from the combined portfolio and stakeholder evaluation is that although substantial progress toward standards and services is being made by the project portfolio and stakeholders generally agree on the value of standards and services aimed at standardization of data, nonfederal end users believe that further attention should be paid to efforts that make it easier to collect and use standardized data, careful choice of which data to standardize, and better communication of the value of such data.

Policy and governance were areas warranting further development in both the portfolio and stakeholder evaluations. The portfolio evaluation found that OS-PCORTF projects did not focus heavily on implementation of developmental components related to policies and governance. (e.g., *Data use policy for conducting PCOR* and *Policy framework to enable patient matching/record linkages* to occur under existing laws*)*. There was a total of 5 developmental components focused policy that were not supported by any project activities. There was only 1 developmental component addressing governance, and no project supported it. The stakeholder evaluation aligned with these findings. For instance, one interviewee commented, *"Solving issues around governance and data provenance are the most difficult and may be the most necessary to enable access to data and as a result increase data capacity."* Other stakeholders noted that developing policy infrastructure for data sharing and data privacy and security was harder than developing the technologies.

In the portfolio evaluation, 2 developmental components addressing data quality (one was for a service, the other was for standard) were not addressed by any project activities. Data

quality was also a concern in the stakeholder evaluation, especially among research network representatives.

Finally, the overall evaluation suggests the need to add two developmental components. The first proposed developmental component, titled *Policy framework for privacy-preserving access and querying of clinical data by researchers,* would represent work that needs to be done to facilitate the development of policies for data access*.* The second developmental component would address work that is already under way, but cannot be recognized within the strategic framework as currently conceived. The strategic framework does not contain a developmental component for the implementation of services to support the functionality ***Linking Clinical and Other Data for Research***. Therefore, although there are projects supporting the implementation of such services, their contribution could not be recognized in the evaluation. We propose the addition of a new developmental component, *Services to securely and privately link data,* which would allow the work already under way, as well as future work, to support linkage of data to be recorded and recognized.

Question 2: To what degree has the OS-PCORTF portfolio of projects enabled the functionalities outlined in the Strategic Framework to improve data capacity?

The evaluation found that 4 of the 5 functionalities are partially enabled and one functionality is mostly enabled: ***Standardized Collection of Standardized Clinical Data***. The heat map shown in Exhibit 13 shows that the OS-PCORTF portfolio of projects conducted between 2012 and 2016 focused most extensively on standards for common data elements, their development, harmonization, representation standards, and policies and governance structures for use. The related developmental components are foundational and technologically straightforward focus areas. The stakeholders had divergent views on the value of putting such emphasis on developing standards for clinical data above other key considerations, including governance, data quality, privacy, and security. The divergence may arise from the differing perspectives of the stakeholders, i.e., overseeing projects versus conducting projects, as well as the short- and long-term perspectives they bring to building data capacity. Those in day-to-day operations (short-term view) recognize that a one-size-fits-all standard is not easily achievable, may not be widely acceptable, may be difficult to operationalize, may be too costly, and perhaps most importantly, may not have the desired uptake. Alternatively, those stakeholders at the "50,000-foot" level (e.g., agency leaders) view standards as efficient and reusable, which promotes data consistency across agencies and throughout the marketplace while reducing the burden of data capture. The tension between these viewpoints is an opportunity for future work.

Stakeholders clearly agreed that clinical researchers are the primary stakeholders for data capacity-building efforts, and other stakeholders (patients, clinicians, etc.) are downstream beneficiaries. From a clinical researcher's perspective, the ***Use of Clinical Data for Research*** functionality is likely the most important for conducting PCOR. Clinical data are used for a variety of analyses, ranging from observational studies that can inform disease

burden to pragmatic trials that compare one or more active interventions. However, the results from a well-designed study are only as good as the data that are analyzed—the patient data must be accurate, consistent, complete, and linkable across data sources. AHIMA has long advocated and researched the barriers and facilitators to achieving high-level clinical data quality, and its recommendations are considered the "gold standard" in data for clinical care. Future work could be to more closely engage with AHIMA and its members so to align their knowledge with HHS's aims behind the 5 functionalities. Furthermore, the OS-PCORTF could fund new projects to support innovative ways for collecting patient data that do not rely on physicians' or nurses' manual data entry, e.g. wireless blood pressure cuffs that auto-populate EHRs with structured data.

Three of the 5 functionalities were deemed partially enabled: a) ***Linking Clinical and Other Data for Research***; b) ***Collection of PPI***; and c) ***Use of Enhanced Publicly Funded Data Systems for Research***. That means a majority of their related developmental components mapped to activities that were either "not started" or "partially achieved," as opposed to "fully achieved." They had fewer developmental components than the other 2 functionalities discussed above and had varying statuses of developmental component achievement. Therefore, these areas are recommended as candidates for future investments. As previously noted, four recently initiated projects were excluded from the evaluation because too little information was available regarding their progress at the time of the evaluation. Three of the four projects are expected to impact the ***Use of Clinical Data for Research*** and ***Standardized Collection of Standardized Clinical Data***. One project is anticipated to affect ***Linking Clinical and Other Data for Research*** and another project is expected to impact ***Collection of PPI***. The exact impact on the developmental components, and degree of additional enablement of the functionalities cannot be predicted now, but will clearly add to the current level of enablement.

Question 3: Is the Roadmap and Strategic Framework sufficiently comprehensive as to build clinical PCOR data capacity and advance researchers' ability to capture, store, access, link, exchange, and analyze data securely and efficiently?

This evaluation demonstrates that the OS-PCORTF portfolio has sufficient breadth such that all 5 functionalities are at least partially enabled. Both federal and nonfederal stakeholders generally agreed that the 5 core functionalities provide sufficient guideposts for building data capacity in PCOR. Yet a consideration is whether the breadth of coverage is sufficient to support PCOR as effectively and efficiently as possible, versus focusing on the depth of achievement within specific functionalities. It is worth considering if specific functionalities should take priority over others for increasing the quantity, quality, and accessibility of data for PCOR.

HHS recognizes the importance of data quality. The Strategic Framework has several milestones related to data quality (e.g., ***Use of Clinical Data for Research*** functionality). However, none of the activities within the OS-PCORTF projects mapped to the four

developmental components that address data quality. The importance of data quality was a recurring theme from stakeholders both from federal and nonfederal perspectives. One interviewee said that 2 important elements for building data capacity for PCOR—interoperability and data linkage—rest upon a foundation of robust data quality. Moving forward, HHS may need to consider the "lift" required to measure and improve data quality for PCOR.

Some stakeholders also questioned whether **PPI** should be its own functionality even though they recognized the growing importance of PPI for PCOR. Their concerns revolved around unanswered questions in the field, which include the lack of evidence on how PPI data are measurably reliable and valid. The evaluation found that PPI-related projects had missing developmental components, which further supports lack of development in this functionality.

Although many of those interviewed are well-aware of publicly funded data such as Medicare and Medicaid claims, the wording of this functionality was not well understood. One nonfederal interviewee stated that she "*needs education on what the funded data systems" are* before she could answer whether this functionality is appropriate. Yet both federal and nonfederal stakeholders pointed to Sentinel and the NDI as resources that have particular breadth and depth of impact.

Several stakeholders agreed with the importance of linking clinical data with other data sources and assuring interoperability to achieve a robust nationwide data infrastructure for PCOR. However, many stakeholders remarked that even though extensive investments have been made to promote and achieve data linkages, there is still more to achieve. This thinking aligns with the findings from the portfolio evaluation pertaining to ***Linking Clinical and Other Data for Research*** functionality. The portfolio evaluation indicated that only one of the 6 developmental components were in mid to late implementation status; 3 developmental components had statuses of early implementation, and 2 developmental components had no projects addressing the developmental component. The Linkage functionality has no milestones, and thus no developmental components for services, which is one of the reasons a developmental component is proposed related to linkage: *Services to securely and privately link data.* As HHS revamps the Roadmap and Strategic Framework and considers potential future funding, it may consider whether additional milestones should be added for the linkage functionality.

The developmental components are as appropriate as the functionalities for gauging the development of data capacity for PCOR. Exhibit 4 shows that almost half of the policy and governance-related developmental components did not map to project activities. The stakeholders were clear on the need for additional work in this area, with particular emphasis on policies and governance to promote data quality.

4.1 Considerations for Future Work

The results of the evaluation have pointed to areas where future work may be needed.

4.1.1 Use of Clinical Data for Research

The portfolio evaluation found that few projects addressed infrastructure for the deployment of policies and data use, access, and governance. One specific area of work identified in both evaluations and that has both policy and technical aspects was improving data access for PCOR while maintaining effective data privacy. Areas of potential effort include technical services and standards for privacy preserving, secure linkage of data, and ways to determine the ongoing socio-legal challenges to making patient data available for research.

Stakeholders (primarily research network representatives) suggested focusing on efforts to improve data quality. Data quality has been part of OS-PCORTF efforts to date, and some OS-PCORTF projects that are not part of this evaluation are focused on improving the quality of data available for research. Nevertheless, continued work is needed on data quality issues as new types of data are made available to researchers. Ongoing discussions with stakeholders to assess the specific challenges being faced around data quality will be needed. Several organizations, including American Health Information Management Association, ASTM International, and the International Organization for Standards (ISO) might assist with frameworks for assessing data quality and/or experience with assessment.

Finally, although the reuse of data is attractive, EHRs and other standard data sources may not always capture the data needed for research. Several researchers emphasized the perspective that the data needed for research is "different" than data routinely captured in EHRs. An initiative exploring the nature of data needed for research, but not typically captured as a byproduct of normal clinical care might provide insight into new mechanisms for data capture needed to make data maximally useful for researchers.

4.1.2 Standardized Collection of Standardized Clinical Data

Many stakeholders expressed concerns about both the financial and time burden of collecting standardized data for PCOR. Future efforts that demonstrate monetary value of standards or examine standards' impact on the efficiency and effectiveness of PCOR may be needed. Efforts may also include exploring how organizations may develop policies that require contractors and grantees to use certain standards. Such an approach would extend already existing efforts to require grant applicants to include data sharing plans in their proposals (https://grants.nih.gov/grants/policy/data_sharing/data_sharing_guidance.htm).Finally, more work is needed to promote greater support to clinicians tasked with collecting data for PCOR. Although practicing clinicians are often not the consumers of data used in research, they are almost always the frontline staff tasked with collecting clinical data, and to a large

degree impact the quality of those data. Improved health IT usability and implementation are warranted.

4.1.3 Linking Clinical and Other Data for Research

Future work is needed on efforts to link clinical and other data for research both at the individual and aggregate level. Alternatives may include but are not limited to additional work to explore and refine deterministic and probabilistic linkage methods. This work would entail outreach to ethical, legal, technical, and social leaders to inform strategy as to how new approaches and evolving research needs may make necessary a refresh on how HHS considers this sensitive topic. One approach that might provide certain advantages would be the creation of a task force or some other multi-stakeholder group, rather than trying to address these issues through a traditional project. Finally, other institutions have dealt with privacy issues related to linking of sensitive data. It may be worthwhile to have cross-disciplinary discussions with financial institutions and others who have grappled with privacy and security of confidential data.

Stakeholders across multiple roles also described the value of a service that would be available to advise researchers within a OS-PCORTF distributed data network, acting as a point of contact from which users can more easily locate available standards and data sources as well as keep abreast with news and updates related to collecting and linking data for PCOR.

4.1.4 Collection of PPI

There is great interest in the ability for patient-provided information to contribute PCOR. However, not enough work to date on PPI, or its more generally known term, patient-generated health data (PGHD) has demonstrated the reliability and validity of device-based data. In particular, evidence of benefit and data quality from the consumer wearables market is lacking. Future work could focus on this area and seek to build public-private partnerships around strategies, standards, and even certifications for making PGHD into research-grade data. Additionally, support for initiatives exploring new and better ways to collect patient data, e.g. wireless devices that auto-populate EHRs with structured data could prove valuable.

4.1.5 Use of Enhanced Publicly Funded Data Systems for Research

Although the portfolio and stakeholder evaluations identified few shortcomings related to publicly funded data sources, there is a need to identify ways to access and link to data in publicly enhanced repositories, such as Medicare and Medicaid data, the Chronic Conditions Warehouse, the Healthcare Cost and Utilization Project (HCUP), data from the Veteran's Administration and Indian Health Service, and the National Death Index. Efforts might include economic analyses and exploration of business models that can be used to increase

access to these valuable data at reduced costs to clinical researchers and/or the general public. Policy and governance to guide access and use for research is also needed.

4.1.6 Fostering Better Awareness of OS-PCORTF Initiatives to Build Data Capacity

The stakeholder evaluation revealed that the OS PCORTF projects and products reviewed for this evaluation were not well known among researchers and suggested the need for broader dissemination of the products of these projects. Additional work is needed to foster greater awareness of initiatives to build data capacity for PCOR.

5. CONCLUSION

This formative evaluation, completed in mid-2017, documented progress of the OS PCORTF portfolio of projects that were active or completed between 2012 to 2016. The findings indicate that data capacity for PCOR has advanced through the structuring, linking, and sharing of e-health data across patient groups and repositories throughout the health care ecosystem. Work continues, and will undoubtedly lead to additional improvements. Further, it is important to remember that improving data capacity is a process which will never be truly complete. As medicine advances, there will always be new data and data types to structure, collect, link, and analyze.

The stakeholders interviewed recommended HHS identify opportunities to disseminate and share the knowledge that has been gained to date, both within the federal government and with outside researchers. One way to do this would a publicly funded meta-data system or catalogue of past and present federally funded data capacity-building projects. Another way is to work with OS PCORTF awardees to specify the organizations that would benefit from their end products.

As the amount of e-health data grows, well-directed efforts should focus on continually improving data quality to promote consistency across data sets that ultimately improve the reliability and validity of research results. Those efforts would do well to further the work done to date in promoting effective governance mechanisms among agencies, research entities, and health systems. Additionally, stakeholders who generate the bulk of e-health data (systems and users) would benefit from greater assistance as to how they can economically and efficiently integrate the standards that the OS-PCORTF work and other organizations generate. This task is not easy; those interviewed noted that these areas are the most difficult challenges in building capacity for PCOR.

New interagency initiatives that are just getting under way may benefit from, or create synergies with, the OS-PCORTF aims to build data capacity. For example, the 21st Century Cures Act[6] includes several provisions that could benefit from lessons learned and progress made from the portfolio, as well as create synergies with the OS-PCORTF on data sharing by requiring NIH recipients to share their data (Section 2014), developing recommendations for a formal policy to enhance rigor and reproducibility of scientific research (Section 2039), on accessing sharing and use of health data for research purposes (Section 2063), addressing and expediting interoperability of data among EHRS (Section 4003), and promoting policies ensuring that patients have access to their electronic data (Section 4006). In particular, the Act address the implementation of policies and mechanisms for secure and private data sharing of identifiable and sensitive data from federally funded research. The Precision Medicine Initiative (Now the "All of Us" initiative) to promote individualized care has developed Privacy and Trust Principles to guide PMI activities regarding governance; transparency; participant engagement and preferences; data

sharing, access, and use; and data quality and integrity.[7] Each of these new initiatives hold the promise of enriching or extending existing OS-PCORTF investments in building data capacity to support PCOR.

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APPENDIX A: GLOSSARY OF KEY TERMS

Activities are defined as work units within a project to achieve the project's intended goals (create data sets, expand electronic health record [EHR] reporting capacity, etc.). Activities equate most directly to the tasks associated with a project. However, to be listed as an activity, it must be operationally possible to assess progress toward completion of the activity. In addition, whereas tasks strictly support the project's deliverables, an activity may continue beyond the end of the project if the project specifies actions or events that will happen once the project is completed.

Application programming interface (API) is a "software application function that can be invoked or controlled through interactions with other software applications. APIs allow the user experience to be seamless between 2 or more software applications, since the APIs work behind the actual user interface."[20]

Blue Button is an EHR-agnostic data and implementation standard that allows patients to download plain text versions of their health records. The Blue Button symbol signifies that a site has functionality for customers to go online and download health records.[21]

Common data elements (CDEs) are "clinical concepts that contain standardized and structured metadata and have unambiguous intent and a clearly delineated value domain. CDEs, such as 'systolic blood pressure,' help to define clinical or administrative concepts, optimizing the data to be reused by researchers and clinicians."[20]

Comparative effectiveness research (CER) is defined as follows: The ACA authorized the conduct, dissemination, and expanded capacity for research that assists patients, clinicians, purchasers, and policy-makers in making informed health decisions. The law refers to this research as patient-centered outcomes research (PCOR) or CER. It defines CER as research evaluating and comparing health outcomes and the clinical effectiveness, risks, benefits of 2 or more medical treatments, services, and items.[22]

Components are needed to enable the PCORTF's 5 core functionalities. They include the following component types: (1) Standards, (2) Services, (3) Policies and Governance Structures. These component types provide needed infrastructure for research data capacity.

Clinical document architecture (CDA) is a Health Level Seven International (HL7) document markup standard that specifies the structure and semantics of clinical documents for exchanging health information. Also see HL7 in this glossary.

Consolidated clinical document architecture (C-CDA) is an implementation guide that specifies a library of templates and prescribes their use for a set of specific document types.

Data access framework (DAF) was established as a standards initiative by HL7 in 2013. DAF is focused on the identification, testing, and validation of the standards necessary to access and extract data from within an organization's health IT systems, from an external organization's health IT systems, or from health IT systems across multiple organizations.[23]

Data capacity, in the context of "building data capacity," means either the creation of more data (new registries, new networks) or the creation of what is needed to make existing and future electronic health data more usable or "liquid" for CER purposes.

Developmental components (Developmental components) are granular subcomponents of the 5 named OS-PCORTF components— (1) Standards, (2) Services, (3) Policies and Governance Structures—that enable the core functionalities detailed in the Strategic Framework.

Electronic health record (EHR) is a digital version of a patient's paper chart. "EHRs are real-time, patient-centered records that make information available instantly and securely to authorized users. While an EHR does contain the medical and treatment histories of patients, an EHR system is built to go beyond standard clinical data collected in a provider's office and can be inclusive of a broader view of a patient's care."[20]

FHIR (pronounced "FIRE") stands for Fast Healthcare Interoperability Resources. "FHIR is a next generation standards framework created by HL7. FHIR combines the best features of HL7's v2, HL7 v3, and CDA product lines while leveraging the latest Web standards and applying a tight focus on implementability."[24]

Functionalities as defined by the Strategic Framework are the core research functions involved with collecting, linking, and analyzing data for PCOR: (1) Uses of Clinical Data for Research, (2) Standardized Collection of Standardized Clinical Data, (3) Linking of Clinical and Other Data for Research, (4) Collection of Participant-Provided Information, and (5) Use of Enhanced Publicly Funded Data Systems for Research.[12]

HL7 stands for Health Level Seven International and is a "not-for-profit, ANSI-accredited standards developing organization dedicated to providing a comprehensive framework and related standards for the exchange, integration, sharing, and retrieval of electronic health information that supports clinical practice and the management, delivery, and evaluation of health services." HL7 oversees multiple data standards including C-CDA, CDA, FHIR, and others.[25]

Interoperability is defined as the ability of a system to exchange electronic health information with and use electronic health information from other systems without special effort on the part of the user. Interoperability is made possible by the implementation of standards.[20]

LOINC stands for Logical Observation Identifiers Names and Codes and is both an organization and a standard focused primarily on describing clinical laboratory data.[26]

Metadata[27] is a set of data that describes and gives information about other data. A **Metadata Standard**[28] is a requirement that defines the meaning or semantics of the data to ensure proper use and accurate interpretation of the data by its owners and users.

Mobile Health (mHealth) is a term to describe mobile technologies as tools and platforms for health research and health care delivery.

Milestones as used in ASPE's 2016 roadmap are generalized developmental components, serving to group the subsidiary developmental components. Milestones also suggest a time frame in which the subsidiary developmental components will be achieved.[3]

Natural language processing (NLP) is the ability of a computer program to extract data from free text and classify free text according to machine-based rules. In the clinical setting, NLP converts providers' notes and narratives into structured, standardized data formats.[29]

Patient-centered outcomes research (PCOR) is the evaluation of questions and outcomes that are meaningful and important to patients and caregivers.[8] PCOR assesses the benefits and harms of preventive, diagnostic, therapeutic, palliative, or health delivery system interventions to inform decision making, highlighting comparisons and outcomes that matter to people, such as survival, function, symptoms, and health-related quality of life.

Patient matching/record linkage refers to the matching and linkage of patient records. Record linkage (RL) refers to the task of finding records in a data set that refer to the same entity across different data sources (e.g., data files, books, Web sites, databases).[30]

Patient portal is a "secure online Web site that gives patients convenient 24-hour access to personal health information" stored within an affiliated health care organization's EHR.[31] A patient portal is traditionally considered to be different than a personal health record (PHR). Also, see PHR in this glossary.

Personal health record (PHR) is "an electronic application used by patients to maintain and manage their health information in a private, secure, and confidential environment." A PHR is traditionally considered to be different than a patient portal.[32]

Privacy is "concerned with the collection, storage, and use of personal information, and examines whether data can be collected in the first place, as well as the justifications, if any, under which data collected for one purpose can be used for another (secondary) purpose."[33]

Registry (patient registry) is a collection of information about individuals, usually focused around a specific diagnosis or condition. Individuals provide information about themselves to these registries on a voluntary basis. Registries can be sponsored by a government agency, nonprofit organization, health care facility, or private company.

Services refer to resources that researchers can use to capture, store, link, analyze, or exchange data or evidence. Services can be provided through a distributed model provided off-site (such as through the Internet), over a network, or through a cloud-based model. Such services will enable researchers to perform critical tasks that they may not have the capacity, expertise, or resources to perform on their own. For this evaluation, services are assumed to be based on standards developed for search services.[12]

SNOMED-CT is "a clinical terminology created by a range of health care specialists to support clinical decision making and analytics in software programs."[34]

Standards are nationally accepted specifications that have been widely approved and adopted because of market forces, community consensus, or regulatory requirements. These include, for example, specifications for capturing, storing, representing, linking, and exchanging data in a secure manner so that accurate information is conveyed to the recipient of the data.[12]

Structured data is defined as "data created through constrained choices in the form of data entry devices, including drop-down menus, check boxes, and pre-filled templates." Structured data also conform to a prespecified syntax.[27]

Structured data capture (developmental component) "was established as a standards initiative in 2013. This developmental component is focused on the identification, testing, and validation of standards necessary to enable an EHR system to retrieve, display, and fill a structured form or template, and store/submit the completed form to an external system and/or repository."[35]

APPENDIX B: DEVELOPMENTAL COMPONENTS SUPPORTING MILESTONE ACHIEVEMENT, BY FUNCTIONALITY*

Functionality: Standardized Collection of Standardized Clinical Data		
Milestone	**Developmental Component**	**Status**
A. By 2019, support the development of a set of research Common Data Elements (CDEs) and support development of a governance structure for CDE harmonization.	CDE -value set creation and Harmonization	Early Implementation
	Alignment of clinical and research standards.	Mid-Implementation
	Governance structure for CDE development and harmonization	Mid-Implementation
	CDEs for All of Us (formerly Precision Medicine)	No Implementation
B. By 2019, support the development of repositories/portals for CDEs, standards for utilizing CDEs for research, and services to allow researchers to easily utilize standardized components	Services for contribution and/or harmonization of CDEs	Partial Implementation
	Service(s) that code elements and questions used to collect data for research to common clinical standards (e.g., SNOMED CT)	Mid-Implementation
	CDE representation standards	Mid-Implementation
	Standards for forms using CDEs	Mid-Implementation
	Standards for EHRs to interact with forms	Mid-Implementation
C. By 2019, support the development of policies to promote the adoption and use of standardized collection components and services	Policies for required use of CDEs across research and by EHR and Health Information Exchange (HIE) vendors	Full Implementation
	Mechanisms to align policies and incentives across HHS agencies	Partial Implementation

* It is important to note that this assessment of milestone achievement is only based on the projects included in this formative evaluation from 2016-2016. As noted on page 38 (Table 8), more recent projects will continue to contribute to the developmental components and achievement of milestones. In addition, in consultation with agency leadership, ASPE will continue to update the strategic framework including the milestones, based on the OS-PCORTF mandates and Department priorities. Appendix F contains a list of OS-PCORTF projects current at the time of this report. For the most recent list of projects, please go to https://aspe.hhs.gov/patient-centered-outcomes-research-trust-fund.

Functionality: Collection of Participant-Provided Information		
Milestone	**Developmental Component**	**Status**
A. By 2019, support the development of policies and share best practices for collection and integration of PPI for PCOR.	Policies for incorporating PPI into clinical research	Partial Implementation
	Security and privacy policies for handling health data on mobile devices	No Implementation
B. By 2019, leverage existing standards and support the development of a core set of standards for the collection and integration of PPI for PCOR, by leveraging existing standards and filling gaps.	Standards for PPI, e.g., mobile devices, wearables etc.	Partial Implementation
	Standards for integrating PPI with EHR data	Partial Implementation
	Standards for personal medical device data (auto-reported information)	Partial Implementation
	Standards for secure data capture, storage, and transmission for mobile devices**	Full Implementation***
C. By 2019, support the development of tools and services that will facilitate the collection and exchange of PPI, including national services for electronic capture and management of PPI and release of data for PCOR.	Services for patients and designated proxies (e.g., caregivers) to contribute directly to research databases	Partial Implementation
	Secure services to collect PPI	Mid-Implementation

* It is important to note that this assessment of milestone achievement is only based on the projects included in this formative evaluation from 2016-2016. As noted on page 38 (Table 8), more recent projects will continue to contribute to the developmental components and achievement of milestones. In addition, in consultation with agency leadership, ASPE will continue to update the strategic framework including the milestones, based on the OS-PCORTF mandates and Department priorities. Appendix F contains a list of OS-PCORTF projects current at the time of this report. For the most recent list of projects, please go to https://aspe.hhs.gov/patient-centered-outcomes-research-trust-fund.

** Previously worded "Standards for Collection of PPI"

*** This status was calculated on previous wording of developmental component.

Functionality: Linking of Clinical and Other Data for Research		
Milestone	**Developmental Component**	**Status**
A. By 2018, leverage existing standards, and support the development of needed standards for patient data linkage.	Define use cases for patient matching and define currently available methods that work best for each use case	Partial Implementation
	Patient matching standards (includes standard attributes for patient matching and standardizing algorithms for patient matching)	Partial Implementation
	Standards and methods to privately and securely link and aggregate clinical data to determine eligibility for research studies, according to patient preferences (consent decisions)	Partial Implementation
	De-duplication standards and best practices	No Implementation
B. By 2018, support the development of a policy framework to facilitate patient data linkage in accordance with existing laws.	Policy framework to enable patient matching/record linkages to occur under existing laws	No Implementation

* It is important to note that this assessment of milestone achievement is only based on the projects included in this formative evaluation from 2016-2016. As noted on page 38 (Table 8), more recent projects will continue to contribute to the developmental components and achievement of milestones. In addition, in consultation with agency leadership, ASPE will continue to update the strategic framework including the milestones, based on the OS-PCORTF mandates and Department priorities. Appendix F contains a list of OS-PCORTF projects current at the time of this report. For the most recent list of projects, please go to https://aspe.hhs.gov/patient-centered-outcomes-research-trust-fund.

Functionality: Use of Clinical Data for Research		
Milestone	**Developmental Component**	**Status**
A. By 2018, support the development of standards that enable secure, electronic query of structured data across clinical research and delivery systems.	Standards for needed application programming interfaces (APIs)	Mid-Implementation
	Standards to answer practice- and population-level questions using electronic clinical data	Partial Implementation
B. By 2019, establish services and tools to support data access, querying, and use, including privacy-preserving analytics and queries.	Services to support collection and extraction of data	Mid-Implementation
	Analytical services that support system level results (network-based or population level)	Partial Implementation
C. By 2018, develop and test metadata standards that describe data quality.	Metadata standards for data quality including data completeness, data comprehensiveness and validity	No Implementation
D. By 2019, develop support services and tools to test the quality of unstructured and structured data to answer PCOR questions	Services for structuring unstructured data	Partial Implementation
	Services for assessing data quality including data completeness, data comprehensiveness and validity	No Implementation

* It is important to note that this assessment of milestone achievement is only based on the projects included in this formative evaluation from 2016-2016. As noted on page 38 (Table 8), more recent projects will continue to contribute to the developmental components and achievement of milestones. In addition, in consultation with agency leadership, ASPE will continue to update the strategic framework including the milestones, based on the OS-PCORTF mandates and Department priorities. Appendix F contains a list of OS-PCORTF projects current at the time of this report. For the most recent list of projects, please go to https://aspe.hhs.gov/patient-centered-outcomes-research-trust-fund.

Functionality: Use of Enhanced Publicly-Funded Data Systems for Research		
Milestone	**Developmental Component**	**Status**
A. By 2019, support the development and enhancement of strategic publicly-funded data systems to facilitate their access and use, and ease retrieval of data for research purposes.	Services to access and use public data sources	Partial Implementation
	Services to enhance the timeliness of vital statistics data	Partial Implementation
	Safety-net clinical data	Mid-Implementation
	Cancer registry data research standards	Full Implementation

* It is important to note that this assessment of milestone achievement is only based on the projects included in this formative evaluation from 2016-2016. As noted on page 38 (Table 8), more recent projects will continue to contribute to the developmental components and achievement of milestones. In addition, in consultation with agency leadership, ASPE will continue to update the strategic framework including the milestones, based on the OS-PCORTF mandates and Department priorities. Appendix F contains a list of OS-PCORTF projects current at the time of this report. For the most recent list of projects, please go to https://aspe.hhs.gov/patient-centered-outcomes-research-trust-fund.

APPENDIX C: TECHNICAL EXPERT PANEL AND REVIEWERS OF FINAL REPORT

RTI formed a technical expert panel (TEP) to assist with the development of an evaluation plan for the Office of the Secretary (OS) Patient-Centered Outcomes Research (PCOR) Trust Fund (TF) data infrastructure initiatives, which are intended to build data capacity for comparative clinical effectiveness research. The TEP provided insight into the evaluation plan's conceptual framework, measurement strategy and the draft evaluation plan through document review and participation in one in-person meeting and 2 virtual meetings. The in-person meeting took place in Washington, developmental component. This document outlines the charge to the TEP and the logistics to support the TEP's work.

C.1 Composition of the TEP

The TEP included individuals with PCOR, informatics, and / or evaluation expertise. Both the health system and patient perspectives were included. As shown in ***Exhibit A-1***, the TEP included individuals representing the academic, commercial, and nonprofit sectors. RTI ensured that experts were also versed in the data infrastructure needs for PCOR by identifying experts involved with research in more than one of the expertise areas.

Table C-1. Individuals who Served on OS-PCORTF Evaluation Technical Expert Panel

Name	Organization
Lesley Curtis, PhD	Duke-PCORnet Coordinating Center
Abel Kho, MD*	Northwestern Medicine
Anna Tosteson, ScD	Geisel School of Medicine at Dartmouth
Charles Friedman, PhD*	University of Michigan
Mark Frisse, MD, MBA*	Vanderbilt University
John Glaser, MD*	Cerner
Shawn Murphy, MD, PhD	Partners HealthCare
Lori Frank, PhD*	PCORI
Richard Wang, PhD	Massachusetts Institute of Technology
Brian Quinn, PhD	Robert Wood Johnson Foundation
Danny van Leeuwen*	Health Hats
John Walsh	COPD Foundation

* Participated in the small working group sessions. In addition, Chuck Friedman provided extremely helpful comments on the moderator guides for the stakeholder evaluation.

RTI reviewers of the report:

- Linda Dimitropoulos, PhD, is Vice President of the Division for eHealth, Quality and Analytics at RTI International. Dr. Dimitropoulos is a recognized leader in health informatics and health IT policy.
- Jennifer Popovic, DVM, MA is the Director for the Program on Health Data and Common Methods. She has a doctorate in veterinary medicine and 15 years of experience with data analysis and database planning, design and implementation. Dr. Popovic's background is in health informatics, fusing clinical science and data science. She was a key player in the FDA Sentinel project.
- Alan Blatecky, MBA, is the former director of the Office of Cyberinfrastructure at the National Science Foundation. At RTI, he serves as a senior advisor for advanced data technologies and cyberinfrastructure capabilities.

External reviewers:

- Mark Frisse MD, MS is the Accenture Professor of Biomedical Informatics in the School of Medicine at Vanderbilt University. His work focuses on the intersection between health care informatics, economics, policy, and health care transformation. His primary research is directed toward an understanding of economic sustainability and toward the development of technical and administrative measures to enable effective care coordination and to ensure the integrity of security and privacy efforts.
- John Glaser, PhD, is the senior vice president of Population Health. Dr. Glaser is focused on driving Cerner's population health technology and product strategies, interoperability and government policy development. He impacts clients by devoting his career to advancing health care through innovation and commits to helping clients maximize their investment in health care information technology.
- Maryan Zirkle, MD, MS, MA, is a Senior Program Officer on the Research Infrastructure team at the Patient-Centered Outcomes Research Institute (PCORI). She is responsible for providing intellectual and organizational support for the development and regulation of PCORI's National Patient-Centered Clinical Data Research Network.

APPENDIX D: STANDARD OPERATING PROCEDURES FOR CODING

1. Coding Workflow and Guidelines

1.1. Open the project's Statement of Work (SOW) source document.

1.2. Code text offering background or context on the project to the pre-established 'Project Background' node for that project.

1.2.1. Add sub-nodes under Project Background to reflect the goals of the project. This will help you parse the information into relevant sections/categories for coding each project goal.

1.3. Create a node for each project goal discussed in the SOW and code text pertaining to each goal to the appropriate 'Goal' node.

1.3.1. When creating the 'Goal' node, attempt to limit the name of the goal to 5-7 words.

1.3.2. Enter a brief, clear statement of the goal in the 'Description' field for that node.

1.4. Create a node for each project activity discussed in the SOW under the related project goal node and code text that describes/details of each activity to the appropriate 'Activity' node.

1.4.1. Coders should define "activities" loosely and code details about tasks and sub-tasks at the 'Activity' level rather than creating sub-nodes for more granular pieces of activities.

1.4.2. To the extent that deliverables are produced for project activities or report progress or results of project activities, they should be coded under the associated project activities rather than being treated as a separate activity.

1.4.3. When coders identify discrepancies between 'Project Goals' and actual project activities (i.e., scope drift or change), coders should send specific details to the Project Manager to add to agenda for next call with ASPE.

1.5. For each activity, determine which developmental components (Developmental components) the activity contributes to accomplishing. Developmental components should be selected only if the activity contributes to accomplishing them in a **literal, not conceptual** sense. Copy the relevant developmental component nodes from the 'developmental component Library' under the contributing 'Activity' node, and code text that offers evidence for the activity's contribution to or support of the selected developmental component to the 'developmental component' node.

1.5.1. Coders will code as many Developmental components as are applicable.

1.5.2. If a developmental component contributes to accomplishing any part of a project, code it as such.

1.5.3. Coders will err on the side of only coding to a developmental component if it is a "full fit." A full-fit exists when text directly supports an intent to achieve, or the actual achievement of a specific developmental component. Text that describes the infrastructure necessary to achieve a developmental component is not a full-fit. [Examples of "full" and "marginal" fits to be identified and added.]

1.5.4. If an activity does not map to any of the Developmental components in the 'developmental component Library,' coders will code to the function-less, component-less "Does not map to existing Developmental components" node. There should not be any Developmental components associated with an activity

coded to the "Does not map" node. More than one activity may be coded to the "Does not map" code for a single project.

1.5.4.1. There are 2 "Does not map to existing Developmental components" nodes from which to choose:

1.5.4.1.1. "43 -- Does not map to existing Developmental components – Administrative"

1.5.4.1.2. "44 -- Does not map to existing Developmental components - Possible developmental component"

1.5.4.2. Coders will create Annotations [following instructions provided in Google doc] as appropriate providing contextual information explaining why the activity was problematic to map to be presented during the coding review for further discussion and decision-making about how to code (or not code).

1.5.5. Coders will not add any developmental component case nodes, but rather send the Project Manager an email suggesting additional codes for consideration.

1.5.6. The Project Manager will bring suggested code additions to the attention of Project Leadership at weekly administrative meetings (standing agenda item) or if more urgent, via email.

1.6. Repeat process for all other project sources.

1.6.1. If text related to goals identified from reviewing SOW is found in other project documents, only code it to the Goal node if it is not duplicative of the previous text. In other words, if it is simply a restatement of the same goal and the goal hasn't drifted or changed, it does not need to be recoded.

1.6.2. Coders should scan progress reports to see if they are comprehensive and cumulative, in which case only the most recent progress report needs to be coded.

1.6.3. Progress reports may contain links to other documents, such as deliverables. If it is not possible to determine the status of activities identified through existing documents or to determine whether mapping to a developmental component is appropriate, then evaluate whether the linked document contains useful information for the project. If the information is useful, then the coder can import the document into their individual DB (basic instructions for importing provided separately by Task Leader by email) (with Project Manager's assistance as needed), and coded only that information which is necessary to support the Developmental components or status of the activity.

1.6.3.1. Imported documents should include "imported by [coder Initials] [date of import]" at the end of the file name (e.g., 13-006_Deliverable X_imported by YB 1.20.17).

1.6.3.2. Imported documents should be added to the project folder on the share in a subfolder entitled "Project Docs Imported by Coder."

1.7. Review coded text for each 'Activity' node to assign status.

1.7.1. Coders will enter a single status code per activity per project.

1.7.2. For summative projects, coders should evaluate whether the deliverables were met to code achievement status.

1.8. Coders will create Memos [following instructions provided in Google doc] in a project to capture overall impressions about a project.

1.8.1. Naming convention for Memos: REMIS # -- Memo Title -- Coder Initials.

1.9. Upon completion of coding, the coder will participate in a meeting with the Review team to present and discuss the key findings from their coding.
   1.9.1. In advance of the meeting, the coder will prepare a bulleted list of challenges and questions encountered in coding the project's documents for discussing with the reviewers.
   1.9.2. Following the review meeting, the coder will revise the coding in their DB as needed to reflect any changes agreed upon with the Review team.

2. Coding Inclusion/Exclusion Criteria

2.1. Always code at the sentence-level (ensuring there is sufficient context for later interpretation).
2.2. Coders will not code text that is operational/administrative in nature.
2.3. For PCOR projects that build upon other non-PCOR projects, coders will only focus on the PCOR project (i.e., will not seek out or review documents from the non-PCOR project).
   2.3.1 Coders will not make assumptions about prior work (PCOR or non-PCOR).
2.4. We want to determine Trust Fund progress as opposed to more general progress. If a project supports non-PCOR work as well as what ASPE funded them to do, coders will only focus on the ASPE-funded piece.
2.5. We will only code to what they actually ended up doing, not what they said they'd do. As we review documents, those from later in the project are the best indicators of what they actually got done.
2.6. We will only note a contribution to a developmental component for standard services, i.e., a service that utilizes one or more recognized, current (at the time it is being performed) standards.
2.7. If the project is about setting up broad-based structures relevant for many if not all clinical conditions, and the project references one particular clinical condition as an example or test, the example or test should not be coded to a developmental component related to that condition.
2.8. We will not code *internal* governance (governance of the project) but we do want to code *external* governance (governance of external entities, i.e. HL7 balloting).

3. Addressing Questions that Arise During Coding

3.1. When coders experience questions related to the use of NVivo or the structure of the DB, they should email the Project Manager and Task Leader.
3.2. Coders will create Annotations [following instructions provided in Google doc] in a project to capture questions or brief comments they have about specific text, which should be presented during the coding review for further discussion and decision-making about how to code (or not code).
3.3. The coding team will meet on a regular basis, more often during the early part of the project, to discuss patterns, questions, logistical challenges, etc. that arise.
3.4. When coders find that they do not have the requisite expertise (or simply need advice) to code an activity in a project document, they should include this among the challenges and questions in the bulleted list they prepare in advance of the meeting with the Review team.
3.5. When coders determine that we may need to obtain additional project documentation from ASPE and/or hold an ad hoc call with the project team to determine how an activity maps to the Developmental components or determine

the status of an activity, they should populate a row in the 'Additional Info Needed to Code' spreadsheet in the Data Analysis share folder.

SOPs for Final NVivo Project Review

Important…

Please complete these steps ***before*** your scheduled call with the Review Committee. Email either of them if you have any questions.

1. Review Background Node and Goal(s) to ensure that each are populated with appropriate content

2. Review and confirm developmental component numbering is accurate, e.g. all Developmental components have their numbers

3. Review and confirm that each Activity points to at least one developmental component

4. Review and confirm the data in each classification spreadsheet (project, activity, developmental component)
    a. Review and Confirm there are NO 'unassigned' fields in the Activities classification sheet
    b. Only should have levels of achievement OR 'Not Applicable' (use 'Not Applicable' for all 'Does not map' Developmental components)
    c. Review and confirm that all activities have an assignment (are no zeroes)
    d. Confirm that all activity assignments are current (that they have not changed since the previous review)

5. Review and confirm that every Activity has "aggregate from child nodes" turned ON

6. Review and confirm that **Developmental components are mapped to completed actions** (not goals or intended work)
    a. Content must display the work that was done. *hint* Text is in past tense

7. Review all "does not map" assignments
    a. If "administrative" confirm that this still applies
    b. Review and confirm any "possible developmental component". Then in a MEMO, write what new developmental component you think is needed
    c. If you are still unsure about a "possible developmental component" then note that in a MEMO for discussion during the coding review

8. For all formative projects, compare functionalities and Developmental components assigned with information in latest progress report (new format)

9. For all projects, review NORC summarization in NORC Portfolio Report

10. You are DONE until the coding review. Congratulations and thanks!

[For Review Committee only: Confirm that all extraneous fields have been removed]
[For Review Committee only: When all above are APPROVED, Review Committee will enter 'COMPLETE' at bottom of MEMO]

APPENDIX E: ANALYSIS RESULTS

List of Projects included in the Document Review with Type, Goals, Activities, Activity Status, and Developmental components (Note: 12-007/13-004 are linked projects

Project	Type	Goal	Activity	Activity Status	Developmental Component
12-003 -- Maintenance and support of the Chronic Condition Warehouse for CER	F	N/A	N/A	N/A	N/A
12-004 -- MPCD Beta Test (Summative: CMS; 09/24/2012 - 09/15/2013)	S	Goal 1 -- Conduct beta testing of MPCD to evaluate usability and features	Activity 1.1 -- Modify database's web portal so that it uses a hidden website for access during beta release	N/A	N/A
	S	Goal 1 -- Conduct beta testing of MPCD to evaluate usability and features	Activity 1.2 -- Evaluate the experiences of users testing the beta release of the database	N/A	N/A
	S	Goal 1 -- Conduct beta testing of MPCD to evaluate usability and features	Activity 1.3 -- Perform 3 additional CER studies	N/A	N/A
12-007/13-004-NLM -- Infrastructure for Use of EHRs in Comparative Effectiveness Research/Data Infrastructure for Use in EHRs in Comparative Effectiveness Research (CER)	S	Goal 1 -- Require use of selected CDEs & pt assessment instruments in HHS & PCORI funded CER	Activity 1.1 -- Add new initiatives to NIH CDE Resource Portal	Fully Achieved	14 -- Policies for required use of CDEs across research and by EHR and health information exchange (HIE) vendors

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	S	Goal 1 -- Require use of selected CDEs & pt assessment instruments in HHS & PCORI funded CER	Activity 1.2 -- Help develop & deploy NIH guidance for using standard CDEs in PCOR funding announcements.	Fully Achieved	14 -- Policies for required use of CDEs across research and by EHR and health information exchange (HIE) vendors
	S	Goal 1 -- Require use of selected CDEs & pt assessment instruments in HHS & PCORI funded CER	Activity 1.3 -- Perform activities above w.NIH-wide committees & other stakeholders	Fully Achieved	15 -- Mechanisms to align policies and incentives across Health and Human Services (HHS) agencies
	S	Goal 2 -- Define CDEs using standard terminologies & value sets	Activity 2.1 -- Contribute to developmental component Initiative - ID CDEs	Fully Achieved	14 -- Policies for required use of CDEs across research and by EHR and health information exchange (HIE) vendors
	S	Goal 2 -- Define CDEs using standard terminologies & value sets	Activity 2.1 -- Contribute to Developmental component Initiative - ID CDEs	Fully Achieved	18--Service(s) that code elements and questions used to collect data for research to common clinical standards (e.g., SNOMED CT)
	S	Goal 2 -- Define CDEs using standard terminologies & value sets	Activity 2.1 -- Contribute to Developmental component Initiative - ID CDEs	Fully Achieved	19 -- CDE—value set creation and harmonization
	S	Goal 2 -- Define CDEs using standard terminologies & value sets	Activity 2.2 -- Contribute to Developmental component Initiative - ID eCRFs	Partially Achieved	24 -- Standards for forms using CDEs

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	S	Goal 3 -- Create infrastructure to maintain, distribute & use standard CDEs & pt assessment instruments	Activity 3.1 -- Implement design for repository of CDE resources	Partially Achieved	15 -- Mechanisms to align policies and incentives across Health and Human Services (HHS) agencies
	S	Goal 3 -- Create infrastructure to maintain, distribute & use standard CDEs & pt assessment instruments	Activity 3.1 -- Implement design for repository of CDE resources	Partially Achieved	16 -- Services for contribution and-or harmonization of CDEs
	S	Goal 3 -- Create infrastructure to maintain, distribute & use standard CDEs & pt assessment instruments	Activity 3.1 -- Implement design for repository of CDE resources	Partially Achieved	19 -- CDE—value set creation and harmonization
	S	Goal 3 -- Create infrastructure to maintain, distribute & use standard CDEs & pt assessment instruments	Activity 3.1 -- Implement design for repository of CDE resources	Partially Achieved	20 -- Alignment of clinical and research standards
	S	Goal 3 -- Create infrastructure to maintain, distribute & use standard CDEs & pt assessment instruments	Activity 3.2 -- Contribute to Developmental component initiative for auto-population of eCRFs by EHR systems	Partially Achieved	23 -- Standards for EHRs to interact with forms and forms libraries
	S	Goal 3 -- Create infrastructure to maintain, distribute & use standard CDEs & pt assessment instruments	Activity 3.1 -- Implement design for repository of CDE resources	Partially Achieved	24 -- Standards for forms using CDEs

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
12-007+13-004-ONC -- Infrastructure for Use of EHRs in Comparative Effectiveness Research + Development of Data Infrastructure for Use in EHRs in Comparative Effectiveness Research - Development of Meaningful Use Standards for CER Data Elements	S	Goal 1 -- Develop standardized mechanism enabling an EHR to capture structured data for a specified use case	Activity 1.1 -- Create use case document to help analyze tech standards needed to support interoperability between research & clinical data generated in health care setting	Fully Achieved	06 -- Services to support collection and extraction of data
	S	Goal 1 -- Develop standardized mechanism enabling an EHR to capture structured data for a specified use case	Activity 1.1 -- Create use case document to help analyze tech standards needed to support interoperability between research & clinical data generated in health care setting	Fully Achieved	20 -- Alignment of clinical and research standards
	S	Goal 1 -- Develop standardized mechanism enabling an EHR to capture structured data for a specified use case	Activity 1.3 -- Create implementation guide for tech standard so EHR vendors can integrate CDE-based data into EHRs	Fully Achieved	20 -- Alignment of clinical and research standards
	S	Goal 1 -- Develop standardized mechanism enabling an EHR to capture structured data for a specified use case	Activity 1.4 -- ID early adopters of tech standards to prep for pilots	Fully Achieved	20 -- Alignment of clinical and research standards
	S	Goal 1 -- Develop standardized mechanism enabling an EHR to capture structured data for a specified use case	Activity 1.1 -- Create use case document to help analyze tech standards needed to support interoperability between research & clinical data generated in health care setting	Fully Achieved	22 -- CDE representation standards

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	S	Goal 1 -- Develop standardized mechanism enabling an EHR to capture structured data for a specified use case	Activity 1.3 -- Create implementation guide for tech standard so EHR vendors can integrate CDE-based data into EHRs	Fully Achieved	22 -- CDE representation standards
	S	Goal 1 -- Develop standardized mechanism enabling an EHR to capture structured data for a specified use case	Activity 1.4 -- ID early adopters of tech standards to prep for pilots	Fully Achieved	22 -- CDE representation standards
	S	Goal 1 -- Develop standardized mechanism enabling an EHR to capture structured data for a specified use case	Activity 1.3 -- Create implementation guide for tech standard so EHR vendors can integrate CDE-based data into EHRs	Fully Achieved	23 -- Standards for EHRs to interact with forms and forms libraries
	S	Goal 1 -- Develop standardized mechanism enabling an EHR to capture structured data for a specified use case	Activity 1.4 -- ID early adopters of tech standards to prep for pilots	Fully Achieved	23 -- Standards for EHRs to interact with forms and forms libraries
	S	Goal 1 -- Develop standardized mechanism enabling an EHR to capture structured data for a specified use case	Activity 1.1 -- Create use case document to help analyze tech standards needed to support interoperability between research & clinical data generated in health care setting	Fully Achieved	24 -- Standards for forms using CDEs
	S	Goal 1 -- Develop standardized mechanism enabling an EHR to capture structured data for a specified use case	Activity 1.2 -- Summarize current landscape of available tech standards to develop eCRFs	Fully Achieved	24 -- Standards for forms using CDEs
	S	Goal 1 -- Develop standardized mechanism enabling an EHR to capture structured data for a specified use case	Activity 1.3 -- Create implementation guide for tech standard so EHR vendors can integrate CDE-based data into EHRs	Fully Achieved	24 -- Standards for forms using CDEs

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	S	Goal 1 -- Develop standardized mechanism enabling an EHR to capture structured data for a specified use case	Activity 1.4 -- ID early adopters of tech standards to prep for pilots	Fully Achieved	24 -- Standards for forms using CDEs
	S	Goal 2 -- Create infrastructure to maintain, distribute & use CDEs	Activity 2.1 -- Web portal of CDEs and standardized assessment instruments	Fully Achieved	14 -- Policies for required use of CDEs across research and by EHR and health information exchange (HIE) vendors
	S	Goal 2 -- Create infrastructure to maintain, distribute & use CDEs	Activity 2.1 -- Web portal of CDEs and standardized assessment instruments	Fully Achieved	15 -- Mechanisms to align policies and incentives across Health and Human Services (HHS) agencies
	S	Goal 2 -- Create infrastructure to maintain, distribute & use CDEs	Activity 2.1 -- Web portal of CDEs and standardized assessment instruments	Fully Achieved	19 -- CDE—value set creation and harmonization
	S	Goal 2 -- Create infrastructure to maintain, distribute & use CDEs	Activity 2.1 -- Web portal of CDEs and standardized assessment instruments	Fully Achieved	20 -- Alignment of clinical and research standards
12-008 -- CER Inventory	S	Goal 1 -- Maintain CER Inventory	Activity 1.1 -- Continue designing a system for categorization and cataloging of CER	N/A	N/A
	S	Goal 1 -- Maintain CER Inventory	Activity 1.2 -- Test and understand the performance of the inventory	N/A	N/A

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Project	Type	Goal	Activity	Activity Status	Developmental Component
13-002-CMS - Maintenance and Support of the Chronic Condition Warehouse for Comparative Effectiveness Research	S	Goal 1 -- Provide Access, Maintenance and Licensing for CCW PCOR Enhancements	Activity 1.3 -- Support CER (PCOR) Projects	Fully Achieved	39 -- Services to access and use public data sources
	S	Goal 1 -- Provide Access, Maintenance and Licensing for CCW PCOR Enhancements	Activity 1.1 -- Develop New SOW	Not Applicable	43 -- Does not map to existing Developmental components - Administrative
	S	Goal 1 -- Provide Access, Maintenance and Licensing for CCW PCOR Enhancements	Activity 1.2 -- Renewal of licenses and maintenance of infrastructure	Not Applicable	43 -- Does not map to existing Developmental components - Administrative
13-003 -- Creating the Foundational Blocks for the Learning Health Care System - Structured Data Capture (Formative; ONC; 1/27/14-9/30/17)	F	Goal 1 -- Identify, Develop, Pilot, and Ballot standards for CDEs	Activity 1.4 -- Develop, select, validate, pilot, and ballot standards for CDE and eCRF	Fully Achieved	06 -- Services to support collection and extraction of data
	F	Goal 1 -- Identify, Develop, Pilot, and Ballot standards for CDEs	Activity 1.4 -- Develop, select, validate, pilot, and ballot standards for CDE and eCRF	Fully Achieved	22 -- CDE representation standards
	F	Goal 1 -- Identify, Develop, Pilot, and Ballot standards for CDEs	Activity 1.4 -- Develop, select, validate, pilot, and ballot standards for CDE and eCRF	Fully Achieved	23 -- Standards for EHRs to interact with forms and forms libraries

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	F	Goal 1 -- Identify, Develop, Pilot, and Ballot standards for CDEs	Activity 1.4 -- Develop, select, validate, pilot, and ballot standards for CDE and eCRF	Fully Achieved	24 -- Standards for forms using CDEs
	F	Goal 1 -- Identify, Develop, Pilot, and Ballot standards for CDEs	Activity 1.4 -- Develop, select, validate, pilot, and ballot standards for CDE and eCRF	Fully Achieved	28 -- Patient matching standards (includes standard attributes for patient matching and standardizing algorithms for patient matching)
	F	Goal 1 -- Identify, Develop, Pilot, and Ballot standards for CDEs	Activity 1.4 -- Develop, select, validate, pilot, and ballot standards for CDE and eCRF	Fully Achieved	41 -- Cancer registry data
	F	Goal 1 -- Identify, Develop, Pilot, and Ballot standards for CDEs	Activity 1.1 -- Environmental scan of existing technical standards for structured data capture	Fully Achieved	43 -- Does not map to existing Developmental components - Administrative
	F	Goal 1 -- Identify, Develop, Pilot, and Ballot standards for CDEs	Activity 1.2 -- Create use case document to guide structured data capture efforts	Fully Achieved	44 -- Does not map to existing Developmental components - Possible developmental component

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Project	Type	Goal	Activity	Activity Status	Developmental Component
	F	Goal 1 -- Identify, Develop, Pilot, and Ballot standards for CDEs	Activity 1.3 -- Develop Draft Implementation Guide	Fully Achieved	44 -- Does not map to existing Developmental components - Possible developmental component
13-006 -- Expanding Data Collection Infrastructure of the National Program of Cancer Registries for Comparative Effectiveness Research (Summative; Developmental component; 4/8/13-8/31/15)	S	Goal 1 -- Expand data collection infrastructure of the National Program of Cancer Registries (NPCR)	Activity 1.1 -- Enhance specialized cancer registries	Fully Achieved	41 -- Cancer registry data
	S	Goal 1 -- Expand data collection infrastructure of the National Program of Cancer Registries (NPCR)	Activity 1.2 -- Expand EHR reporting to cancer registries	Fully Achieved	41 -- Cancer registry data
13-008 -- Strategic Opportunities for Building Data Infrastructure for CER	S	N/A	N/A	N/A	N/A
14-009 - Strengthening and Expanding Community Health Applied Research Network (CHARN) Registry to Conduct Patient-Centered Outcomes Research	S	Goal 1 -- Build Data and Research Capacity for PCOR in the Safety Net	Activity 1.1 --Create readily usable de-identified analytic data sets	Fully Achieved	11 -- Standards to answer practice and population-level questions using electronic clinical data

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	S	Goal 1 -- Build Data and Research Capacity for PCOR in the Safety Net	Activity 1.3 -- Maintain the infrastructure for PCOR and Quality Improvement in the Safety Net	Fully Achieved	13 -- Governance structure for contribution and/or harmonization of common data elements (CDEs)
	S	Goal 1 -- Build Data and Research Capacity for PCOR in the Safety Net	Activity 1.1 --Create readily usable de-identified analytic data sets	Fully Achieved	42 -- Safety-net clinical registry data
	S	Goal 1 -- Build Data and Research Capacity for PCOR in the Safety Net	Activity 1.2 -- Establish a process for investigators to access CHARN Data	Fully Achieved	42 -- Safety-net clinical registry data
14-012 -- Creating the Foundational Blocks for the Learning Health Care System - Data Access Standards for Electronic Health Records (Summative; ONC; 11/1/13-9/30/16)	S	Goal 1 - Develop Local Access API Standards	Activity 1.3 - Community-developed reference implementation and the testing tools (through feedback from pilots) for The Local Access API initiative	Fully Achieved	06 -- Services to support collection and extraction of data
	S	Goal 1 - Develop Local Access API Standards	Activity 1.1 - Analysis of existing standards and drafting initial IGs for Local Access	Fully Achieved	12 -- Standards for needed application programming interfaces (APIs)
	S	Goal 1 - Develop Local Access API Standards	Activity 1.2 - Ballot process for standards for The Local Access API initiative	Fully Achieved	12 -- Standards for needed application programming interfaces (APIs)

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	S	Goal 1 - Develop Local Access API Standards	Activity 1.3 - Community-developed reference implementation and the testing tools (through feedback from pilots) for The Local Access API initiative	Fully Achieved	12 -- Standards for needed application programming interfaces (APIs)
	S	Goal 2 - Develop Secure Stakeholder Access Standards	Activity 2.3 - Community-developed reference implementation and the testing tools (through feedback from pilots) for The Secure Stakeholder Access Initiative	Fully Achieved	06 -- Services to support collection and extraction of data
	S	Goal 2 - Develop Secure Stakeholder Access Standards	Activity 2.1 - Analysis of existing standards and drafting initial IGs for Secure Stakeholder Access	Fully Achieved	12 -- Standards for needed application programming interfaces (APIs)
	S	Goal 2 - Develop Secure Stakeholder Access Standards	Activity 2.2 - Ballot process standards for The Secure Stakeholder Access Initiative	Fully Achieved	12 -- Standards for needed application programming interfaces (APIs)
	S	Goal 2 - Develop Secure Stakeholder Access Standards	Activity 2.3 - Community-developed reference implementation and the testing tools (through feedback from pilots) for The Secure Stakeholder Access Initiative	Fully Achieved	12 -- Standards for needed application programming interfaces (APIs)

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Project	Type	Goal	Activity	Activity Status	Developmental Component
	S	Goal 3 - Develop Standards for Distributed Access Initiative	Activity 3.3 - Community-developed reference implementation and the testing tools (through feedback from pilots) for The Distributed Access initiative	Fully Achieved	06 -- Services to support collection and extraction of data
	S	Goal 3 - Develop Standards for Distributed Access Initiative	Activity 3.1 - Analysis of existing standards and drafting initial IGs for Distributed Access	Fully Achieved	12 -- Standards for needed application programming interfaces (APIs)
	S	Goal 3 - Develop Standards for Distributed Access Initiative	Activity 3.2 - Ballot standards for The Distributed Access initiative	Fully Achieved	12 -- Standards for needed application programming interfaces (APIs)
	S	Goal 3 - Develop Standards for Distributed Access Initiative	Activity 3.3 - Community-developed reference implementation and the testing tools (through feedback from pilots) for The Distributed Access initiative	Fully Achieved	12 -- Standards for needed application programming interfaces (APIs)
15-003 -- Improving Beneficiary Access to Health Information "Blue Button" to Enable a 'Data-as Service' Platform (Summative; CMS; 10/01/14 - 06/30/16)	S	Goal 1 -- Improve Access to Health Information via Blue Button Application	Activity 1.2 -- Build a Data-as-a-Service prototype	Fully Achieved	06 -- Services to support collection and extraction of data
	S	Goal 1 -- Improve Access to Health Information via Blue Button Application	Activity 1.1 -- Implement Data Transformation for Blue Button Data	Fully Achieved	07 -- Services for structuring unstructured data

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	S	Goal 1 -- Improve Access to Health Information via Blue Button Application	Activity 1.3 -- Enhance Security	Fully Achieved	34 -- Secure services to collect PPI
	S	Goal 1 -- Improve Access to Health Information via Blue Button Application	Activity 1.2 -- Build a Data-as-a-Service prototype	Fully Achieved	35 -- Standards for collection of PPI
	S	Goal 1 -- Improve Access to Health Information via Blue Button Application	Activity 1.4 -- Developer Outreach	Not Applicable	43 -- Does not map to existing Developmental components - Administrative
	S	Goal 1 -- Improve Access to Health Information via Blue Button Application	Activity 1.5 -- Beneficiary Outreach	Not Applicable	43 -- Does not map to existing Developmental components - Administrative
15-012 -- Improving the Mortality Data Infrastructure for Patient-Centered Outcomes	F	Goal 1 -- Improve mortality data infrastructure via timely delivery of death records and linkage with NHCS	Activity 1.3 -- Pilot linkage of NHCS data with NDI data	Fully Achieved	26 -- Policies to enable patient matching - record linkages to occur under existing laws
	F	Goal 1 -- Improve mortality data infrastructure via timely delivery of death records and linkage with NHCS	Activity 1.2 -- Pilot draft standards for electronic exchange of death info from EHRs to EDRS	Partially Achieved	11 -- Standards to answer practice and population-level questions using electronic clinical data
	F	Goal 1 -- Improve mortality data infrastructure via timely delivery of death records and linkage with NHCS	Activity 1.2 -- Pilot draft standards for electronic exchange of death info from EHRs to EDRS	Partially Achieved	22 -- CDE representation standards

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	F	Goal 1 -- Improve mortality data infrastructure via timely delivery of death records and linkage with NHCS	Activity 1.1 -- Strengthen mortality data infrastructure of states and NCHS-DVS	Partially Achieved	40 -- Services to enhance the timeliness of vital statistics data
15-013 -- Utilizing Data from Various Data Partners in a Distributed Manner	F	Goal 1 -- Enable distributed regression analysis	Activity 1.3 -- Provide technical and user documentation	Not Applicable	43 -- Does not map to existing Developmental components - Administrative
	F	Goal 1 -- Enable distributed regression analysis	Activity 1.4 -- Determine potential solutions for vertically partitioned data	Not Started	09 -- Analytical services that support system-level results (network-based or population level)
	F	Goal 1 -- Enable distributed regression analysis	Activity 1.1 -- Develop software application to automate distributed regression analysis	Partially Achieved	09 -- Analytical services that support system-level results (network-based or population level)
	F	Goal 1 -- Enable distributed regression analysis	Activity 1.2 -- Test distributed regression analysis application in a distributed research network	Partially Achieved	09 -- Analytical services that support system-level results (network-based or population level)
15-014 -- Cross-Network Directory Service	F	Goal 1 -- Develop and Implement A Cross-Network Directory Service	Activity 1.2 -- Develop and test detailed design for CNDS	Fully Achieved	06 -- Services to support collection and extraction of data

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	F	Goal 1 -- Develop and Implement A Cross-Network Directory Service	Activity 1.1 -- Identify key functionalities and technical design for CNDS	Fully Achieved	43 -- Does not map to existing Developmental components - Administrative
	F	Goal 1 -- Develop and Implement A Cross-Network Directory Service	Activity 1.3 -- Release CNDS and conduct additional analyses and produce user materials	Partially Achieved	06 -- Services to support collection and extraction of data
	F	Goal 1 -- Develop and Implement A Cross-Network Directory Service	Activity 1.3 -- Release CNDS and conduct additional analyses and produce user materials	Partially Achieved	39 -- Services to access and use public data sources
15-015 -- Collection of Patient-Provided Information through a Mobile Device Application for Use in Comparative Effectiveness and Drug Safety Research	F	Goal 1 -- Enable collection of patient-provided health data for linkage with existing data for use in research	Activity 1.1 -- Develop a cohort of eligible subjects	Not Applicable	43 -- Does not map to existing Developmental components - Administrative
	F	Goal 1 -- Enable collection of patient-provided health data for linkage with existing data for use in research	Activity 1.2 -- Select data elements and develop infrastructure	Not Applicable	43 -- Does not map to existing Developmental components - Administrative
	F	Goal 1 -- Enable collection of patient-provided health data for linkage with existing data for use in research	Activity 1.4 -- Link data provided by patients with existing data	Not Started	34 -- Secure services to collect PPI

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	F	Goal 1 -- Enable collection of patient-provided health data for linkage with existing data for use in research	Activity 1.3 -- Develop application interface	Partially Achieved	33 -- Services for patients and their designated proxies (e.g., caregivers) to contribute directly to research databases
	F	Goal 1 -- Enable collection of patient-provided health data for linkage with existing data for use in research	Activity 1.3 -- Develop application interface	Partially Achieved	34 -- Secure services to collect PPI
15-016 -- Conceptualizing a Data Infrastructure of the Capture and Use of Patient-Generated Health Data (Formative; ONC; 6/16/15-6/30/18)	F	Goal 1 -- Advance patient engagement in research by increasing capability to utilize PGHD in research & health care delivery	Activity 1.1 -- Develop policy framework addressing 7 areas	Partially Achieved	31 -- Policies for incorporating PPI into clinical research
	F	Goal 1 -- Advance patient engagement in research by increasing capability to utilize PGHD in research & health care delivery	Activity 1.2 -- Conduct pilots to test concepts & implementation of policy framework	Partially Achieved	31 -- Policies for incorporating PPI into clinical research
	F	Goal 1 -- Advance patient engagement in research by increasing capability to utilize PGHD in research & health care delivery	Activity 1.2 -- Conduct pilots to test concepts & implementation of policy framework	Partially Achieved	36 -- Standards for PPI (e.g., mobile device wearables)
	F	Goal 1 -- Advance patient engagement in research by increasing capability to utilize PGHD in research & health care delivery	Activity 1.2 -- Conduct pilots to test concepts & implementation of policy framework	Partially Achieved	37 -- Standards for integrating PPI with electronic health record data

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	F	Goal 1 -- Advance patient engagement in research by increasing capability to utilize PGHD in research & health care delivery	Activity 1.2 -- Conduct pilots to test concepts & implementation of policy framework	Partially Achieved	38 -- Standards for personal medical device data
15-018 -- Security and Privacy Standards for Patient Matching, Linking and Aggregation; Formative; ONC; 6/16/15 - 9/30/18	F	Goal 1 -- Improve data quality, standardize attributes and improve algorithm match rates	Activity 1.1 -- Identify and align the recommendations of the recent ONC Patient Matching report out with ONC...	Not Applicable	43 -- Does not map to existing Developmental components - Administrative
	F	Goal 1 -- Improve data quality, standardize attributes and improve algorithm match rates	Activity 1.3 -- Catalog and determine the feasibility of using current and potential standards, algorithm and emerging techniques...	Not Applicable	43 -- Does not map to existing Developmental components - Administrative
	F	Goal 1 -- Improve data quality, standardize attributes and improve algorithm match rates	Activity 1.5 -- Patient Matching Challenge AND Community of Practice	Partially Achieved	17 -- Services to allow members of the research community to "voice" value for specific standardized collection components and, in turn, discover value expressed by others
	F	Goal 1 -- Improve data quality, standardize attributes and improve algorithm match rates	Activity 1.4 -- Test and tune multiple patient matching algorithms against data sets representative of the PCOR environment (i.e. claims, clinical research)	Partially Achieved	20 -- Alignment of clinical and research standards

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	F	Goal 1 -- Improve data quality, standardize attributes and improve algorithm match rates	Activity 1.5 -- Patient Matching Challenge AND Community of Practice	Partially Achieved	20 -- Alignment of clinical and research standards
	F	Goal 1 -- Improve data quality, standardize attributes and improve algorithm match rates	Activity 1.2 -- Develop Patient Matching Use Cases as it applies to both the clinical and research community	Partially Achieved	27 -- Define use cases for patient matching and define currently available methods that work best for each use case
	F	Goal 1 -- Improve data quality, standardize attributes and improve algorithm match rates	Activity 1.4 -- Test and tune multiple patient matching algorithms against data sets representative of the PCOR environment (i.e. claims, clinical research)	Partially Achieved	28 -- Patient matching standards (includes standard attributes for patient matching and standardizing algorithms for patient matching)
	F	Goal 1 -- Improve data quality, standardize attributes and improve algorithm match rates	Activity 1.5 -- Patient Matching Challenge AND Community of Practice	Partially Achieved	28 -- Patient matching standards (includes standard attributes for patient matching and standardizing algorithms for patient matching)
	F	Goal 2 -- Create an open-source visual tool for patient matching and aggregation	Activity 2.1 -- Tool Specifications Document - Design and Component Development v.1	Fully Achieved	44 -- Does not map to existing Developmental components - Possible developmental component

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	F	Goal 2 -- Create an open-source visual tool for patient matching and aggregation	Activity 2.2 -- Deliver integration with PS API and NPPES v.2	Not Started	44 -- Does not map to existing Developmental components - Possible developmental component
	F	Goal 2 -- Create an open-source visual tool for patient matching and aggregation	Activity 2.3 -- Distribute Version 2 to CHARN and other partners	Partially Achieved	44 -- Does not map to existing Developmental components - Possible developmental component
	F	Goal 2 -- Create an open-source visual tool for patient matching and aggregation	Activity 2.4 – Pilots	Partially Achieved	44 -- Does not map to existing Developmental components - Possible developmental component
	F	Goal 3 -- Define the security layer...necessary to protect Open APIs and facilitate the aggregation of data from multiple data sources	Activity 3.1 -- Define profiles and reference implementations	Fully Achieved	12 -- Standards for needed application programming interfaces (APIs)

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	F	Goal 3 -- Define the security layer...necessary to protect Open APIs and facilitate the aggregation of data from multiple data sources	Activity 3.1 -- Define profiles and reference implementations	Fully Achieved	29 -- Standards and methods to privately and securely link and aggregate clinical data to determine eligibility for research studies, according to patient preferences (consent decisions)
	F	Goal 3 -- Define the security layer...necessary to protect Open APIs and facilitate the aggregation of data from multiple data sources	Activity 3.2 -- Initiate pilots and document lessons learned	Partially Achieved	12 -- Standards for needed application programming interfaces (APIs)
	F	Goal 3 -- Define the security layer...necessary to protect Open APIs and facilitate the aggregation of data from multiple data sources	Activity 3.2 -- Initiate pilots and document lessons learned	Partially Achieved	17 -- Services to allow members of the research community to "voice" value for specific standardized collection components and, in turn, discover value expressed by others

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	F	Goal 3 -- Define the security layer...necessary to protect Open APIs and facilitate the aggregation of data from multiple data sources	Activity 3.2 -- Initiate pilots and document lessons learned	Partially Achieved	29 -- Standards and methods to privately and securely link and aggregate clinical data to determine eligibility for research studies, according to patient preferences (consent decisions)
	F	Goal 4 -- Include Clinical Data Research Networks and their nodes in the piloting and testing of the proposed standards and services	Activity 4.1 -- Initiate pilots and document lessons learned	Not Applicable	43 -- Does not map to existing Developmental components - Administrative
	F	Goal 5 -- Integrate with the National Plan and Provider Enumeration System (NPPES)	Activity 5.1 -- Develop a standards-compliant interface to the NPPES that would allow provider information to be retrieved and updated in a timely manner	Fully Achieved	09 -- Analytical services that support system-level results (network-based or population level)
	F	Goal 5 -- Integrate with the National Plan and Provider Enumeration System (NPPES)	Activity 5.1 -- Develop a standards-compliant interface to the NPPES that would allow provider information to be retrieved and updated in a timely manner	Fully Achieved	12 -- Standards for needed application programming interfaces (APIs)

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
15-019-Cdevelopmental component -- PCOR Privacy and Security Blueprint, Legal Analysis and Ethics Framework for Data Use & Use of Technology for Privacy	F	Goal 1 -- Provide recommendations for a framework based on public health legal & ethical analysis	Activity 1.1 -- Develop Legal & Ethical Implications for PCOR Data Use report	Partially Achieved	01 -- Privacy and security policies for querying and accessing clinical data by researchers conducting PCOR
	F	Goal 1 -- Develop a privacy and security data infrastructure blueprint and legal analysis	Activity 1.1 -- Prioritization of PCOR use cases, data flows, and type and purpose of data	Fully Achieved	44 -- Does not map to existing Developmental components - Possible developmental component
	F	Goal 1 -- Develop a privacy and security data infrastructure blueprint and legal analysis	Activity 1.2 -- Develop a PCOR Legal Analysis and Ethics Framework for PCOR Pilots and Projects	Partially Achieved	01 -- Privacy and security policies for querying and accessing clinical data by researchers conducting PCOR
	F	Goal 1 -- Develop a privacy and security data infrastructure blueprint and legal analysis	Activity 1.4 -- Establish a Privacy, Security, and Ethical Legal Analysis Framework	Partially Achieved	01 -- Privacy and security policies for querying and accessing clinical data by researchers conducting PCOR
	F	Goal 1 -- Develop a privacy and security data infrastructure blueprint and legal analysis	Activity 1.3 -- Landscape Analysis of Consent Technology for Research	Partially Achieved	04 -- Consent standards

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	F	Goal 1 -- Develop a privacy and security data infrastructure blueprint and legal analysis	Activity 1.4 -- Establish a Privacy, Security, and Ethical Legal Analysis Framework	Partially Achieved	04 -- Consent standards
	F	Goal 1 -- Develop a privacy and security data infrastructure blueprint and legal analysis	Activity 1.4 -- Establish a Privacy, Security, and Ethical Legal Analysis Framework	Partially Achieved	15 -- Mechanisms to align policies and incentives across Health and Human Services (HHS) agencies
16-002 -- Improving Beneficiary Access to their Health Information through an Enhanced Blue Button Service	F	Goal 1 -- Enable researchers to access beneficiary data by implementing BBonFHIR, which allows beneficiaries to connect their data with applications and services	Activity 1.1 -- Develop the BBonFHIR service	Fully Achieved	06 -- Services to support collection and extraction of data
	F	Goal 1 -- Enable researchers to access beneficiary data by implementing BBonFHIR, which allows beneficiaries to connect their data with applications and services	Activity 1.1 -- Develop the BBonFHIR service	Fully Achieved	12 -- Standards for needed application programming interfaces (APIs)
	F	Goal 1 -- Enable researchers to access beneficiary data by implementing BBonFHIR, which allows beneficiaries to connect their data with applications and services	Activity 1.1 -- Develop the BBonFHIR service	Fully Achieved	33 -- Services for patients and their designated proxies (e.g., caregivers) to contribute directly to research databases

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	F	Goal 1 -- Enable researchers to access beneficiary data by implementing BBonFHIR, which allows beneficiaries to connect their data with applications and services	Activity 1.1 -- Develop the BBonFHIR service	Fully Achieved	34 -- Secure services to collect PPI
	F	Goal 1 -- Enable researchers to access beneficiary data by implementing BBonFHIR, which allows beneficiaries to connect their data with applications and services	Activity 1.2 -- Pilot the BBonFHIR service	Fully Achieved	34 -- Secure services to collect PPI
	F	Goal 1 -- Enable researchers to access beneficiary data by implementing BBonFHIR, which allows beneficiaries to connect their data with applications and services	Activity 1.1 -- Develop the BBonFHIR service	Fully Achieved	35 -- Standards for collection of PPI
	F	Goal 1 -- Enable researchers to access beneficiary data by implementing BBonFHIR, which allows beneficiaries to connect their data with applications and services	Activity 1.2 -- Pilot the BBonFHIR service	Fully Achieved	35 -- Standards for collection of PPI
	F	Goal 1 -- Enable researchers to access beneficiary data by implementing BBonFHIR, which allows beneficiaries to connect their data with applications and services	Activity 1.4 -- Document data formats and software code for publication	Not Applicable	43 -- Does not map to existing Developmental components - Administrative

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	F	Goal 1 -- Enable researchers to access beneficiary data by implementing BBonFHIR, which allows beneficiaries to connect their data with applications and services	Activity 1.5 -- Promote the availability of BBonFHIR to external sources	Not Applicable	43 -- Does not map to existing Developmental components - Administrative
	F	Goal 1 -- Enable researchers to access beneficiary data by implementing BBonFHIR, which allows beneficiaries to connect their data with applications and services	Activity 1.3 -- Launch full production of the BBonFHIR service	Partially Achieved	06 -- Services to support collection and extraction of data
	F	Goal 1 -- Enable researchers to access beneficiary data by implementing BBonFHIR, which allows beneficiaries to connect their data with applications and services	Activity 1.3 -- Launch full production of the BBonFHIR service	Partially Achieved	33 -- Services for patients and their designated proxies (e.g., caregivers) to contribute directly to research databases
	F	Goal 1 -- Enable researchers to access beneficiary data by implementing BBonFHIR, which allows beneficiaries to connect their data with applications and services	Activity 1.3 -- Launch full production of the BBonFHIR service	Partially Achieved	34 -- Secure services to collect PPI
16-003-CDC-- developmental component -- Development of a Natural Language Processing (NLP) Web Service for Public Health Use	F	Goal 1 -- Develop a Natural Language Processing (NLP) Web Service	Activity 1.1 -- Pilot implementation of NLP Web Service	Partially Achieved	06 -- Services to support collection and extraction of data

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	F	Goal 1 -- Develop a Natural Language Processing (NLP) Web Service	Activity 1.1 -- Pilot implementation of NLP Web Service	Partially Achieved	07 -- Services for structuring unstructured data
	F	Goal 1 -- Develop a Natural Language Processing (NLP) Web Service	Activity 1.1 -- Pilot implementation of NLP Web Service	Partially Achieved	39 -- Services to access and use public data sources
16-003-FDA -- Development of a Natural Language Processing (NLP) Web Service for Public Health Use	F	Goal 1 -- Develop a Natural Language Processing (NLP) Web Service to Convert Unstructured Data	Activity 1.1 -- Pilot Implementation of NLP Web Service	Partially Achieved	06 -- Services to support collection and extraction of data
	F	Goal 1 -- Develop a Natural Language Processing (NLP) Web Service to Convert Unstructured Data	Activity 1.1 -- Pilot Implementation of NLP Web Service	Partially Achieved	07 -- Services for structuring unstructured data
	F	Goal 1 -- Develop a Natural Language Processing (NLP) Web Service to Convert Unstructured Data	Activity 1.1 -- Pilot Implementation of NLP Web Service	Partially Achieved	39 -- Services to access and use public data sources
16-008 -- Precision Medicine Informatics	F	Goal 1 -- Develop core data policies for the PMI National Cohort	Activity 1.3 -- Disseminate hierarchy within the main LOINC release distribution	Partially Achieved	11 -- Standards to answer practice and population-level questions using electronic clinical data
	F	Goal 1 -- Develop core data policies for the PMI National Cohort	Activity 1.2 -- Develop clinically-relevant roll-up hierarchy for LOINC terms	Partially Achieved	19 -- CDE—value set creation and harmonization
	F	Goal 1 -- Develop core data policies for the PMI National Cohort	Activity 1.3 -- Disseminate hierarchy within the main LOINC release distribution	Partially Achieved	19 -- CDE—value set creation and harmonization

(continued)

Project	Type	Goal	Activity	Activity Status	Developmental Component
	F	Goal 1 -- Develop core data policies for the PMI National Cohort	Activity 1.1 -- Identify high priority content for representing new LOINC hierarchy	Partially Achieved	22 -- CDE representation standards
	F	Goal 1 -- Develop core data policies for the PMI National Cohort	Activity 1.2 -- Develop clinically-relevant roll-up hierarchy for LOINC terms	Partially Achieved	22 -- CDE representation standards

APPENDIX F: OS-PCORTF PROJECT LIST

FY2017 Investments

Note that no 2017 projects were evaluated in this report

Technologies for Donating Medicare Beneficiary Claims Data to Research Studies (NIH & CMS)

The proposed project aims to leverage and, where appropriate, merge technologies under development with the goal of providing a safe and secure mechanism for Medicare beneficiaries to donate their claims data to scientific research studies. The project goal is to create a research data donation client application ("app") that would enable researchers to receive the data contributed by patients. This app would leverage current activities that are simplifying the data contribution process for patients and would encourage increased data sharing across multiple research studies through a streamlined registry of available data donation opportunities.

Developing a Strategically Coordinated Registry Network (CRN) for Women's Health Technologies (FDA in partnership with NIH/NLM and ONC)

The project goal is to create a strategically Coordinated Registry Network (CRN) for women's health technologies that will collect patient reported outcomes and employ the standards for Structured Data Capture (Developmental component) from EHRs for data collection and exchange to both enhance existing registries and enrich PCOR data infrastructure pertinent to women's health conditions.

Enhancing Data Resources for Studying Patterns and Correlates of Mortality in Patient-Centered Outcomes Research (CDC in partnership with CMS and FDA)

The project goal and objectives are the linkage of data on fact, cause, and manner of death from the National Death Index (NDI) to several federal population-based health data platforms, to demonstrate the feasibility of such linkage, enable high-value patient-centered outcomes research (PCOR) on patterns and correlates of mortality via the resulting linked data, and to facilitate collaboration between federal partners regarding strengthening the infrastructure and methods for linking healthcare data to mortality outcomes and using such linked data for PCOR.

Advancing the Collection and Use of Patient-Reported Outcomes through Health Information Technology (AHRQ in partnership with ONC)

The goal of this project is to develop technical tools for collecting and integrating patient-reported outcome (PRO) assessments into electronic health records (EHRs) or other health information technology (IT) products, by:

- Refining and/or developing health IT standards that can be used to support sharing of PRO data through APIs and relevant health IT products for research;
- Supporting the development of user-friendly, PRO-collection applications that utilize the health IT standards or APIs; and

- Implementing private/public partnerships for pilot-testing these technical tools in a health system that supports both healthcare delivery and research.

Harmonization of Various Common Data Models and Open Standards for Evidence Generation (FDA in partnership with NIH/NLM/NCI and ONC)

This goal of this project is facilitating the use of Real World Data (RWD) sources (e.g., claims, EHRs, registries, electronic Patient Reported Outcomes (ePRO)) to support evidence generation for regulatory and clinical decision making. This project will:

- Develop common data architecture as the intermediary between various Common Data Models.
- Create a sustainable, flexible, modifiable shared resource that can evolve over time with changing requirement.
- Validate the common data architecture through a specific use case that would evaluate the safety of newly approved oncology drugs which help to allow the immune system to target cancers.

Establish methods and develop processes, policies and governance for ongoing curation, maintenance and sustainability of the common data architecture, building upon existing resources, standards and tools.

FY2016 Investments

Note that only 2016 Projects designated with an "" were evaluated in this report*

*Improving Beneficiaries' Access to their Health Information through an Enhanced Blue Button Service**

The enhanced Blue Button service, BBonFHIR, creates an upgraded data service that enables CMS beneficiaries to connect their MyMedicare.gov data to the applications and services they trust including research platforms. BBonFHIR creates an integration model for the industry with structured data formats and standard interfaces, making it simpler for beneficiaries to automate linking their data to research studies. Researchers will be able to recruit beneficiaries to research studies by sending those recruits to a web page or mobile app that would request that a beneficiary gives the researcher access to their data at CMS. Upon agreeing to this request, the beneficiary will be taken to a CMS page to authenticate and authorize the access. This process dramatically simplifies acquisition and transformation of beneficiary claims information to support research studies.

*Development of a Natural Language Processing (NLP) Web Service for Structuring and Standardizing Unstructured Clinical Information**

While there have been strides through Meaningful Use and other activities to implement standardized electronic health record (EHR) systems, there continue to be parts of the medical record, laboratory reports, and other clinical reports that are reported in free-form text narratives. This project will develop an NLP Web Service on the Public Health Community Platform (PHCP) that will have the core functionality to accept and process unstructured textual information and return standardized common data elements and coded data from selected terminologies (ICD-9-CM, ICD-O-3, LOINC, SNOMED CT, MedDRA, etc.). This project will pilot implementation of the NLP Web Service using cancer data and surveillance data for blood products and vaccines and provide guidance to other federal

agencies, public health agencies, and patient-centered outcomes researchers on how to include their domain-specific terminologies and coding rules.

Source Data Capture from EHRs: Using Standardized Clinical Research Data

This project will demonstrate a single point data capture approach from the electronic health record (EHR) to an electronic data capture system (EDC system) using the Retrieve Form for Data Capture (RFD) standard. This allows data collected in the EHR to be used as part of an FDA-regulated clinical research protocol, eliminating the need for duplicate entry, and potentially saving time, money, and eliminating an opportunity for errors. This project will provide patient-centered outcomes researchers with a cloud-based, HIPAA and 21CFRPart 11-compliant tool to seamlessly integrate EHR and EDC systems.

Use of the ADAPTABLE Trial to Strengthen Methods to Collect and Integrate Patient-reported Information with Other Data Sets and Assess Its Validity

The goal of this project is to develop, pilot and evaluate methods to validate and integrate patient-reported information with data obtained from the EHR in the context of the ADAPTABLE trial, the first major randomized comparative effectiveness trial to be conducted by the National Patient-Centered Clinical Research Network. The trial encompasses several key features, including enrollment of 20,000 patients across 6 large health care systems; an internet portal to consent patients and collect patient-reported information regarding risk factors, medications, and experiences; and reliance on existing EHR data sources for baseline characteristics and outcomes follow-up. The project will generate tools and data standards that could be deployed in other PCOR studies beyond the ADAPTABLE trial.

Standardization and Querying of Data Quality Metrics and Characteristics for Electronic Health Data

Understanding the characteristics of a data source is critical for investigators in their determination regarding whether the data is fit for use, but currently no standards exist for describing the quality and completeness of electronic health data. Metadata standards are needed to describe the quality, completeness, and stability of data sources, and to enable metadata querying. Effective use of the growing number of data sources and distributed networks will require adoption of a uniform approach to describing the quality characteristics of electronic health data, as well as the data capture characteristics at the institutional, provider, and health plan level and data domain level. This project will develop, test, and implement a standards-based approach to describing data quality and presenting data quality metrics.

Harmonization of Clinical Data Element Definitions for Outcome Measures in Registries

Syntax and standardization of electronic formats and methods of transfer of information has been a priority for EHRs and registries. There has been much less focus on whether specific clinical definitions are the same across institutions. This project will convene a series of clinical topic specific working groups, including registry holders, EHR developers, policy makers developing quality measures and other types of mandatory reporting, clinicians, health systems, industry, FDA, CMS, AHRQ, CDC , ONC, NIH, the National Quality Forum and patients to discuss the various definitions currently used and how definitions can be harmonized in order to promote common definitions for outcome measures across data collection and reporting systems.

Precision Medicine Informatics (Now the "All of Us" Initiative)

The goal of this project is to develop core data policies and standards essential for the Precision Medicine Initiative (PMI) National Cohort as well as evaluate implementation feasibility issues. A core goal of the PMI is to assemble a "longitudinal cohort of 1 million or more Americans who have volunteered to participate in research." The data infrastructure to enable the creation of this major national resource is expected to exert a transformational impact on all aspects of the clinical research stakeholder. An improved architecture that places the patient at the center and returns information to members of the cohort is a core component of the vision of this initiative. In spite of the extensive investments made in data standards, and improved understanding of the core principles that facilitate intra-operability, many challenges remain. There are not yet agreed-upon core data elements for many critical components needed for the cohort. This project will develop the data policies and technology data standards needed for the PMI National Cohort, including standards for syntax (structure), semantics (common naming and coding practices) and transmission.

*Creation of LOINC Equivalence Classes**

The goal of this project is to create a flexible, extensible, and computable mechanism for rolling LOINC codes into clinically relevant equivalence groups that enable more efficient processing aggregation of laboratory data and other data from diverse health IT systems. The primary focus of this work will be on laboratory tests. The project will develop an enhanced software tool that searches the LOINC database to then derive a clinically relevant roll-up hierarchy for LOINC terms.

OS-PCORTF Resource Center

The purpose of this contract is to maximize the impact of the OS-PCORTF by providing services to OS-PCORTF awardees to enhance implementation and collaboration, supporting execution against the OS-PCORTF Strategic Framework, and furnishing logistical support through 2019. Resource center functions will include portfolio assessment support, technical support for project development and implementation, communication and dissemination support, and assistance with portfolio management tasks.

FY2015 Investments

Utilizing Data from Various Data Partners in a Distributed Manner

This project will develop the capability to conduct rapid and secure distributed regression analysis that enables research sites within a distributed research network to maintain control of patient-level data while generating valid regression estimates across the network without the need to aggregate interim data, transfer sensitive information, or conduct meta-analysis. The deliverable from this project will be open-source software that allows stakeholders to perform automated distributed regression within actual PCOR distributed data networks.

Improving the Mortality Data Infrastructure for Patient-Centered Outcomes Research

The National Center for Health Statistics (NCHS) proposes to improve mortality data infrastructure through more timely delivery of state death records (i.e., fact of death and cause of death) to the National Death Index (NDI) database, and through linkage of NDI records with National Hospital Care Survey data. The deliverables from this project include: 1) an additional 22 states that can report the fact of death to NCHS for at least 80 percent

of the deaths occurring within their states within 5 days of death and within 10 days for cause of death and 2) improved processes for mortality file closing.

Cross-Network Directory Service

This project will create an open-source interoperable service to allow: 1) a way for data partners to easily participate in multiple data research networks, 2) a way for queries to seamlessly move across such networks, and 3) a mechanism to share analytic capabilities and knowledge across networks. This project also will pilot test this cross-network directory service across at least 2 existing networks: FDA's Mini-Sentinel and PCORI's PCORnet.

Collection of Patient-Provided Information via a Mobile Device Application for Use in Comparative Effectiveness and Drug Safety Research

This investment will enable patients to transmit data through mobile devices to a secure data repository. Researchers will be able to query both the patient-provided data repository and traditional data providers that belong to the Mini-Sentinel distributed database. Since PCORnet and Mini-Sentinel share the same coordinating center, this investment will directly benefit research performed by both organizations. The deliverable from this effort will be a generalizable mobile device application to capture data from pregnant women (i.e., drug exposures, outcomes, risk factors and confounders) that will be linked with a large distributed database (i.e., Mini-Sentinel). This effort will serve as a pilot for and collaborate with the effort noted below.

Conceptualizing a Data Infrastructure for the Capture and Use of Patient-Generated Health Data

Patient-generated health data (PGHD) can be collected today directly from patients using surveys and structured questionnaires in both paper and electronic form, and electronically through devices (e.g., glucometers) and mobile technology. However, PGHD is not sufficiently represented in either records of care or in research. In order to increase the capture and use of PGHD to meet the needs of researchers, patients and providers, this project will develop a policy framework for the use of PGHD in research and care delivery that addresses needed tools, data donation policies, regulatory gaps, use of EHRs for PGHD, and interoperability of PGHD. In addition to the policy framework, project deliverables include the results from pilot tests of the framework.

Security and Privacy Standards for Patient Matching, Linking and Aggregation

This project will standardize patient attributes and algorithms for use in reliably matching patients across organizations. It will also create the privacy and security application programming interface (API) specification guides that enable real-time linking and matching of patients with associated research, claims and clinical data. Deliverables include: an environmental scan, standards and a specifications/implementation guide for the patient attributes, matching algorithms and the privacy and security API specification.

PCOR: Privacy and Security Blueprint, Legal Analysis and Ethics Framework for Data Use, and Use of Technology for Privacy

Patient-level data are essential to understanding and improving health outcomes. These data must be made available to researchers in a way that ensures the protection of patient privacy while providing sufficient granularity to allow meaningful conclusions to be drawn. Yet, current laws and policies around use of patient-level data are nuanced and sometimes conflicting, creating confusion for researchers, providers and patients. Frameworks are

needed to address the many legal and privacy and security-related policy issues that affect use of this data for research. This project will develop these frameworks and also identify, refine, harmonize, validate, recommend, and pilot standards that support an individual's consent and preferences for research.

Improving Beneficiary Access to Health Information Blue Button to Enable a Data-as Service Platform

CMS established a Blue Button platform to give Medicare beneficiaries access to their own health information in electronic form. However, this tool had limited functionality and scalability, which made it difficult for beneficiaries to use and share their health information. The purpose of this project was to develop a plan to redesign the Blue Button to enable it as a 'Data-as-a-Service' platform to empower patients and enable the use of the data with third party applications.

FY2014 Investments

Creating the Foundational Blocks for the Learning Health Care System: Structured Data Capture

This project will identify and develop the functional and technical specifications necessary to enable an EHR system to retrieve, display, and fill a structured form or template, and store and submit the completed form to an external repository. The goal of this project is to develop, pilot, and ballot technical data standards for common data elements as well as an electronic template for use in case reporting. Electronic case reporting refers to the ability of an EHR to automatically identify and report specific cases and submit this information in a particular format or template to an end point (e.g., clinical research, public health registry surveillance system).

Creating the Foundational Blocks for the Learning Health Care System: Data Access Standards for Electronic Health Records (EHRs)

The goal of this project was to develop technical standards for how health care providers, researchers, and the public health community access and extract data from EHRs in order to conduct patient-centered outcomes research. This project sought to make it easier to get data out of an EHR in a consistent and reproducible way and is a critical next step to enabling and simplifying data aggregation across widely distributed EHR systems (i.e., distributed population queries). To accomplish this goal, the ONC developed an API that will connect to a provider's EHR to extract data in a standard way. An API is a technology that allows one software program to access the services provided by another software program.

Strengthening and Expanding Community Health Applied Research Network (CHARN) Registry to Conduct Patient-Centered Outcomes Research

CHARN is a network of the Human Resources and Service Administration's (HRSA's) community health centers and universities that was established in 2010 to build capacity to conduct patient-centered outcome research to improve patient care at federally supported community health clinics. These clinics serve people who are significantly underrepresented in traditional health research, including those who are uninsured, poor, and members of racial and ethnic minority groups. This project supported the maintenance of the current CHARN infrastructure and expansion of the CHARN database to include data for calendar years 2006, 2007, and 2011-2013, which completed the CHARN database from 2006 through 2013. The project also successfully supported efforts to make CHARN data available

to more PCOR investigators, including outside researchers, by developing a public use analytic database file and developing a data analysis file.

FY2013 Investments

Expanding Data Collection Infrastructure of the National Program of Cancer Registries for Comparative Effectiveness Research

The 2 foci of this project were to 1) enhance specialized cancer registries by creating data sets for CER with extended longitudinal follow-up and data collection of disease recurrence, progression, and vital status for 2011 colon, rectum and breast cancer, and myelogenous leukemia cases; and 2) expand EHR reporting to central cancer registries for CER by addressing requirements to implement Meaningful Use reporting to central cancer registries including enhancement of software tools and methodology for management and consolidation of electronic data reported on a real-time basis from EHRs and through data linkages.

Maintenance and Support of the Chronic Condition Warehouse for Comparative Effectiveness Research

The purpose of this project was to supplement the CMS Chronic Conditions Warehouse expanded data infrastructure to support its use for PCOR. The overarching objectives of the project were to provide access, continued maintenance and renewal of licensing for the PCOR. Access to researchers focused on supporting approximately 20 approved researchers conducting PCOR project via the CCW Virtual Research Data Center (VDRC). VDRC operations and maintenance included such activities as creating SAS data set files, exploring new data files, renewing and tracking equipment maintenance, updating data dictionaries.

Strategic Opportunities for Building Data Infrastructure for Comparative Effectiveness Research

The purpose of this foundational project was to provide background research, technical and analytic consultation, and meeting and document preparation assistance to HHS as it shapes its strategy for building its data capacity. The objectives to fulfill this purpose included creating a data infrastructure document to provide the conceptual basis/framework for further information gathering and reporting on strategic opportunities; performing an environmental scan of the current landscape of public and private data infrastructure activities; developing series of policy papers to identifying gaps and opportunities for achieving key goals; and preparing a written draft strategic plan to articulate concrete, strategic opportunities where HHS could influence and invest in building PCOR data infrastructure.

FY2012 Investments

CER Inventory

The primary objective of the CER Inventory was to accurately and comprehensively inventory federal and nonfederal CER activities (within both for-profit and not-for-profit organizations). OS-PCORTF supported improvements to the inventory logic, design, and user interface identified during the conduct of the inventory; supporting server hardware requirements; updating and maintaining necessary software licenses; and providing necessary subject matter and technical staff to operate the inventory.

Development of Data Infrastructure for Use in EHRs in CER

This joint project of the Office of the National Coordinator for Health Information Technology (ONC) and the National Institutes of Health's National Library of Medicine (NLM) was guided by the principle that applicability and power of CER studies would be increased by the use of structured data definitions—common data elements (CDEs)—which comply with the consensus-derived health data standards established for "meaningful use" of EHRs. The challenge was to define and collect patient data (e.g., lab test results, marital status, and patient-reported depression) in a standardized way in different CER studies, to enable the comparison of results and facilitate use of EHR systems as a source of valid CER data. As a result of work on the project, the NLM created and continues to populate the NIH CDE Repository which currently contains 12 classifications of CDEs totaling 19,904 elements across the classifications. It also contains 10 classifications of eCRFs that contain 2,236 individual eCRFs.

MPCD Beta Test

The Multi-Payer Claims Database (MPCD) represented a public/private partnership with the goal of evaluating the benefits of consolidating longitudinal health care claims data from public and private payers, to facilitate comparative effective research. As part of its development, MPCD underwent an internal beta test, supported by an award from the OS-PCORTF, to evaluate its utility by surveying the experiences of federal and contracted researchers who were requesting and using customized data extracts for CER studies.

APPENDIX G: MODERATOR GUIDES

Guide 1 – Federal Agency Leadership

1. INTRODUCTION – BACKGROUND INFORMATION

First, I'd simply like to ask:

1.1 What is your job title and how long have you been involved with projects at [FEDERAL AGENCY] to build data capacity in PCOR?

2. STRATEGIC VISION

Next, I have a couple of questions regarding [FEDERAL AGENCY'S] strategic vision.

2.1 What has been [FEDERAL AGENCY'S] strategic vision for its PCOR Trust Fund portfolio of project[s] to build data capacity in PCOR?

2.2 How did [FEDERAL AGENCY'S] selection of the portfolio of PCOR Trust Fund project[s] reflect that vision?

2.2.1 Probe: In your opinion, what gaps in data capacity were being addressed?

3. ENABLING CORE FUNCTIONALITIES VIA OS-PCORTF PROJECT OUTPUTS

Thank you. Now I'd like to ask you about project(s) outcomes.

3.1 From your perspective, what key outcomes has or have your project[s] achieved?

3.2 From your perspective, what are the challenges [FEDERAL AGENCY] has experienced related to the PCOR Trust Fund project[s] you are overseeing?

4. CONTRIBUTION OF OS-PCORTF PROJECTS TO FEDERAL STAKEHOLDER INITIATIVES

Let's now focus on the OS-PCORTF portfolio across HHS.

4.1 What outcomes from the portfolio of projects do you think have been key to HHS's mission?

4.2 Overall, how has the portfolio of projects impacted data capacity for clinical research across HHS?

4.3 Did the PCOR Trust Fund project[s] promote coordination between [AGENCY NAME] and any other agencies?

4.3.1 Probe if yes: How did it do so?

4.3.2 Probe if no: How could it have done so?

4.4 In your opinion, were there sufficient opportunities for the agencies to coordinate efforts around each other's priorities?

Now, I'd like to ask about data infrastructure sustainability. I'm defining sustainability as, "Whether what's been done to date will be useful over time and be the foundation for future projects."

4.5 What effect or effects do you think [AGENCY's NAME] project[s] have or has had on the sustainability of data capacity in PCOR?

4.5.1 Probe: What could be done to make the data capacity more sustainable?

4.5.2 Probe: What are the challenges to sustaining the data capacity?

5. ADDRESSING THE NEEDS OF FEDERALLY AND PRIVATELY FUNDED RESEARCHERS

We're nearing the end of the interview. I have 2 questions regarding future needs and opportunities.

5.1 If there were the resources, what project or projects would you like to see as a next phase?

5.2 What do you see as [AGENCY's NAME] future role in building data capacity for PCOR?

Finally, I have 2 last questions.

6.1 Are there any other observations about your experiences with PCOR Trust Fund initiative that you'd like to share?

6.2 Lastly, are there particular questions you would like us to ask of the project leads, research network leads, health system or payer representatives?

Thank you for your time and for speaking with us.

I'd like to ask you something aside from the interview. Is there anyone you worked with on the PCOR TF project(s) that you think would be good for us to interview? We're particularly interested in people from research networks, health payers, or others such as patient advocates or software developers.

END

Guide 2 – Project Leadership

1. INTRODUCTION – BACKGROUND INFORMATION

To start off…

1.1 First, can you confirm that you led or are leading the following PCOR Trust Fund project[s]? [INSERT KNOWN PROJECT NAME(S) BELOW)

_____

_____

1.1.1 Did you lead the project[s] for the entire duration?

1.1.1.1 Probe IF NO: For how long did you lead each project?

1.2 What other PCOR Trust Fund project[s], if any, have you participated in developing or implementing?

1.2.1 Probe IF YES: In what role[s] and for what duration?

_____

_____

2. APPROPRIATENESS OF CORE FUNCTIONALITIES TO ADDRESS GAPS IN DATA CAPACITY FOR PCOR

My next question focuses on the functionalities of the PCOR Trust Fund portfolio. You will find it helpful to refer to the Overview document we provided you prior to this call. [Confirm they have the document, be ready to send to them]

2.1. Overall, how do you think the functionalities reflect the requirements for building data capacity in PCOR, including comparative effectiveness research and patient-centered outcome research?

2.1.1 Probe: What if any functionalities could be added, removed or revised?

3. EVALUATING PROJECT OUTPUTS TO ENABLE THE CORE FUNCTIONALITIES

The following 2 questions explore how relevant the functionalities might have been for the projects you worked on.

3.1 What key outcomes has or have your project[s] achieved relative to the 5 functionalities?

3.2. From your perspective, what if any key outcomes from your project might not have been reflected in any of the 5 functionalities?

4. ASSESSING HOW PROJECTS INFORM AND CONTRIBUTE TO KEY FEDERAL STAKEHOLDERS' INITIATIVES, AVOID DUPLICATION, AND FOSTER COORDINATION ACROSS HHS

These next 2 questions ask about the portfolio of PCOR Trust Fund projects as a whole, and how well the projects aligned and promoted coordination across HHS for building data capacity for research.

4.1 Considering the projects on page 2 of the Overview document, how do these projects for bu104ilding data capacity align with other initiatives your (agency/organization) is working on? Please provide specific examples.

4.2 In your opinion, has the portfolio sufficiently promoted coordination between [AGENCY NAME] and other agencies?

4.1.1 Probe if yes: Can you provide examples how?

4.1.2 Probe if no: Can you provide examples of how not and what could have been done better?

5. ADDRESSING THE RESEARCH NEEDS OF FEDERALLY AND PRIVATELY FUNDED RESEARCH NETWORK STAKEHOLDERS

We're nearing the end of the interview. I have 4 questions regarding the needs of end users.

5.1 Who would you expect to be the end users of your OS-PCORTF project[s] output—research networks, health systems, clinical researchers, etc.?

5.1.1 Probe: How do you think those end users will be impacted by your project's (s') deliverables?

5.2 In your opinion, were there any deliverables you think end users would value but your project(s) could not deliver?

5.3 To your knowledge, how has or have your PCOR Trust Fund project(s) improved data capacity for PCOR, including comparative effectiveness research and patient-centered outcome research?

5.3.1 Probe: Please elaborate.

5.4 What lessons learned do you think HHS should take away from the portfolio of PCOR Trust Fund project[s]?

6. CONCLUSION

I have 2 last questions for you.

6.1 If there were resources available for another round of data capacity development, what project or projects would you like to see?

6.2 Are there any other observations about the PCOR Trust Fund initiative that you'd like to share?

Thank you for your time and for speaking with us.

I'd like to ask you something aside from the interview. Is there anyone you worked with on the PCOR TF project(s) that you think would be good for us to interview? We're particularly interested in people from research networks, health payers, or others such as patient advocates or software developers.

END

Guide 3 – Research Network Representatives

1. INTRODUCTION – BACKGROUND INFORMATION

First off:

1.1 What is your job title and how long have you worked at [ORGANIZATION]?

_____

_____

1.2 What work does [ORGANIZATION] do in building data capacity for PCOR, such as PCOR or comparative effectiveness research?

1.3 In looking at the projects on page 2 of the Overview document we sent you beforehand, how familiar are you with any of them and the outputs they produced?

1.3.1 To your knowledge, has your organization used any of the products developed or knowledge gained from the PCOR Trust Fund projects?

1.3.2 If yes, how has your organization used the products developed?

1.3.3 If no, which projects on the list we provided might be of most value to your organization?

2. APPROPRIATENESS OF CORE FUNCTIONALITIES TO ADDRESS GAPS IN DATA CAPACITY FOR PCOR

My next questions focus on the functionalities of the PCOR Trust Fund portfolio. You will find it helpful to refer to the Overview document we provided you prior to this call. [Confirm they have the document, be ready to send to them]

2.1 In general, how do you think the 5 functionalities reflect the requirements for building data capacity in PCOR, including comparative effectiveness research and patient-centered outcome research?

2.1.1 Probe: What if any functionalities could be added, removed or revised?

3. EVALUATING PROJECT OUTPUTS TO ENABLE THE CORE FUNCTIONALITIES

The following 3 questions explore how applicable you think the functionalities might be, given [ORGANIZATION's] work around building data capacity in PCOR.

3.1 Specifically, how relevant are the 5 functionalities to your organization's efforts related to building data capacity for PCOR?

3.2 What key outcomes has or have your organization achieved relative to the 5 functionalities?

3.3 From your perspective, what, if any, key outcomes from your organization's work on PCOR might not be reflected in the 5 functionalities?

4. ASSESSING HOW PROJECTS INFORM AND CONTRIBUTE TO KEY FEDERAL STAKEHOLDERS' INITIATIVES, AVOID DUPLICATION, AND FOSTER COORDINATION ACROSS HHS

These next 2 questions ask about the research community's building of data capacity for PCOR.

4.1 In what ways does [ORGANIZATION] collaborate with various stakeholder groups to build data capacity?

4.1.1 Probe: Governmental agencies and projects?

4.1.2 Probe: Industry partners?

4.1.3 Probe: Any other stakeholders of importance to note?

4.2 In your experience, what barriers to collaboration with stakeholders have you encountered in building data capacity for PCOR?

5. ADDRESSING THE RESEARCH NEEDS OF FEDERALLY AND PRIVATELY FUNDED RESEARCH NETWORK STAKEHOLDERS

We're nearing the end of the interview. I have 4 questions regarding the needs of end users.

5.1 Who are the end users of [ORGANIZATION'S] output— other research networks, health systems, clinical researchers, others.?

5.1.1 Probe: What problem(s) related to data capacity are you trying to help those end users solve?

5.2 What data capacity problems do you think [ORGANIZATION] will need to be addressed for end users working in PCOR within the next 3 years?

5.2.1 Probe: Beyond 3 years?

6. CONCLUSION

I have 2 last questions for you.

6.1 If future federal efforts for addressing data capacity development projects in PCOR were to happen, what efforts would you like to see?

6.2 What lessons learned from your experience working to build data capacity for PCOR would you share with the federal government?

Thank you for your time and for speaking with us.

END

Guide 4 – Payers - HCO Representatives

1. INTRODUCTION – BACKGROUND INFORMATION

First off:

1.1 What is your job title and how long have you worked at [ORGANIZATION]?

_____

_____

1.2 What work does [ORGANIZATION] do in building data capacity for PCOR, such as PCOR or comparative effectiveness research?

1.3 In looking at the projects on page 2 of the Overview document we sent you beforehand, how familiar are you with any of them and the outputs they produced?

1.3.1 To your knowledge, has your organization used any of the products developed or knowledge gained from the PCOR Trust Fund projects?

1.3.2 If yes, how has your organization used the products developed?

1.3.3 If no, which projects on the list we provided might be of most value to your organization?

2. APPROPRIATENESS OF CORE FUNCTIONALITIES TO ADDRESS GAPS IN DATA CAPACITY FOR PCOR

My next questions focus on the functionalities of the PCOR Trust Fund portfolio. You will find it helpful to refer to the Overview document we provided you prior to this call. [Confirm they have the document, be ready to send to them]

2.1 In general, how do you think the 5 functionalities reflect the requirements for building data capacity in PCOR, including comparative effectiveness research and patient-centered outcome research?

2.1.1 Probe: What if any functionalities could be added, removed or revised?

3. EVALUATING PROJECT OUTPUTS TO ENABLE THE CORE FUNCTIONALITIES

The following 3 questions explore how applicable you think the functionalities might be, given [ORGANIZATION's] work around building data capacity in PCOR.

3.1 Specifically, how relevant are the 5 functionalities to your organization's efforts related to building data capacity for PCOR?

3.2 What key outcomes has or have your organization achieved relative to the 5 functionalities?

3.3 From your perspective, what, if any, key outcomes from your organization's work on PCOR might not be reflected in the 5 functionalities?

4. ASSESSING HOW PROJECTS INFORM AND CONTRIBUTE TO KEY FEDERAL STAKEHOLDERS' INITIATIVES, AVOID DUPLICATION, AND FOSTER COORDINATION ACROSS HHS

These next 2 questions ask about the research community's building of data capacity for PCOR.

4.1 In what ways does [ORGANIZATION] collaborate with various stakeholder groups to build data capacity?

4.1.1 Probe: Governmental agencies and projects?

4.1.2 Probe: Industry partners?

4.1.3 Probe: Any other stakeholders of importance to note?

4.2 In your experience, what barriers to collaboration with stakeholders have you encountered in building data capacity for PCOR?

5. ADDRESSING THE RESEARCH NEEDS OF FEDERALLY AND PRIVATELY FUNDED RESEARCH NETWORK STAKEHOLDERS

We're nearing the end of the interview. I have 4 questions regarding the needs of end users.

5.1 Who are the end users of [ORGANIZATION'S] output— other research networks, health systems, clinical researchers, others.?

5.1.1 Probe: What problem(s) related to data capacity are you trying to help those end users solve?

5.2 What data capacity problems do you think [ORGANIZATION] will need to be addressed for end users working in PCOR within the next 3 years?

5.2.1 Probe: Beyond 3 years?

6. CONCLUSION

I have 2 last questions for you.

6.1 If future federal efforts for addressing data capacity development projects in PCOR were to happen, what efforts would you like to see?

6.2 What lessons learned from your experience working to build data capacity for PCOR would you share with the federal government?

Thank you for your time and for speaking with us.

END

Guide 5 – Patient Advocates

1. INTRODUCTION – BACKGROUND INFORMATION

First, I'd like to ask:

1.1 How long have you been a patient advocate and what influenced you to become one?

1.2 What experiences have you had managing your own health data or helping others manage their health data?

Next, I'd like to ask you about health information technology (health IT) for carrying out PCOR. For example, health IT for PCOR can include using smartphones or online health records to collect and share personal health data with researchers.

1.3 What experiences have you had with health IT in PCOR?

1.3.1 Probe IF EXPERIENCED: Have any of those experiences been from one or more projects funded by the federal government? And if so, can you describe the project[s]?

1.3.2 Probe IF NOT EXPERIENCED: In your experience, what was used to help patients collect and share data for the research?

2. APPROPRIATENESS OF CORE FUNCTIONALITIES TO ADDRESS GAPS IN DATA CAPACITY FOR PCOR

Thank you. Now I'd like to get you opinion as to how health IT may impact PCOR.

2.1 In your opinion, how does health IT impact patients' and researchers' ability to conduct PCOR?

2.1.1 Probe: What capabilities do you think patients find most valuable? For example, patients being able to securely download their Medicare data?

2.1.2 Probe: What capabilities do you think patients don't find to be valuable?

3. EVALUATING PROJECT OUTPUTS TO ENABLE THE CORE FUNCTIONALITIES

3.1 What do you think it is about any health IT capabilities that make some more valuable than others for patients?

4. ASSESSING HOW PROJECTS INFORM AND CONTRIBUTE TO KEY FEDERAL STAKEHOLDERS' INITIATIVES, AVOID DUPLICATION, AND FOSTER COORDINATION ACROSS HHS

I'd now like to ask you about the federal government and health IT for PCOR.

4.1 From your perspective, what impact do you think the federal government has on using health IT for PCOR?

5. ADDRESSING THE RESEARCH NEEDS OF FEDERALLY AND PRIVATELY FUNDED RESEARCH NETWORK STAKEHOLDERS

We're nearing the end of the interview.

5.1 In your opinion, what kinds of work would you like to see carried out at the federal level to improve the use of health IT in PCOR?

5.2 In your opinion, what do you think slows the use of health IT in PCOR at the federal level?

6. CONCLUSION

6.1 Are there any observations about health IT for PCOR that you'd like to share?

Thank you for your time and for speaking with us.

END

APPENDIX H: OVERVIEW DOCUMENT

Gaining Expert Perspectives on Building Data Capacity for PCOR

RTI International is evaluating the impact that the Office of the Secretary's Patient-Centered Outcomes Research Trust Fund (OS-PCORTF) has had on building data capacity for PCOR, including comparative effectiveness research (CER) and patient-centered outcome research (PCOR). As part of the evaluation, we are interviewing experts who are or have been affiliated with PCOR Trust Fund projects. The interviews will:

1. Understand key stakeholders' views of the appropriateness of the core functionalities to address gaps in data capacity for PCOR.
2. Evaluate whether and how various key stakeholders have used the products from the OS-PCORTF projects to enable the core functionalities.
3. Assess how OS-PCORTF projects informed and contributed to key federal stakeholders' research needs, helped to avoid duplication, and fostered coordination across HHS.
4. Assess how OS-PCORTF projects and products are perceived to have addressed the research needs of federally and privately funded research network stakeholders.

During our telephone interview with you, we will discuss your experiences and perspectives from working with OS-PCORTF-funded projects. Over the course of our discussion, we will reference the data infrastructure "functionalities" listed in the table below, as well as the OS-PCORTF-funded projects being evaluated (see page 2).

Background

Through the OS-PCORTF, ASPE is currently funding projects to build data capacity for developing and maintaining a comprehensive, interoperable, data network to collect, link, and analyze data for CER and PCOR. Investments in data infrastructure address foundational components that will support both current and future efforts to advance PCOR, as well as other federal initiatives.

OS-PCORTF's current focus is enhancing and improving data capacity for PCOR related to 5 core functionalities:

1. **Use of Clinical Data for Research** stems from multiple sources of clinical data available for research (e.g., EHRs, data available via patient portals, registries); and efforts in this area are focused on improving access and interoperability of clinical data for query and analysis.
2. **The Standardized Collection of Standardized Clinical Data** supports the use of common data elements to enable more effective and efficient linking and aggregation across data sources.
3. **The Linking of Clinical and Other Data for Research** allows researchers to collect longitudinal patient information and to link data sets with other relevant information for research.
4. **The Collection of Participant-Provided Information** via new data collection technologies provides means for collecting patient-generated information critical to PCOR.
5. **Use of Enhanced Publicly Funded Data Systems for Research** focuses on efforts to leverage current investments in federally available data and infrastructure to inform future infrastructure development.

Collectively, these investments and initiatives have begun to lay the foundation for data capacity and infrastructure that can be leveraged by federal and nonfederal researchers conducting PCOR including CER and PCOR.

Please contact **Alexa Ortiz** at amortiz@rti.org if you have any questions. Thank you.

SUBSET OF OS-PCORTF PROJECTS INCLUDED IN EVALUATION (ARRANGED BY AGENCY)

ASPE

- Comparative Effectiveness Research (CER) Inventory (FY2012): To inventory Federal and non-Federal CER activities (within both for-profit and not-for-profit organizations).

CDC

- Expanding Data Collection Infrastructure of the National Program of Cancer Registries for Comparative Effectiveness Research (FY2013): To enhance cancer registries with extended longitudinal follow-up and data collection of disease and expand EHR reporting to central cancer registries for CER.

- Improving the mortality data infrastructure for patient-centered outcomes research (FY2015): To improve mortality data infrastructure through more timely delivery of state death records (i.e., fact of death and cause of death) to the National Death Index (NDI) database.

- Development of a Natural Language Processing (NLP) Web Service for Structuring and Standardizing Unstructured Clinical Information (FY2016): To develop an NLP Web Service on the Public Health Community Platform (PHCP) to accept and process unstructured textual information and return standardized data.[a]

CMS

- Improving Beneficiaries' Access to their Health Information through an Enhanced Blue Button Service (FY2016): To create an upgraded data service that enables CMS beneficiaries to connect their MyMedicare.gov data to the applications and services they trust including research platforms.

- Improving Beneficiary Access to Health Information "Blue Button" to Enable a 'Data-as Service' Platform (FY2015): To redesign the Blue Button to enable it as a 'Data-as-a-Service' platform to empower patients and enable the use of the data with third party applications.

- Maintenance and Support of the Chronic Condition Warehouse for Comparative Effectiveness Research (FY2013): To supplement the Centers for Medicare and Medicaid Services (CMS) Chronic Conditions Warehouse expanded data infrastructure to support its use for PCOR.

- The Multi-Payer Claims Database (MPCD) Beta Test (FY2012): To evaluate the benefits of consolidating longitudinal health care claims data from public and private payers, to facilitate comparative effective research.

FDA

- Collection of patient-provided information through a mobile device application for use in comparative effectiveness and drug safety research (FY2015): To transmit data through mobile devices to a secure data repository and enable researchers to query data that belong to the Mini-Sentinel distributed database.

- Cross-network directory service (FY2015): To create a service that allows data partners to participate in multiple data research networks, and share queries and analytic capabilities.[b]

[a] FDA is also part of this joint project; [b] NIH is also part of this joint project.

- Utilizing data from various data partners in a distributed manner (FY2015): To develop the capability for research sites to conduct rapid and secure distributed regression analyses using patient data.

HRSA

- Strengthening and Expanding Community Health Applied Research Network (CHARN) Registry to Conduct Patient-Centered Outcomes Research (FY2014): To support the current CHARN infrastructure and expansion of the CHARN database to include data for calendar years 2006, 2007, and 2011-2013.

NIH

- Creation of LOINC Equivalence Classes (FY2016): To assemble a "longitudinal cohort of 1 million or more Americans who have volunteered to participate in research."[c]

ONC

- Conceptualizing a Data Infrastructure for The Capture and Use of Patient-Generated Health Data (FY2015): To develop a policy framework for the use of PGHD in research and care delivery that addresses needed tools, data donation policies, regulatory gaps, and use of EHRs for PGHD.
- Creating the Foundational Blocks for the Learning Health Care System: Data Access Standards for Electronic Health Records (EHRs) (FY2014): To develop technical standards for how users extract data from EHRs to conduct patient-centered outcomes research.
- Creating the Foundational Blocks for the Learning Health Care System: Structured Data Capture (FY2014): To develop the functional and technical specifications necessary to enable an EHR system to exchange data to an external repository.
- Development of Data Infrastructure for Use in EHRs in Comparative Effectiveness Research (CER) (FY2012): To define and collect patient data in a standardized way in different CER studies, to enable the comparison of results and facilitate use of EHR systems as a source of valid CER data.
- PCOR: Privacy and Security Blueprint, Legal Analysis and Ethics Framework for Data Use, & Use of Technology d\for Privacy (FY2015): To develop legal and privacy and security-related policy research frameworks for patient data.
- Security and Privacy Standards for Patient Matching, Linking and Aggregation (FY2015): To standardize patient attributes and algorithms for matching patients across organizations.
- Strategic Opportunities for Building Data Infrastructure for Comparative Effectiveness Research (FY2013): To provide background research, technical and analytic consultation, and meeting and document preparation assistance to HHS as it shapes its strategy for building

[c] CDC is also part of this joint project.