National Workshop Report: Advancing the Use of Electronic Data in Patient-Centered Outcomes Research

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About this Report
This document provides a summary of the National Workshop to Advance the Use of Electronic Data in Patient-Centered Outcomes Research that took place in Palo Alto, CA, in July 2012. All presentations from the workshop, as well as an archive of the workshop webcast, are available at www.pcori.org. If you have any questions regarding the workshop or content of this document, please contact us at info@pcori.org.

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About PCORI
PCORI was authorized by the Patient Protection and Affordable Care Act of 2010 as a non-profit, nongovernmental organization and is charged with helping patients, clinicians, purchasers, and policy makers make better-informed health decisions by “advancing the quality and relevance of evidence about how to prevent, diagnose, treat, monitor, and manage diseases, disorders, and other health conditions.” It does this by producing and promoting high-integrity, evidence-based information that comes from research guided by patients, caregivers, and the broader healthcare community.

PCORI’s research is guided by a strong patient-centered orientation desired to inform the process of choosing among prevention, diagnosis and treatment strategies, and directing attention to individual and system differences that may influence strategies and outcomes. PCORI is committed to producing useful, relevant clinical evidence through the support of new research and the analysis and synthesis of existing research.

PCORI is committed to transparency and a rigorous stakeholder-driven process that emphasizes patient engagement. PCORI uses a variety of forums and public comment periods to obtain public input to enhance its work.

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Executive Summary

Overview
On July 2-3, 2012, PCORI convened a select group of stakeholders for the “The National Workshop to Advance the Use of Electronic Data in Patient-Centered Outcomes Research,” in Palo Alto, California.¹ This was the first workshop jointly sponsored by PCORI’s Methodology Committee (MC), Board of Governors, and staff.

The two-day workshop focused on developing ideas for PCORI to facilitate the creation of a national data infrastructure to support high quality patient-centered outcomes research (PCOR). Nearly 100 experts and key decision-makers attended the event, including officials from key agencies within the Department of Health and Human Services (HHS), experts on electronic health data, and representatives from patient and provider groups.² During the meeting, participants shared their knowledge about current electronic data (eData) healthcare research initiatives and discussed what they viewed as the biggest challenges to advancing the use of electronic data in the conduct of PCOR. One main take-away from the meeting was a broad recognition among participants that in order for an electronic clinical research network to truly advance PCOR, patients will need to be deeply involved in the creation and governance of the infrastructure.

Speakers
The keynote speech was delivered by Francis Collins, Director of the National Institutes of Health, who shared his vision of a National Patient-Centered Research Network.

A survey of the landscape was provided by speakers Erin Holve of AcademyHealth, Lucila Ohno-Machado of University of California San Diego, and Bob Kocher of Venrock. Erin Holve described the work by the EDM Forum to harness the learnings from ongoing data initiatives funded by AHRQ, Lucila Ohno-Machado spoke about her experiences with SCANNER and the UC-Research eXchange Informatics Consortium, and Bob Kocher discussed the potential role of the private sector.

Lessons from the Field: Opportunities and Challenges
During this segment of the workshop, participants learned about several different models that could form the basis for an ideal approach to a national patient-centered clinical research network for PCORI. These networks include the HMO Research Network (HMORN) and its Virtual Data Warehouse (VDW), The Mini-Sentinel Pilot, DARTNet, SCANNER (SCAlable National Network for Effectiveness Research), C3N (Collaborative Chronic Care Network), Patient Registries, and a vision for “Networks of Networks”. Participants discussed the virtues of these

¹ See Appendix A for a workshop agenda.
² See Appendix B for a list of workshop participants.
networks as well as the challenges that would exist in transforming these networks into an ideal patient-centered research network.

**Breakout Sessions**

During the workshop, attendees were divided into breakout groups to discuss specific themes. These themes were:

- Governance,
- Data standards and interoperability,
- Architecture and data exchange,
- Privacy and ethical issues,
- Methods for overcoming imperfect data,
- Unconventional approaches, and Incorporating Patient Reported Outcomes (PROs) intoElectronic Data

**Participant Recommendations**

Each breakout group facilitated a brainstorming session in support of PCORI’s imperative to promote and facilitate the development of a sustainable infrastructure for conducting PCOR. This resulted in three to four prioritized recommendations for PCORI per group.³ Through a voting process, the recommendations produced across the seven breakout groups were narrowed to top 10 recommendations that comprised a framework and action items in support of PCORI’s role in improving the national data infrastructure.

**Operationalizing the Recommendations: Emerging themes from the breakout sessions**

Harlan Krumholz, PCORI Board of Governors, and Steve Goodman, PCORI Methodology Committee, challenged the audience to think about the “adjacent possible,” that is, the concrete next steps for PCORI, given the recommendations that were generated through the breakout sessions. This discussion focused primarily on three broad topics: governance, data infrastructure building blocks (standards, architecture, and methods), and unconventional approaches to future strategic investments.

**Key Takeaways: What Would a National Patient-Centered Clinical Research Network Look Like?**

For PCORI, data infrastructure would ideally be flexible enough to address important gaps that currently exist in supporting PCOR. Therefore it would possess several characteristics such as:

- Coverage of large, diverse populations
- Capability of complete capture of longitudinal data
- Capability to capture and link clinical data to patient-reported data
- Establishment of patient and clinician engagement in governance
- Affordability
- Ability to link back to health systems
- Capability to randomize patients (at individual and cluster-level)

³ See Appendix C for a full list of recommendations.
Next Steps
During the two-day workshop, new approaches for the use of electronic data to advance PCOR began to emerge. Next steps for PCORI staff and leadership were to determine how PCORI could continue to advance the vision outlined in Palo Alto and, in doing so, fully and meaningfully engage patients and caregivers in developing a patient-centered clinical research network.
Overview
On July 2-3, 2012, PCORI convened a select group of stakeholders for the “The National Workshop to Advance the Use of Electronic Data in Patient-Centered Outcomes Research,” in Palo Alto, CA. This was the first workshop jointly sponsored by PCORI’s Methodology Committee (MC), Board of Governors, and staff. The two-day workshop focused on developing ideas for how PCORI could facilitate the creation of a national data infrastructure to support high quality patient-centered outcomes research (PCOR).

The goals of the workshop were to:

1. Achieve common understanding of the current landscape of efforts to build clinical data infrastructure that could support a robust patient-centered outcomes research agenda, including the strengths, weaknesses, potential and challenges of these efforts.
2. Identify promising approaches and areas that may constitute opportunities for PCORI to contribute to advancing the quality, capacity, or efficiency of one or more PCOR data infrastructures – by expanding or linking databases or registries, by enhancing the accuracy or validity of clinical data across systems, or by addressing complex questions such as governance, data-sharing, privacy, or collaboration.

Speakers
To kick off the workshop, Francis Collins, Director of the National Institutes of Health, challenged PCORI and the workshop attendees to think big as he described a bold vision for his national patient-centered clinical data research network. Dr. Collins urged participants to seize this moment to begin defining a process that would move us closer to a network that is well-designed to answer research questions in real-world settings at low marginal cost.

The National Patient-Centered Clinical Data Research Network envisioned by Dr. Collins would cover 20-30 million lives with broad representation across gender, geographic location, ethnic backgrounds, age, education level, and socioeconomic status. It would have longitudinal follow up over many years, include trained personnel in each of the participating organizations, promote data access policies that allow for broad research while protecting patient privacy, and involve patients integrally in its governance.

Such a network would enable researchers to design and implement observational trials at low cost, conduct randomized studies in diverse populations and in real-world practice settings, and reduce expenses associated with conducting clinical research studies. Additionally, patients would play a central role in the decision-making process about which studies would move forward.
To illustrate the potential of such a network, Dr. Collins presented three examples of the types of studies that can be accomplished with the network:

1. Research to determine whether mobile health-based interventions in prevention or chronic disease management improve outcomes;
2. Research studies with high numbers of patient participation and longitudinal follow up that compare the safety and effectiveness of interventions for the treatment and prevention of chronic low back pain; and
3. Trials to resolve conflicting data, such as in pharmacogenomic questions.

According to Dr. Collins, this type of clinical data research network is feasible today for numerous reasons, including:

- Health organizations with EHRs that have reached the point of making a large-scale network feasible
- Scientific opportunities, such as ‘omics’ approaches
- A sense of urgency to get answers to clinical questions
- The ability to engage in a two way conversation with the public about research
- The lessons learned from pilot efforts funded through the ARRA data methods investment about the general feasibility of such networks

Dr. Collins urged PCORI to consider the establishment of this National Patient-Centered Clinical Data Research Network as an opportunity to make a significant, tangible contribution to the research enterprise.

**Survey of Landscape**

To give participants an accurate picture of the current landscape of data networks, several leaders in the field shared their knowledge and experiences with the group.

**Erin Holve, AcademyHealth**, discussed the work being conducted by the Electronic Data Methods (EDM) Forum, which seeks to advance the national dialogue on use of electronic clinical data to generate evidence that improves patient outcomes. The EDM Forum facilitates communication and collaboration across 11 AHRQ-funded research projects to harness the learnings from these projects to help address gaps on issues related to governance, informatics, and methods.

Informed by this work, Dr. Holve articulated some of the challenges that need to be addressed in order to move toward a national electronic health data infrastructure. These include: governance challenges; the politics around and the lengthy timeframe required for facilitating partnerships; the need to better understand and convey the types of information that these data networks can and cannot produce; the struggle of defining patient-centeredness within
the research community; and the challenge of collecting, using, and implementing patient-reported information.

To link emerging data and tools in a marketplace of people and ideas committed to transforming clinical research, the gaps that must be closed (or the “Miracle Mile”), according Dr. Holve, are: data exchange, interoperability, data quality, and sustainability (the need to demonstrate the value of evidence in order for clinicians and patients to perceive that participation in and use of research is of relevance to them).

Lucila Ohno-Machado, University of California San Diego (presentation here) also presented a call to action. To accelerate research through an infrastructure with large data depositories, Dr. Ohno-Machado acknowledged the need to address privacy issues from a policy and technological standpoint. She also stressed the importance of lowering the barriers to sharing data and tools for data analysis.

Dr. Ohno-Machado described data initiatives underway within the state of California, specifically SCANNER (the SCAlable National Network for Effectiveness Research) and UC Research eXchange, an integration of clinical data warehouses from five University of California Medical Centers. Dr. Ohno-Machado discussed the use of distributed data networks to enable linkages of networks across states and even potentially countries, and to overcome concerns about inappropriate use of confidential data.

Among Dr. Ohno-Machado’s recommendations was the need to improve:

- Data harmonization using a common data model
- Privacy encryption and assessment of risk of re-identification
- Architecture that is either distributed or centralized
- Stakeholder representation in governance

Dr. Ohno-Machado concluded by defining the ultimate goal for patient-centered data sharing as enabling a patient to select preferences for data sharing from home.

Bob Kocher, Venrock, a representative of the venture capital community, urged PCORI to look to the private sector as partners in translating data into patient care, and in improving decision support tools, EHRs, payment systems and analytic tools. To maximize PCORI’s impact, Dr. Kocher highlighted the following four recommendations:

- **Liberate the data** – make as much data publicly available as possible and build upon the public-private partnership, Health DataPalooza, which allows individuals outside of the research community to use and apply the data in novel ways. Innovative approaches for utilizing publically available data could improve the patient and healthcare consumer experience. He further recommends considering that penalties be imposed upon organizations that have violated patient privacy
- **Support personalization** – of care and help individual patients understand their risk trade-offs
Collaborate with non-traditional partners – tap into the giant ecosystem of private sector as partners for investment and work with partners who will be able to move PCORI toward its mission

Enable answering of questions that impact large populations and systems – liberate health systems and care delivery data in order to enable the exploration of novel methods for creating efficiencies within the health care system

Lessons from the Field

Opportunities
During the workshop, attendees learned about six “models” that might serve as starting points for PCORI, including system-based models, practice-based research network models, and models built on social networks within a patient community:

- **HMO Research Network (HMORN) Virtual Data Warehouse (VDW)** – supported and governed by HMORN, this network pulls together data from clinical systems (including pharmacy, laboratory, pathology, disease registries, radiology and modern EHRs in all care settings) across its 19 health systems.
- **The Mini-Sentinel Pilot** – sponsored by the U.S. Food and Drug Administration (FDA) to create an active surveillance system to monitor the safety of FDA-regulated medical products. Mini-Sentinel uses pre-existing electronic healthcare data from multiple sources.
- **DARTNet** – launched with support from AHRQ, this practice-based research network captures electronic health record (EHR) data from 85 organizations and more than 400 practices.
- **SCANNER (SCAlable National Network for Effectiveness Research)** – funded by AHRQ, this network is designed to enable near and real-time comparative effectiveness research.
- **C3N (Collaborative Chronic Care Network)** – this network of patients, clinicians and researchers is testing a new system for chronic illness care. The first C3N being created is for patients with inflammatory bowel disease (IBD).
- **Patient Registries** – these networks use observational study methods to collect uniform data (clinical and other) to evaluate specified outcomes for a defined population.

The C3N’s collaboration with the ImproveCareNow network was described in detail. ImproveCareNow is a collaboration of pediatric gastroenterologists working together to improve care and health for children with inflammatory bowel disease (IBD). According to data shared by Peter Margolis of Cincinnati Children’s Hospital, the first 15 centers that joined this network, had remission rates for IBD rise from 55% to 75% without any change in medication. C3N’s collaboration with ImproveCareNow’s network enables the capture of the collective knowledge of clinicians, patients, and researchers. Dr. Margolis described three layers of activities that enable this work:
1. **Community Building (social operating system).** ImproveCareNow’s core leadership team is comprised of patients, clinicians, and researchers. Efforts to engage patients have led to the development of a patient/family advisory council, patient involvement with the quality improvement team, and a peer mentoring program that links less experienced patients to those who are more experienced. The result of this effort is the creation of a community of patients that can contribute to improving care.

2. **Development of a Technical Operating System.** To lower the transactional costs associated with data collection, ImproveCareNow is developing a data system that draws patient data directly from EHRs and offers clinicians smart forms that enable them to more efficiently collect data at the point of care.

3. **Enable Rapid Learning and Discovery Through the Scientific Operating System (SOS).** The SOS is comprised of an “Enhanced Registry” that allows for the capture of data directly from EHRs and provides automated decision support to clinicians. This assistance allows clinicians to rapidly evaluate the patient condition and make recommendations on approaches to care. It also promotes the ability to conduct experiments, including personalized evidence–based medicine (n-of-1) experiments.

Another model discussed was Richard Platt’s vision of a “network of networks.” To leverage the significant time and resources that have already been dedicated to data networks, Dr. Platt described a way that PCORI investments could be used to create a set of overlapping networks. Each network would control its own governance but would share resources – infrastructure, data curation, analytics, lessons, security, and software development. Each healthcare organization could choose its partner networks and, within each network, the projects in which it participates.

Dr. Platt provided an outline of what this “network of networks” could look like. There would be a coordinating center that operates on behalf of researchers, and using a secure portal, researchers could access any dataset from any data partner that participates in the network. Data partners can choose whether to participate in a particular study. Such a platform would also make it feasible to add other data sources, including patient-reported outcomes.

This vision for a “network of networks” resonated with many of the participants at the workshop and was one to which conversation returned throughout the day (further discussion below).

**Challenges**

A substantial portion of the meeting was spent discussing various challenges, including how to run such networks, issues related to data architecture and exchange, and system interoperability and analytic methods. Additional challenges exist around the need to incorporate patient-reported outcomes into the design and operations of networks, and develop best practices for harnessing and creating structure around patient data shared through online patient communities and networks. In assessing these challenges, several...
attendees noted that while technical barriers should by no means be taken for granted, the biggest challenges are related to managing privacy, regulatory, and proprietary interests.

Additionally, while patient involvement was considered to be an important characteristic for a PCORI network, the discussions of the six models revealed the currently low level of patient involvement across many of the existing networks. Attendees discussed potential components of a patient-centered data network, including having patients serve as members of governing boards, incorporating patient-reported data, creating a feedback loop to patients who provide data, improving transparency about the research uses of data, and enabling patients to determine the policies that are applied to their data.

**Breakout Sessions**
Following the formal presentations, participants were assigned to participate in breakout groups to discuss: governance, data standards and interoperability, architecture and data exchange, privacy and ethical issues, methods, unconventional approaches, or incorporating patient-reported outcomes into electronic data.

Each group was then tasked with generating 3-4 actionable recommendations that support PCORI’s mission to promote the development of a sustainable infrastructure for conducting PCOR.

Recommendations were then ranked according to the following dimensions:
- Time horizon
- Cost
- Feasibility
- Suitability to PCORI’s mission
- Efficient use of existing resources and networks

Each group generated a “poster” showcasing its recommendations. The posters were displayed and all participants, using a controlled number of positive and negative votes, supported or opposed recommendations.

**Recommendations**
Through this process, the following top 10 recommendations were generated:

1. Establish governance guiding principles for a PCORI framework, with a focus on:
   a. Meaningful and representative patient engagement
   b. Data stewardship
   c. Dissemination of information
   d. Sustainability
2. Be the national leader in promoting meaningful and representative patient engagement in the governance of research networks (e.g., identify, advise, and train potential patient participants).

3. Support the development of Patient-Powered Rapid Learning Networks to advance knowledge for patients, clinicians and researchers and to improve outcomes. Patient-Powered Rapid Learning Networks were characterized as groups of patients with a common disease or condition who have come together to share information for research in order to improve care for their shared condition(s).

4. Ensure that these Patient-Powered Rapid Learning Networks are comprehensive, provide longitudinal data, support patient identity management, and data curation.

5. Create a platform that enables patients to share their perspectives on important research questions, and that brings patients and experts together to inform an evolving, patient-centered list of research priorities for PCORI.

6. Support the development of personalized evidence-based medicine and explore different methodologies for accomplishing this. Develop methods to develop an “n=1” research environment to investigate impact on patient experiences using diverse eData.

7. Lead efforts to identify effective strategies for promoting the use of patient-reported outcomes (PROs) in healthcare systems and personal health records. Support efforts that can lower barriers and incentives.

8. Support the development of data standards in healthcare in order to facilitate comparative effectiveness research studies across different health care systems.

9. Sponsor and advocate for the refinement and curation of clinical information models and associated value sets, and common data elements that merge clinical and research requirements.

10. Define policies about appropriate utilization of patient data

   a. Resolve issues around data use and access.
   b. Protect patient privacy and security.

The following are a list of recommendations that were also considered to be important but did not rise to the top ten:

- Seek to broadly understand the patient benefit in the network.
- Understand which groups engage and why, in order to ensure inclusiveness in the network.
- Conduct a survey of initiatives for implementing PROs in healthcare systems and personal health records.
- Explore IRB models that facilitate patient engagement.
- Support methods to develop a portfolio of studies to balance the trade-off between data quantity and validity, and to control for confounding variables in the data.
- Develop guidelines for EHR-based research reporting standards.
Operationalizing the Recommendations: Emerging themes from the breakout sessions

On the second day of the workshop, Harlan Krumholz, PCORI Board of Governors, and Steve Goodman, PCORI Methodology Committee, challenged the audience to think about the “adjacent possible,” that is, the concrete next steps for PCORI given the recommendations that were generated through the breakout sessions. This discussion focused primarily on three broad topics: governance, data infrastructure building blocks, and unconventional approaches to future strategic investments.

**Governance**

This discussion focused on governance as a two-part process. First it highlighted the importance of gathering a diverse group of stakeholders to define the role of patients in governance. Next, it considered establishing guidelines and minimal criteria for including patients in governance, as a prerequisite for network funding.

1. **PCORI should convene diverse groups of stakeholders to discuss network governance and sustainability.** Attendees recommended that PCORI seek to influence the field more broadly by bringing together different groups to help define governance, with particular emphasis on the role of patients. Attendees also saw a role for PCORI in stimulating the conversation on the sustainability of networks. They suggested ensuring that incentives were built into PCORI’s funding initiatives that would move the entire field of electronic health records toward a status that would become self-sustaining. Going forward, data networks and the research they facilitate will need to meet the needs of groups other than government agencies, which have traditionally been the funders of this work.

2. **PCORI should focus on establishing guidelines and training for patient engagement.** Many attendees recommended that PCORI establish minimal criteria for good governance practices for research networks that must be met in order to receive PCORI funding. One of these criteria should include meaningful and representative patient engagement in the development of governance policies. Attendees also recommended PCORI develop a scoring process so that researchers compete on the basis of how well they have partnered with patients. Furthermore, with each subsequent funding cycle, the level of patient engagement and partnering would be expected to rise. PCORI could also convene workshops for parties that want to submit letters of intent, in order to help them begin to think about how to partner with patients. PCORI can later re-convene its award recipients to determine how the process can be improved and to enable them to share their experiences.

The attendees envisioned a role for PCORI in identifying and activating more patients by helping to demonstrate the value of a national clinical data research network. Training for both patients and researchers was also viewed as a key opportunity for PCORI by many attendees.

**Data Infrastructure Building Blocks (Standards, Architecture, and Methods)**
Attendees agreed that resolving the issues related to standards, architecture, and methods will be critical to building a robust infrastructure. They acknowledged that PCORI can meaningfully contribute to moving this work forward by:

- Partnering with organizations that are pursuing initiatives on standardization of data elements (i.e. Office of the National Coordinator for Health Information) in order to integrate the patient perspective
- Providing leadership in merging clinical and research requirements
- Fostering the development of standards in the capture of healthcare data
- Prioritizing metadata and metadata standards to facilitate the use of data for secondary purposes
- Supporting methodological work in understanding and operationalizing patient experience and in supporting personalization (n-of-1 trials)
- Developing a framework for measuring data quality
- Sponsoring research to evaluate barriers to using patient reported outcomes
- Facilitating interoperability among EHRs and other data sources to help make data more complete, longitudinal and patient-centered
- Promoting user-centered design, by engaging designers in this process
- Evaluating strategies for engaging patients and assessing how information is ultimately used by patients

**Unconventional Approaches to Future Strategic Investments**

This discussion focused on two competing visions for a data infrastructure that could guide PCORI’s investments:

1. A National Patient-Centered Clinical Data Research Network
2. Rapid Learning Networks to improve outcomes and advance knowledge, which would build off of existing networks, becoming Patient-Powered Rapid Learning Networks

One central focus of this discussion was the vision of a National Data Patient-Centered Clinical Data Research Network. Building upon existing clinical data research networks, PCORI could help these networks transition to include a focus on improving outcomes that are measurable in real time and on advancing knowledge. PCORI could also work with these networks to address patient-centeredness and diversity. Others noted the possibilities in improving outcomes for patients with rare diseases by facilitating the identification of groups of patients that share a rare condition. Attendees also saw a role for PCORI in helping to understand the gaps in existing networks, and in developing a roadmap for filling those gaps. Perceived drawbacks to this approach included concerns about the considerable amount of time and money that would be required to build this national infrastructure, and the potential for the creation of a single National Data Network to discount the capabilities that exist today. Dr. Collins cautioned, however, that though this network would be resource-intensive to build, continuing to invest in networks that may not help move us toward the ideal could ultimately prove to be just as costly.
A second central focus of this discussion was Richard Platt’s vision of a “network of networks.” Based on the idea that the current platform need not be incongruent with a bold vision for the future, this vision supports the integration of existing clinical data research networks with “rapid learning networks”. The benefit of this approach would be the ability to unite patients with shared conditions, who are ready and willing to engage with researchers, and who may have already begun collecting some of their data electronically.

Throughout this discussion, patient representatives clearly communicated that there are patients who are enthusiastic about the opportunity to share their data to help realize the opportunities presented by data networks to improve outcomes and advance knowledge. Attendees saw an important role for PCORI in reaching broad and diverse groups of patients, and in finding a way to harness the tremendous wealth of information that exists in online patient communities.

One recommendation for an immediately actionable item that emerged from the workshop was for PCORI to produce an inventory of existing data networks. This inventory would serve to identify gaps in the landscape and thus help PCORI determine where future investments would be most beneficial.

**Key Takeaways: What Would a National Patient-Centered Clinical Data Research Network Look Like?**

For PCORI, data infrastructure would ideally be flexible enough to address important gaps that currently exist in supporting PCOR. Therefore it would possess several characteristics such as:

- **Coverage of large, diverse populations:** for conducting research in patient subgroups or in rare disease populations
- **Capability of complete capture of longitudinal data:** to capture the total experience of patients over an extended period of time
- **Capability to capture and link clinical data to patient-reported data:** to capture data on patient preferences and experiences and link them to clinical data
- **Inclusion of patient and clinician engagement in governance:** to involve patients and clinicians in determining who has access to the data and for what purposes the data can be used
- **Affordability:** prior efforts to develop data infrastructures have proven to be extraordinarily costly. Efficiency is needed.
- **Ability to link back to health systems:** in order to disseminate research findings rapidly, a data network needs to be bi-directional and link back to clinicians and patients
- **Capable of randomization (individual and cluster):** to conduct scientifically rigorous research.
Next Steps

During the two-day workshop, new visions for the use of electronic health data to advance PCOR began to emerge. Next steps for PCORI staff and leadership will be to determine how PCORI can continue to advance the vision outlined in Palo Alto and, in doing so, fully and meaningfully engage patients and caregivers.
Appendix A: Agenda

Monday, July 2, 2012

8:00 – 8:15 a.m. Welcome and Introduction
Steven Goodman, MD, MHS, PhD, Associate Dean for Clinical and Translational Research, Stanford University School of Medicine, Member, PCORI Methodology Committee

8:15 – 8:45 a.m. What Are We Looking For?
Joe Selby, MD, MPH, Executive Director, PCORI
PCORI’s “Desirable Characteristics” in developing data infrastructure for the conduct of patient-centered outcomes research

8:45 – 9:00 a.m. A Vision for a National Research Network
Francis Collins, MD, PhD, Director, National Institutes of Health, Member, PCORI Board of Governors

9:00 – 9:45 a.m. Survey of the Landscape – Governmental, NGO, Methodological Perspectives
Erin Holve, MPH, MPP, PhD, Director of Research and Education in HSR, Academy Health
Lucila Ohno-Machado, MD, PhD, Chief of the Division of Biomedical Informatics, Associate Dean for Informatics and Technology, University of California, San Diego

9:45 – 10:10 a.m. Questions and Discussion

10:10 – 10:30 a.m. Break

10:30 am – 11:30 a.m. Lessons from the Field
Sebastian Schneeweiss, MD, ScD, Associate Professor, Harvard Medical School, Department of Epidemiology, Member, PCORI Methodology Committee

Case Studies
HMO Research Network (HMORN) – Eric Larson, MACP, MD, MPH, Executive Director, Group Health Research Institute
Sentinel – Patrick Archdeacon, MD, Medical Officer, Food and Drug Administration

Respondents
Regina Greer-Smith, MPH, FACHE, Healthcare Coordinator, Enhanced Medical Services Collaboration
Deven McGraw, JD, LLM, MPH, Director of Health Privacy, Center for Democracy and Technology
Richard Platt, MD, MSc, Director Harvard Pilgrim Healthcare/DEcIDE Network, HMO
Gurvaneet Randhawa, MD, MPH, Program Officer, Agency for Healthcare Research and Quality

11:30am – 12:30pm Lessons from the Field
Robin Newhouse, PhD, RN, FAAN, NEA-BC, Chair and Associate Professor, University of Maryland School of Nursing, Member, PCORI Methodology Committee
Case Studies
DARTNet – David West, PhD, Director Colorado Health Outcomes
SCANNER – Michele Day, PhD, Program Manager, SCANNER

Respondents
Suzanne Bakken, DNSc, RN, FAAN, FACMI, Professor,
Director for the Center for Evidence-Based Practice in the Underserved, Columbia University
Doug Fridsma, MD, PhD, Chief Scientist, Office of the National Coordinator for Health Information Technology
Tracy Lieu, MD, MPH, Director, Division of Research, Kaiser Permanente

12:30 – 1:30 p.m. Lunch

1:30 – 1:50 p.m. Survey of the Landscape – Industry Perspective
Bob Kocher, MD, Partner, Venrock

1:50 – 2:50 p.m. Lessons from the Field
Richard Kuntz, MD, MSc, Senior Vice President and Chief Scientific, Clinical and Regulatory Officer, Medtronic, Member, PCORI Board of Governors

Case Studies
C3N – Peter Margolis, MD, PhD, Co-Director, Center for Health Care Quality, Director of Research, James M. Anderson Center, Cincinnati Children’s Hospital
Registry of Registries – Rich Gliklich, MD, President, Quintiles Outcomes

Respondents
Basit Chaudhry, MD, PhD, Medical Scientist, IBM
Ben Heywood, MBA, President, PatientsLikeMe
Robert Jesse, MD, PhD, Principal Deputy Under Secretary for Health, Department of Veterans Affairs, Member, PCORI Board of Governors

2:50 – 3:10 p.m. Break

** Breakout Groups and Poster Session will not be Webcast **

3:10 – 5:10 p.m. Breakout Groups:
Governance – Including models of governance structure, and key issues such as data ownership and data availability
Data Standards and Interoperability
Architecture
Privacy and Ethical Issues – Including IRB issues, informed consent, and HIPAA
Methods – With an eye towards overcoming imperfect data
Unconventional Approaches
Incorporating Patient Reported Outcomes

5:10 – 5:55 p.m. Break – Campus Walk Optional
5:55 – 7:00 p.m. Poster Session

7:00 – 9:00 p.m. Dinner

Tuesday, July 3, 2012

8:00 – 8:15 a.m. Welcome, Review of Agenda and Recap of Poster Session
Steven Goodman, MD, MHS, PhD, Associate Dean for Clinical and Translational Research, Stanford University School of Medicine, Member, PCORI Methodology Committee

8:15– 10:45 a.m. Charting the Course – Exploring Top Proposals from Poster Sessions
Harlan Krumholz, MD, Harold H. Hines Jr. Professor of Medicine, Yale University School of Medicine, Member, PCORI Board of Governors

10:45 – 11:00 a.m. Break

11:00 – 11:45 a.m. PCORI-Attendee Dialogue
Steven Goodman, MD, MHS, PhD, Associate Dean for Clinical and Translational Research, Stanford University School of Medicine, Member, PCORI Methodology Committee

11:45 a.m. – 11:50 p.m. Wrap Up and Adjourn
Appendix B: Participants

1. **Patrick Archdeacon**, Federal Drug Administration (FDA), Medical Officer, Office of Medical Policy with the Center for Drug Evaluation and Research (CDER)
2. **Naomi Aronson**, Blue Cross and Blue Shield, Executive Director, Association Technology Evaluation Center
4. **Suzanne Bakken**, Columbia University, Professor, Director for the Center for Evidence-Based Practice in the Underserved
5. **David Bates**, Partners Healthcare, Medical Director of Clinical and Quality Analysis, Information Systems
6. **Anne Beal**, PCORI, Chief Operating Officer
7. **Lawrence Becker**, Xerox Corporation, Director of Strategic Partnerships and Alliances
8. **Chris Brancato**, Deloitte Consulting LLP, Specialist Leader, Deloitte Federal Health
9. **Sallie Bernard**, Safeminds, President
10. **Atul Butte**, Stanford, Chief of the Division of Systems Medicine and Associate Professor of Pediatrics
11. **Tanisha Carino**, Avalere Health, Senior Vice President
12. **Basit Chaudhry**, IBM, Medical Scientist
13. **Christopher Chute**, Mayo Clinic, Founding Chair of the Division of Biomedical Informatics
14. **James Cimino**, NIH, Clinical Center Chief, Laboratory for Informatics Development
15. **Carolyn Clancy**, Agency for Health Research and Quality (AHRQ), Director
16. **Francis Collins**, National Institutes of Health (NIH), Director
17. **Stephen Coons**, Patient-Reported Outcome (PRO) Consortium at the Critical Path Institute, Executive Director
18. **Christine Cox**, Centers for Medicare and Medicaid Services (CMS), Director, Information Dissemination Group
19. **Robert Croyle**, NIH, Director of the Division of Cancer Control and Population Sciences
20. **Gregory Daniel**, Brookings, Managing Director, Engelberg Center for Health Care Reform
21. **Michele Day**, University of California – San Diego, Program Manager & Technical Writer, Division of Biomedical Informatics
22. **Scott Defoe**, Medtronics Inc., Marketing Director
23. **Peter DeVault**, EPIC, Director of Enterprise Integration and Interoperability
24. **Allen Douma**, Empower, LLC., CEO
25. **Arnold Epstein**, Harvard University School of Public Health, John H. Foster Professor and Chair of the Department of Health Policy
26. **Ian Eslick**, Lybba, Research Fellow
27. **John Feikema**, Health and Human Services - Office of the National Coordinator (HHS/ONC), Query Health/S&I Framework Coordination
28. **Kevin Fickenscher**, American Medical Informatics Association (AMIA), President & CEO
29. **Terry Field**, University of Massachusetts, Meyers Primary Care Institute, Associate Director
30. **Rachael Fleurence**, PCORI, Scientist
31. **Doug Fridsma**, HHS/ONC – Director, Office of Standards and Interoperability
32. **Gilles Frydman**, Association of Cancer Resources Online, Founder
33. **Sherine Gabriel**, Mayo Clinic, Professor of Medicine and Epidemiology and the William J. and Charles H. Mayo Professor
34. **Richard Gliklich**, Quintiles Outcome, President
35. **Steven Goodman**, Stanford University School of Medicine, Associate Dean for Clinical and Translational Research
36. **Regina Greer-Smith**, Enhanced Medical Services Collaboration, Healthcare Consultant
37. **Harry Greenspun**, Deloitte Consulting LLP, Senior Advisor for the Healthcare Transformation and Technology at the Deloitte Center for Health Solutions
38. **Robert Harrington**, Stanford University, Chair, Department of Medicine
39. **Trevor Hastie**, Stanford University, Professor of Statistics and Biostatistics
40. **Brandon Hayes-Lattin**, Lance Armstrong Foundation, Senior Medical Advisor
41. **William Hersh**, Oregon Health Sciences University, Chair Informatics & Epidemiology
42. **Ben Heywood**, PatientsLikeMe, President
43. **Susan Hildebrandt**, PCORI, Director of Stakeholder Engagement
44. **Erin Holve**, Academy Health, Director of Electronic Data Methods (EDM)
45. **Mark Hornbrook**, Kaiser Permanente Center for Health Research, Health Economist and Chief Scientist
46. **George Hripcsak**, Columbia University, Chair of the Department of Biomedical Informatics
47. **Michael Huerta**, NIH - National Institute of Mental Health, Associate Director
48. **Gail Hunt**, National Alliance for Caregiving, President and CEO
49. **Robert Jesse**, Department of Veterans Affairs, Principal Deputy Under Secretary
50. **Michael Johnsrud**, Avalere Health, Senior Vice President & Principal Scientist
51. **Michael Kahn**, University of Colorado Medical School, Professor
52. **Sarah Knight**, VA, Deputy Director VA HSR&D
53. **Bob Kocher**, Venrock, Partner
54. **Harlan Krumholz**, Yale University School of Medicine, Harold H. Hines, Jr. Professor of Medicine and Epidemiology and Professor of Public Health
55. **Richard Kuntz**, Medtronic, Inc., Senior Vice President and Chief Scientific, Clinical, and Regulatory Officer
56. **Rebecca Kush**, Clinical Data Interchange Standards Consortium, President and CEO
57. **Clete Kushida**, Stanford University Sleep Disorders Clinic, Director (Acting)
58. **Marianne Laouri**, Deloitte Consulting LLP, Specialist Leader
59. **Eric Larson**, Group Health Research Institute, Vice President for Research, Group Health Executive Director & Senior Investigator
60. **Tracy Lieu**, Kaiser Permanente, Director of Division of Research
61. **Bernard Lo**, University of California San Francisco (UCSF) & Greenwall Institute, Professor, Chair of Medical Ethics & President (Greenwall)
62. **Henry Lowe**, Stanford, Director of Clinical Research Informatics
63. **Janet Marchibroda**, Bi-partisan Policy Institute, Chair, Health Affairs & Health IT Initiative
64. **Peter Margolis**, Cincinnati Children’s Hospital, Co-Director, Center for Health Care Quality, Director of Research, James M. Anderson Center
65. **Ross Martin**, Deloitte Consulting LLP, Specialist Leader, Deloitte Federal Health
66. **Michael McGinnis**, Institute of Medicine, Senior Scholar
67. **Deven McGraw**, Center for Democracy and Technology, Director of Health Privacy
68. **David Meltzer**, The University of Chicago, Chief - Section of Hospital Medicine, Director of the Center for Health and the Social Sciences, and Chair of the Committee on Clinical and Translational Science
69. **Kristi Mitchell**, Avalere Health, Vice President
70. **Bob Nease**, Express Scripts, Chief Scientist
71. **Jack Needleman**, University of California Los Angeles, Professor of Health Services
72. **Robin Newhouse**, University of Maryland School of Nursing, Assistant Dean for the Doctor of Nursing Practice Program and Associate Professor, Organizational Systems and Adult Health
73. **Sharon-Lise Normand**, Harvard Medical School, Professor of Health Care Policy (Biostatistics)
74. **Lucila Ohno-Machado**, UC San Diego, Founding Chief, Division of Biomedical Informatics & Associate Dean for Informatics; Director, Biomedical Research Informatics for Global Health Program
75. **Sally Okun**, Patients Like Me, Clinical / Discovery & Informatics Team
76. **Marc Overhage**, Siemens Health Services, Chief Medical Informatics Officer
77. **Thomas Payne**, University of Washington, Medical Director, Information Technology Services
79. **Gurvaneet Randhawa**, AHRQ, Senior Advisor/EDM
80. **Thomas Scarcenocchia**, Observational Medical Outcomes Partnership (OMOP), VP/Chief Technology Officer
81. **Sebastian Schneeweiss**, Harvard Medical School, Associate Professor of Medicine and Epidemiology
82. **Jean Ann Seago**, University of California San Francisco, Professor of Community Health Systems
83. **Joe Selby**, PCORI, Executive Director
84. **Nigam Shah**, Stanford Medical School, Head of the Biomedical Informatics Division
85. **Gail Shearer**, PCORI, Senior Advisor
86. **Susan Sheridan**, PCORI, Deputy Director of Patient Engagement
87. **Bill Silberg**, PCORI, Director of Communications
88. **Jamie Skipper**, HHS-ONC, Policy Analyst
89. **Jean Slutsky**, AHRQ, Director of the Center for Outcomes and Evidence (COE)
90. **Melissa Stern**, PCORI, Director of Strategic Initiatives
91. **Buzz Stewart**, Sutter Health, Vice President and Chief Research and Development Officer
92. **Paul Tang**, Palo Alto Medical Foundation, Vice President, Chief Innovation and Technology Officer

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93. **Jay Tenenbaum**, Cancer Commons, Founder & Chairman
94. **Betsy Thompson**, CMS, Chief Medical Officer, Region IX
95. **Debbie Travers**, University of North Carolina-Chapel Hill, Assistant Professor of Health Care Systems and Emergency Medicine
96. **Sean Tunis**, Center for Medical Technology Policy, President
97. **Sandy Walsh**, California Breast Cancer Organizations, President
98. **Rayneisha Watson**, Deloitte Consulting LLP, Senior Consultant
99. **Harlan Weisman**, Johnson & Johnson, Chief Science and Technology Officer, Medical Devices and Diagnostics
100. **Natalie Wegener**, PCORI, Program Coordinator for Strategic Initiatives
101. **David West**, DARTNet & Colorado Health Outcomes, Head
102. **Jon White**, AHRQ, Lead, IT Portfolio
103. **Andrew Wiesenthal**, Deloitte Consulting LLP, Director
104. **John Wilbanks**, UC Berkley/Creative Commons, Vice President, Science & Creative Commons
105. **Pierre Yong**, HHS, Office of Assistant Secretary for Planning and Evaluation, Acting Director, Division of Health Care Quality and Outcome
106. **Roni Zeiger**, Impatient Science, Co-Founder and President
107. **Judy Zerzan**, Colorado Medicaid Medical Director, Medicaid Medical Director for the Colorado Department of Health Care Policy & Financing
Appendix C: Recommendations from Breakout Groups

Each breakout group was charged with prioritizing three to four recommendations for PCORI. During the breakout sessions, several additional recommendations were discussed. This list contains all the prioritized recommendations from each breakout session which were submitted for voting.

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| Governance | Considered topics related to research network partnerships, the structures that govern those partnerships, and data sharing and data access agreements. While privacy and security are also entwined with governance, this topic was considered in a separate breakout group. | ▪ Establish PCORI criteria for governance with a focus on:  
  - Meaningful and representative patient engagement  
  - Data stewardship  
  - Dissemination of information  
  - Sustainability  
  ▪ Be the national leader to ensure meaningful and representative patient engagement in research networks’ governance  
  ▪ Define mechanism to authorize use of data for PCOR purposes:  
    - Policies to vet and approve use of network resources at level of specific protocols and investigators  
    - Define expectations of data holder and networks that perform PCORI sponsored research |
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| Data Standards & Interoperability | Considered the array of approaches to make meaningful use of data from different sources. Topics for discussion included common data models, technical solutions to achieve interoperability and the role of vendors. | ▪ Sponsor and advocate for refinement and curation of clinical information models and associated value sets, common data elements that merge clinical and research requirements (Goal-consistent structure and names for clinical info)  
▪ Sponsor and advocate for the development of instruments for collecting comparable and consistent patient-reported outcomes  
▪ Sponsor and advocate for development of data standards about the care environment in order to facilitate the analysis of care options |
| Architecture & Data Exchange    | Addressed what architecture is most suited to achieving PCORI’s goals and what are the challenges and requirements to realizing such a network.                                                              | ▪ Build a system with a patient-centered longitudinal view  
▪ Prioritize identity management  
▪ Advocate for data curation                                                                                                           |
| Privacy & Ethical Issues        | Tackled the issue of the role of the IRB and the HIPAA privacy rule particularly as they relate to establishing multi-site research networks.                                                                         | ▪ Seek to broadly understand patients  
▪ Understand which groups engage and why to ensure inclusiveness  
▪ Promote research that defines/supports patient engagement  
▪ Explore IRB models that facilitate patient engagement  
▪ Identify best practices from other industries                                                                                       |
| Methods                         | Addressed both the analytic and informatics methodological challenges in using the large volume of electronic data now available, particularly methods that validate data quality or overcome the problems of imperfect data. | ▪ Support methods to develop a portfolio of studies to balance the eData trade-off and develop methods to assess the level of control of confounding in the data  
▪ Develop a manual for EHR based research reporting standards  
▪ Develop methods to support integration of health practice research as part of the research portfolio (system level change)  
▪ Develop methods for an n=1 research environment to investigate impact on |
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| Unconventional Approaches      | Addressed innovative approaches that may look very different from the current models. | ▪ Establish a national network of patients, researchers, providers to which patients could opt in – a platform for efficient CER supporting diversity and patient governance  
▪ Ask patients what they think are the most important research questions and create a transparent, dynamic list of PCORI’s research priorities, with explanations that incorporate patient and expert input  
▪ Create rapid learning networks to improve outcomes and advance knowledge for patients, clinicians and researchers |
| Incorporating Patient Reported Outcomes (PROs) into Electronic Data | Tackled the question of how to assure that PROs are available for comparative analysis: what are the most reliable, effective and efficient ways of capturing PROs or linking them to electronic data. | ▪ Conduct survey of initiatives for implementation of PROs in healthcare systems & PHRs  
▪ Identify what works at the point of care for the patient and clinician - What is missing from the patient’s point of view?  
▪ Identify and address barriers and incentives for developing and using PROs in healthcare systems and PHRs |