



Real-World Data for Clinical Research: A PCORnet Workshop with the Pharmaceutical and Biologics Industry Meeting Summary

Overview

On March 30, 2015, members of the PCORnet Executive Steering Committee and PCORI leadership joined participants from the pharmaceutical and biologics industry for a one-day workshop on [The National Patient-Centered Clinical Research Network \(PCORnet\)](#). [Participants](#) included over 40 industry representatives. The [agenda](#) was designed to elicit discussions to drive the development of PCORnet as a resource for industry research. PCORI and PCORnet leaders gave presentations about network progress, and the planned demonstration projects were used to illustrate PCORnet's anticipated capabilities.

Key Questions

Throughout the day, discussions centered around data standards and data quality, assessing and mitigating heterogeneity of treatment effects, ensuring meaningful dissemination, early engagement from key stakeholders, and PCORnet's capabilities and operations. The questions below represent the breadth of the questions generated during the meeting for each major discussion area throughout the day. PCORI will endeavor to address these questions and post answers on our website.

Critical Importance of Data Standards and Data Quality

- How generalizable is the data?
- Sample sizes and data availability: how many patients by condition, demographics, etc.?
- Will PCORnet data be linked with Sentinel, or will claims data be otherwise integrated?
- How can data elements be added to the Common Data Model (CDM)?
- How does PCORnet attain longitudinal data for patients that switch plans?
- Does PCORI have standards or guidance with respect to which PRO measures have the best evidence for validity or is it up to each network as to which ones are most relevant?
- What biospecimens will be available?
- Is it possible to leverage the incorporation of genomic data into PCORnet to avoid duplication of efforts?
- Is there an intersection between patients in PPRNs and patients in CDRNs?
- How will decisions be made for the next wave of disease states being targeted?

A Need to Assess and Mitigate Heterogeneity of Treatment Effects Through a Deep Understanding of the Underlying Data

- Integration of biomarker data: what processes are in place for consistency across network physicians in ordering tests?
- Look at matching, propensity scoring, need to understand who patients are regardless of disease; can we identify cohorts that we can compare and draw conclusions on?
- Are multiple heterogeneity estimates combined or explored?

Importance of Ensuring Meaningful Dissemination and Early Engagement from Key Stakeholders

- How do publication programs work, especially with multiple sites?
- What is the potential for PCORnet to collaborate across external bodies such as FDA and industry?
- How would industry stakeholders be involved in developing a PCORnet agenda that represents all stakeholders' needs? Could there be an advisory body of industry leaders?

A Need for Increased Information on PCORnet's Capabilities and Operations

- What is the definition of a PCORnet study?
- When will PCORnet be ready for a request for a study and how will decisions be made?
- What questions can become PCORI questions vs. questions from external funders?
- Is the intent for PCORnet to move to a "service provider" role?
- How do researchers get to use the data?
- What are the regulatory reporting requirements, and who is responsible for reporting?
- How much control do funders have over the questions, including methods, analysis, and dissemination?
- What is the status of policies for IRB, consenting individuals, centralized contracting?

Information Requests

In addition to raising key questions to shape future conversation regarding PCORnet, specific requests for information included:

- Information describing the purpose, identification, and execution of **pre-research queries**.
- Descriptions of the **unique versus universal capabilities** of individual CDRNs and PPRNs, and the overall capabilities of PCORnet as a national network.
- Guidance on **the scope of research that will be suitable for PCORnet**, both for the capabilities of PCORnet now and in the future.

Moving Forward

The workshop raised many key questions and indicated an overall need for more publicly available guidance materials regarding the operations, principles, and priorities of PCORnet. Some of the questions raised during the workshop can and will be answered now, and that information will be made available on www.pcornet.org. Other questions will inform the development of PCORnet and stimulate areas of network collaboration.

Moving forward, PCORI plans to reconvene the pharmaceutical and biologic industry workshop participants in about six months. In the interim, PCORnet will work to develop disease- or drug-based use cases, and PCORI will create a draft framework for a sponsorship model, and additional information will be made publicly available to industry partners. PCORI will also convene interested attendees from PCORnet and industry to conduct workshops to identify concrete pilot studies and other tangible steps.