NIH Collaboratory *Rethinking Clinical Trials®*

Health Care Systems Research Collaboratory

# EHR Core: Accomplishments, Impact, and Future Directions

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NIH Collaboratory Steering Committee Meeting
May 15, 201

# Outline

- Participants and Mission
- Past Activities and Lessons Learned
- Greatest Impact to Date
- Important Activities Moving Forward
- Comments/Discussion

# Participation

## Core leaders:

| | |
|---|---|
| **Rachel Richesson** | Duke University |
| **Greg Simon** | Kaiser Permanente |

## Coordinating Center Support:

| | |
|---|---|
| **Jesse Hickerson** | Project Manager |
| **Tammy Reece** | Project Director |
| **Karen Staman** | Senior Writer |

## Members:

| | |
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| **Nick Anderson** | University of Washington |
| **Alan Bauck** | Kaiser Permanente |
| **Denise Cifelli** | University of Pennsylvania |
| **Lesley Curtis** | Duke University |
| **Pedro Gozalo** | Brown University |
| **Bev Green** | Kaiser Permanente |
| **Ed Hammond** | Duke University |
| **Lauren Heim** | University of California Irvine |
| **Susan Huang** | University of California Irvine |
| **Michael Kahn** | Children's Hospital of Colorado |
| **Reesa Laws** | Kaiser Permanente |
| **Julie Lima** | Brown University |
| **Rosemary Madigan** | University of Pennsylvania |
| **Meghan Mayhew** | Kaiser Permanente |
| **Vincent Mor** | Brown University |
| **Brett Moran** | University of Texas Southwestern |
| **George "Holt" Oliver** | University of Texas Southwestern |
| **Jon Puro** | OCHIN |
| **Jerry Sheehan** | National Library of Medicine |
| **Kari Stephens** | University of Washington |
| **Erik Van Eaton** | University of Washington |
| **Ferdinand Velasco** | Texas Health Resources |
| **Barbara Wells** | National Heart, Lung, and Blood Institute |
| **Wolfgang Winkelmayer** | Baylor University |

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# Mission

- Share experiences using clinical information systems and EHRs to support research
- Identify generalizable approaches and best practices to promote consistent and practical methods for using clinical data to advance healthcare research
- Suggest where tools are needed
- Explore and advocate for cultural and policy changes related to the use of EHRs for identifying populations for research, including measures of quality and sufficiency

- Various use cases for clinical phenotype definitions
- Purpose determines the ideal definition; validation required
- Sharing definitions can reduce variation
- Information must be targeted to different audiences (clinical and technical)
- Lack of standardized EHR data is a major challenge

Perspective

# Electronic health records based phenotyping in next-generation clinical trials: a perspective from the NIH Health Care Systems Collaboratory

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**ABSTRACT**

Widespread sharing of data from electronic health records and patient-reported outcomes can strengthen the national capacity for conducting cost-effective clinical trials and allow research to be embedded within routine care delivery. While pragmatic clinical trials (PCTs) have been performed for decades, they now can draw on rich sources of clinical and operational data that are continuously fed back to inform research and practice. The Health Care Systems Collaboratory program, initiated by the NIH Common Fund in 2012, engages healthcare systems as partners in discussing and promoting activities, tools, and strategies for supporting active participation in PCTs. The NIH Collaboratory consists of seven demonstration projects, and seven problem-specific working group 'Cores', aimed at leveraging the data captured in heterogeneous 'real-world' environments for research, thereby improving the efficiency, relevance, and generalizability of trials. Here, we introduce the Collaboratory, focusing on its Phenotype, Data Standards, and Data Quality Core, and present early observations from researchers implementing PCTs within large healthcare systems. We also identify gaps in knowledge and present an informatics research agenda that includes identifying methods for the definition and appropriate application of phenotypes in diverse healthcare settings, and methods for validating both the definition and execution of electronic health records based phenotypes.

**INTRODUCTION**

The US healthcare system is poised to significantly enhance the relevance, number, speed, and cost-effectiveness of clinical trials by embedding them directly within the healthcare delivery system. This transformation[1] will be enabled by capabilities offered by electronic health records (EHRs) and patient-reported outcomes (PROs), changes in the organization and delivery of healthcare, and cooperation toward the development of a *learning health system*[2 3] in which evidence generated in clinical settings is routinely examined to inform research and practice. Widespread electronic collection of operational and clinical data[4] have enhanced the potential for *pragmatic clinical trials* (PCTs), randomized controlled trials designed for broad generalizability, typically using multiple clinical sites and broader eligibility criteria.[5] In contrast to explanatory trials, for which the goal is to detect biological effects of new treatments, PCTs are designed to support clinical decision-making by evaluating interventions in 'real-world' practice conditions.[6] PCTs therefore recruit participants from heterogeneous practice settings, and pose challenges for reconciling the variation in healthcare operations, widely disparate information systems, and differences in data capture fidelity. The routine implementation of PCTs is a key element in achieving the vision of the learning health system,[7] but achieving this on a global scale will require innovations, including new ethical frameworks to assess consent and risk,[8 9] new methodologies to work with observational data, and more effective partnerships among healthcare systems.

Advancing our understanding and ability to conduct PCTs within healthcare systems using innovative approaches is a key focus of the NIH Collaboratory. The use of EHRs to support trial activities, including the identification of patient cohorts with precise clinical attributes, is an important component of this vision and the next generation of clinical trials. The Collaboratory is leveraging previous work in phenotype definition and execution, and adding new use cases and requirements to inform the practice of using EHRs for research, advancing the science for both informatics and evidence-based healthcare. In the following sections, we will describe the NIH Collaboratory and the Phenotype, Data Standards, and Data Quality (PSQ) 'Core' working group, including their early experiences with EHR data queries, standards considerations, and data quality activities. We will conclude with a proposed research agenda and suggested future directions.

**THE NIH COLLABORATORY**

In 2012, The NIH Common Fund initiated the Health Care Systems Collaboratory (https://www.nihcollaboratory.org/) program to engage healthcare systems as partners in efforts to improve research efficiency and relevance to patients, clinicians, and the healthcare community—and in doing so, advance the national capacity for conducting large, cost-effective studies. The Collaboratory is not a traditional research network but rather a participatory forum, developing and integrating practices that allow diverse healthcare systems to participate in clinical research. It also supports the

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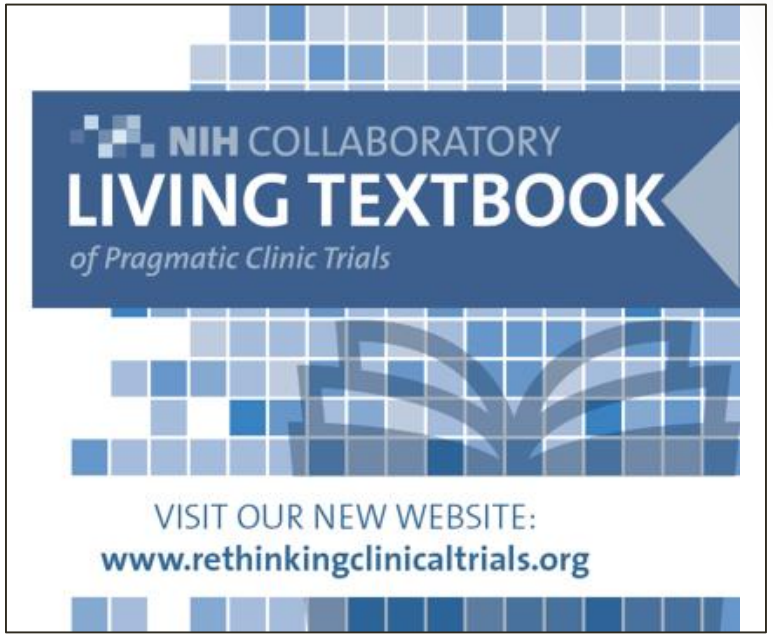
# Living Text Sections

- Using EHR Data in PCTs
- Clinical Phenotyping
- Data Quality
- Data Standards
  - ICD-10
  - RxNorm and Medication Data
- Demonstration project approaches and case studies

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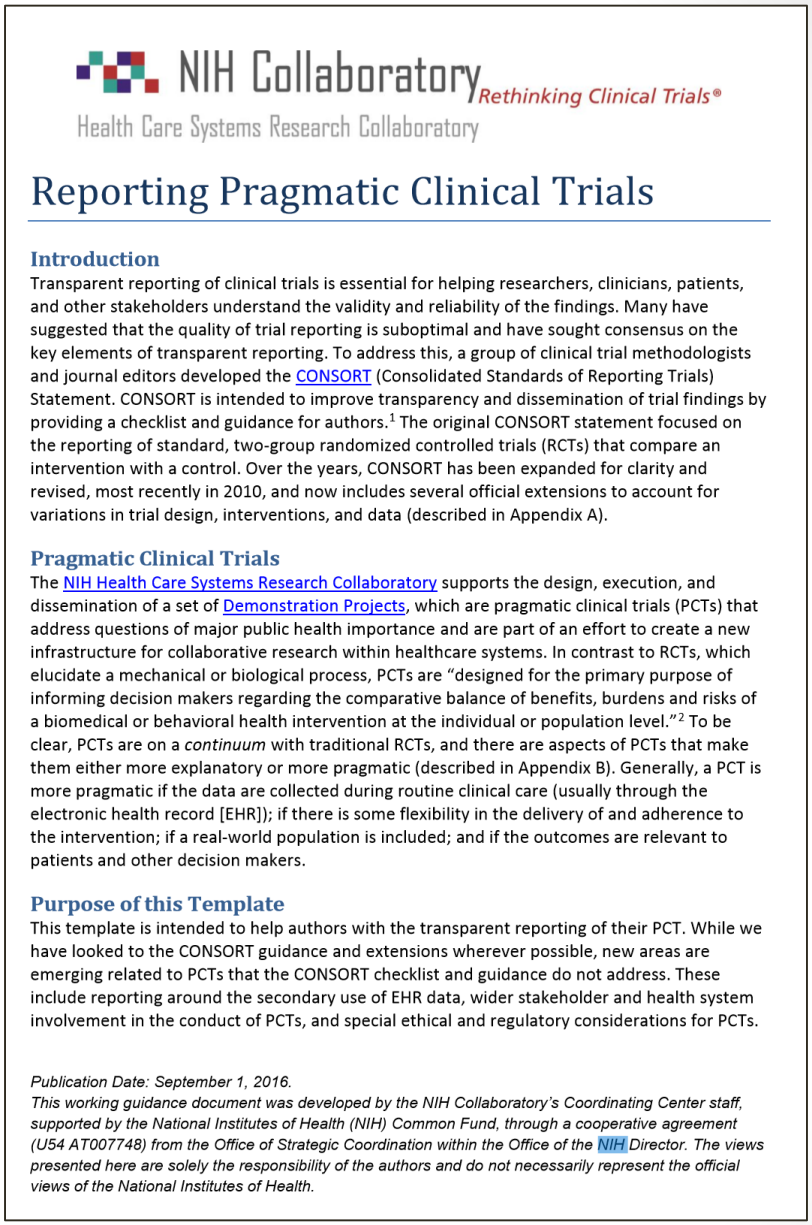
# PCT Reporting Template

PSQ Core additions to the proposed guidance for reporting results from pragmatic trials.

*Find a copy of the PCT Reporting Template in your meeting materials*



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## Reporting Pragmatic Clinical Trials

### Introduction

Transparent reporting of clinical trials is essential for helping researchers, clinicians, patients, and other stakeholders understand the validity and reliability of the findings. Many have suggested that the quality of trial reporting is suboptimal and have sought consensus on the key elements of transparent reporting. To address this, a group of clinical trial methodologists and journal editors developed the CONSORT (Consolidated Standards of Reporting Trials) Statement. CONSORT is intended to improve transparency and dissemination of trial findings by providing a checklist and guidance for authors.[1] The original CONSORT statement focused on the reporting of standard, two-group randomized controlled trials (RCTs) that compare an intervention with a control. Over the years, CONSORT has been expanded for clarity and revised, most recently in 2010, and now includes several official extensions to account for variations in trial design, interventions, and data (described in Appendix A).

### Pragmatic Clinical Trials

The NIH Health Care Systems Research Collaboratory supports the design, execution, and dissemination of a set of Demonstration Projects, which are pragmatic clinical trials (PCTs) that address questions of major public health importance and are part of an effort to create a new infrastructure for collaborative research within healthcare systems. In contrast to RCTs, which elucidate a mechanical or biological process, PCTs are "designed for the primary purpose of informing decision makers regarding the comparative balance of benefits, burdens and risks of a biomedical or behavioral health intervention at the individual or population level."[2] To be clear, PCTs are on a *continuum* with traditional RCTs, and there are aspects of PCTs that make them either more explanatory or more pragmatic (described in Appendix B). Generally, a PCT is more pragmatic if the data are collected during routine clinical care (usually through the electronic health record [EHR]); if there is some flexibility in the delivery of and adherence to the intervention; if a real-world population is included; and if the outcomes are relevant to patients and other decision makers.

### Purpose of this Template

This template is intended to help authors with the transparent reporting of their PCT. While we have looked to the CONSORT guidance and extensions wherever possible, new areas are emerging related to PCTs that the CONSORT checklist and guidance do not address. These include reporting around the secondary use of EHR data, wider stakeholder and health system involvement in the conduct of PCTs, and special ethical and regulatory considerations for PCTs.

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https://www.nihcollaboratory.org/Products/PCT%20Reporting%20Template-2017-01-26.pdf

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# Reporting Specifications for PCTs

- How the population was identified
- Clinical phenotype definitions
  - location to obtain the detailed definitional logic
  - use public repository, e.g., PheKB, NLM VSAC, GitHub
- Data quality assessment methods
- Data management activities during the study, including description of data sources or processes used at different sites, linkage, etc.
- Plan for archiving or sharing the data after the study, including specific definitions for clinical phenotypes and specifications for coding system

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Perspective

# Pragmatic (trial) informatics: a perspective from the NIH Health Care Systems Research Collaboratory

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- Competition for IT resources
- Need to optimize clinical data for research purposes
- Only small proportion of research in EHRs

- Need to capture intervention or control activities
  - Including standard of care
- Need to enable learning & research activities into EHR functions

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https://academic.oup.com/jamia/article/24/5/996/3069877/Pragmatic-trial-informatics-a-perspective-from-the

# Lessons Learned from Collaboratory PCTs

- Not all data needed for trial in EHR
- New data collection difficult
- Data quality issues
- Data transformation issues
- Dynamic and ongoing

# Lessons Learned from Collaboratory PCTs

- Difficult to assimilate data across organizations
  - requires a reference standard
  - requires local data & systems experts
- "Data sharing" is not straightforward in PCTs; has implications for providers and health systems (Greg Simon will address later)
- Resource sharing is important and feasible for our core

# Greatest Impact – "Success Factors for PCTs"

- Organizational "Research Readiness"
- Dedicated and or research-trained data operations staff
- Existence of resources that can support multiple research and QI studies

# Greatest Impact – "Success Factors for PCTs"

- Existence of research processes or resources that can enable research and/or enable the site to join multi-site research projects
  - e.g., use of reference standards (e.g., LOINC or RxNorm) enable faster and more consistent roll-out of definitions, eligibility/enrollment, implementation tools, etc.
- The availability of standard clinical data elements and research functions in EHRs will facilitate conduct of PCTs and sustainability of interventions

# Future Impact – Promote and Promulgate "Success Factors" for PCTs

- Validate checklist for assessing "research readiness"
- Measure and promote research readiness in organizations
- Identify and share tools and best practices ("Roadmap" papers from each demonstration project)
- Advocate for standards and functionality in EHRs to support PCTs

# Feedback

- Charter and mission  (next slide)

- Priority tasks or products for the next year:
  - Checklist for resource and data sharing package
  - Checklist or tool for assessing “research readiness”
  - Others?????

# Mission -- for discussion

- Share experiences using clinical information systems and EHRs to support research
- Identify generalizable approaches and best practices to promote consistent and practical methods for using clinical data to advance healthcare research
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*Thank you*