NIH Collaboratory *Rethinking Clinical Trials®*

Health Care Systems Research Collaboratory

Thoughts from the **P**henotypes, Data **S**tandards & Data **Q**uality Core

Rachel Richesson, PhD, MPH
Duke University School of Nursing

NIH Collaboratory Grand Rounds
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Members

Alan Bauck, Kaiser Permanente Center for Health Research

Denise Cifelli, UPenn

Pedro Gozalo, , Brown Univ. School of Public Health & Providence VA Health Services Research Service

Bev Green, Kaiser Permanente Washington, Kaiser Permanente Washington Health Research Institute

Reesa Laws, Kaiser Permanente Center for Health Research

Rosemary Madigan, UPenn

Meghan Mayhew, Kaiser Permanente Center for Health Research

Vincent Mor, Brown Univ. School of Public Health & Providence VA Health Services Research Service

George "Holt" Oliver, Parkland Health and Hospital System (UT Southwestern)

Jon Puro, OCHIN

Jerry Sheehan, National Library of Medicine

Greg Simon, Kaiser Permanente Washington, Kaiser Permanente Washington Health Research Institute

Kari Stephens, U. Washington

Erik Van Eaton, U. Washington

Duke CC:

Rachel Richesson & W. Ed Hammond (Co-chairs)

Lesley Curtis, Monique Anderson Starks, Jesse Hickerson

Outline

- Background
- Lessons learned
- Desiderata for pragmatic informatics
- Examples from Demonstration Projects
- Future directions
- Discussion

Charter

- Share experiences using EHR to support research
- Identify generalizable approaches and best practices to promote the consistent use of practical methods to use 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

Varied Use of EHRs in Collaboratory PCTs

- Phenotypes for inclusion or exclusion (PPACT, ICD-Pieces)
- Ascertain completed procedure (STOP-CRC)
- Administer additional questionnaires/eligibility screening (TSOS, SPOT)
- LIRE trial uses EHR data to identify cohorts (dynamically as radiology reports are produced), insertions (based on rules in the EHR processing), and as primary source of outcome variables
- Identify study outcomes (SPOT)

Perspective

JAMIA
A SCHOLARLY JOURNAL OF INFORMATICS IN HEALTH AND BIOMEDICINE

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

Rachel L Richesson,[1,2] Beverly B Green,[3] Reesa Laws,[4] Jon Puro,[5] Michael G Kahn,[6] Alan Bauck,[4] Michelle Smerek,[7] Erik G Van Eaton,[8] Meredith Zozus,[9] W Ed Hammond,[2] Kari A Stephens,[10] and Greg E Simon[3]

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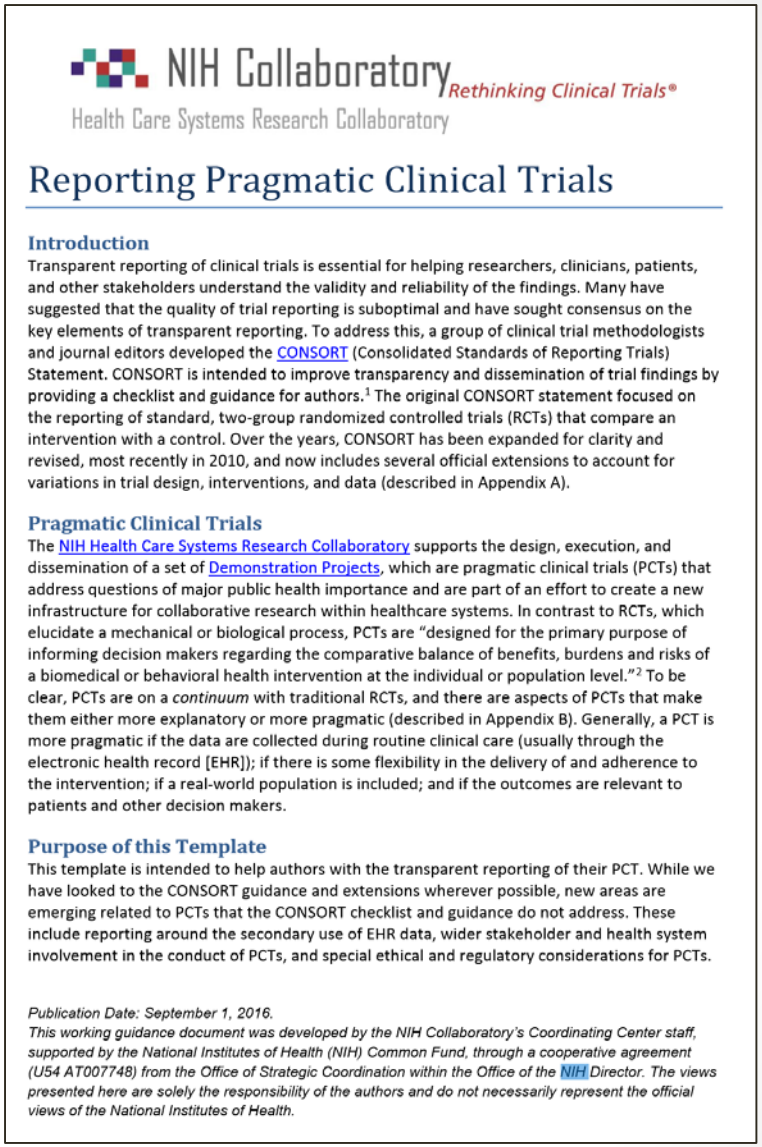
https://academic.oup.com/jamia/article/24/5/996/3069877/Pragmatic-trial-informatics-a-perspective-from-the

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



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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 assessments and methods (Use Collaboratory Recs)
- 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

Data Quality Assessment Recommendations

- Need adequate data and methods to detect the existing variation between populations at different sites or intervention groups
- Population-level data essential to measure and report data quality so results can be appropriately interpreted
- Recommend formal assessment of accuracy, completeness, and consistency for key data
- Data quality should be described, reported, and informed by workflows

https://www.nihcollaboratory.org/Products/Assessing-data-quality_V1%200.pdf

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Lessons Learned from Collaboratory PCTs

- Quality issues
- Difficult to access
- Dynamic

- Not all data needed for trial in EHR
- New data collection difficult

- Difficult to assimilate data across organizations
  - requires a reference standard
  - requires local data & systems experts

Greatest Lesson Learned…

- Researchers do not control the design or data collected in EHR systems….

- PCT researchers should:
  - not try to change what is collected or how it is recorded, but
  - identify how the collection or processing of clinical data can be improved to maximize the utility for research

Desiderata for PCT Researchers

- Ask questions and design trials that can use existing data and systems
- Understand the data generated in the course of healthcare delivery, and ask **how can these data be made more robust to support research and QI?**

Example – Research Design Responsive to Existing Data and Systems

Beverly Green, MD, MPH

Kaiser Permanente Washington Health Research Institute and Kaiser Permanente Washington

Co-PI, STOP CRC

"Strategies and Opportunities to Stop Colorectal Cancer in Priority Populations"

Tracking Colorectal Cancer (CRC) Screening, Follow-up, and Outcomes

- Colorectal cancer screening – is highly efficacious (US Preventive Services Task Force “A” recommendation)
- However screening rates are suboptimal, 62% nationally, and there are disparities
- Only 40% individuals who receive their care in federally qualified health centers (FQHCs) are current for CRC screening
- CRC screening data has the potential to transform population-based screening, follow-up, and outcomes, and decrease overuse
- CRC screening data can be used to efficiently implement evidence-based effective interventions (mailed fecal tests and reminds) and track follow-up testing
- In general the data needed is simple, test date and outcomes. However in practice capturing CRC screening data is not

Colorectal Cancer Screening
Fecal Tests

- STOP CRC –is a pragmatic cluster randomized trial being conducted within the OCHIN primary care research network. OCHIN is a non-profit health information technology organization provides a single EHR to over 400 Federally Qualified Health Centers (FQHCs) with over 3 million patients in 15 states (and is also a PCOR-Net site).
- Capture of fecal testing (FIT), a high sensitivity test used to find microscopic blood is relatively straightforward. There is a test diagnosis, test type, date, and result
- Standardized laboratory codes are available and are used by commercial labs (LOINC - Logical Observation Identifiers Names and Codes) and CPT codes
- Organizations don't always use LOINC coding and labs performed in clinic can be difficult to identify (back office orders).
- OCHIN requires FQHCs to use electronic lab feeds
- OCHIN monitors laboratory data and looks for existing and new codes

Colorectal Cancer Screening
Colonoscopy

- In the US most people current for CRC screening, have completed colonoscopy (even though many prefer fecal testing, and offering it increases screening rates)
- Colonoscopy can be accurately identified using billing/claims codes
- However colonoscopy procedures are not done in FQHCs – reports are received as paper copies and scanned into the EHR – generally not in discoverable fields
- Work arounds exist – search engines, EHR “health maintenance” fields where procedures type, dates, and interval for the next test can be hand entered (and is used variably)
- Even in integrated health care organizations, that collects claims, colonoscopy data can be incomplete (historical/network data/results)
- Clinical (procedure and pathology) results are not captured in discernable fields

Is Colorectal Cancer Screening Data Accurate in the Community Setting?*

- We performed a validation study to determine the accuracy of EHR data in capturing CRC screening within the 26 clinics participating in STOP CRC
- Random sample of 800 age eligible patients stratified by screening status
- Of the 520 patients identified by EHR in need of screening, 459 were confirmed by chart audit – positive predictive value (PPV) was 88%. Most of the disagreement (84%) was due to undetected colonoscopy.
- This influenced STOP CRC's primary outcome, we chose completion of fecal testing and completion of any type of CRC is a secondary outcome (this is in contrast to our studies within Kaiser, where we are able to use both outcomes)

*Petrik AF, Green BB, Vollmer WM, Coronado GD et al. The validation of electronic health records in accurately identifying patients eligible for CRC in safety net clinics. Fam Practice 2016

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Future State – What is Possible?

- National CRC screening programs exist in other countries capture CRC screening testing, follow-up, and outcomes on their entire population.
- Advantages – improvement of tracking of under and over use of CRC screening, ability to monitor and improve outcomes
- State-based - All Payer Claims Databases are increasingly becoming available and could potentially be used to track procedure events (not clinical results)
- Software exists for capturing colonoscopy procedure data (is sometimes used by gastroenterology practices, but is not integrated into primary care or health systems EHRs)
- Pathology data also could be entered in discrete fields (similar to other labs and pharmacy data)
- Organizational motivation?
  - HEDIS CRC screening is a 5 start metric. Hybrid measures (audits) are generally used
  - Tracking positive FIT follow-up and high-risk surveillance (including family history) is at a very early stage or not done in most organizations. Genomics will also have a role in the future.
- What would it take for the US/healthcare systems to have similar to other programs (example: immunizations)?

NIH Workshop on Sustaining Practice Change

- "Pragmatic Clinical Trials - Unique Opportunities For Disseminating, Implementing & Sustaining Evidence-based Practices Into Clinical Care" ; May 24, 2017
  - Research competes with enterprise for IT / data support
  - Need to capture data related to intervention and control
  - Need to create Win-Win scenarios between the research and the organization

Video archive:
https://videocast.nih.gov/Summary.asp?Live=21968&bhcp=1

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- Create infrastructure that support both research and QI
- Create organizational culture and commitment to research as a core mission

Example - Common Resources for Research and QI

- LIRE inspired suggestions to improve institutional resources that would promote data-driven research:
  - LIRE is calculating RVUs from standardly collected coded data from the EHR as a key outcome to determine if the intervention reduced spine related RVU-based services
    - No pipeline within organizations exists to pass on these kinds of calculations = lost opportunity for a pathway for research driven algorithms to improve organizational quality analytics
  - Organizations do not systematically sustain research staff analysts, leading to frequent turnover with data extraction staff and inefficiencies w/ the loss of research project specific knowledge in longitudinal studies
    - Dedicated research analysts within health care organizations would create stability and efficiency for research studies, reducing burden on research teams

Enabling Features of PCTs

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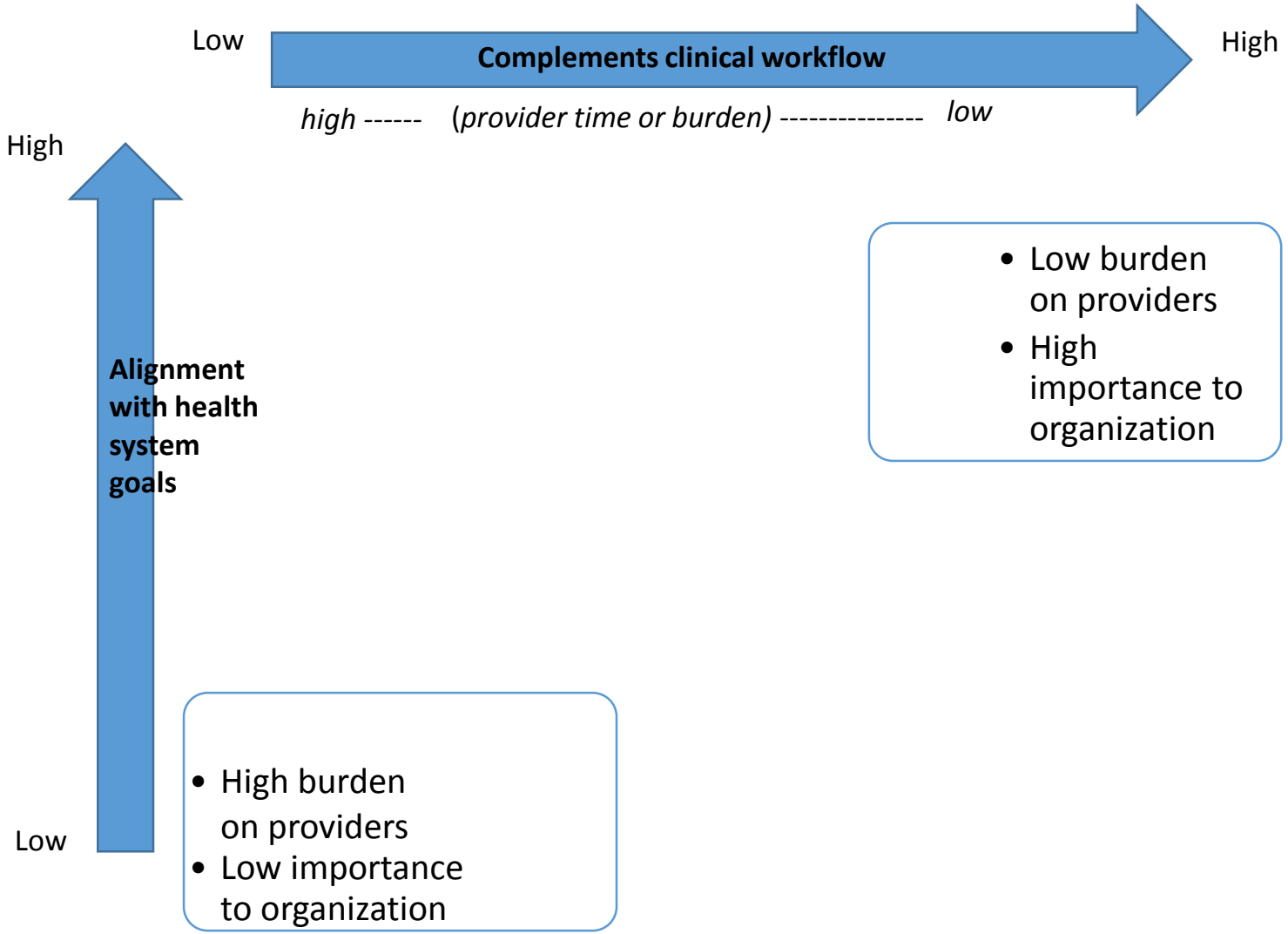

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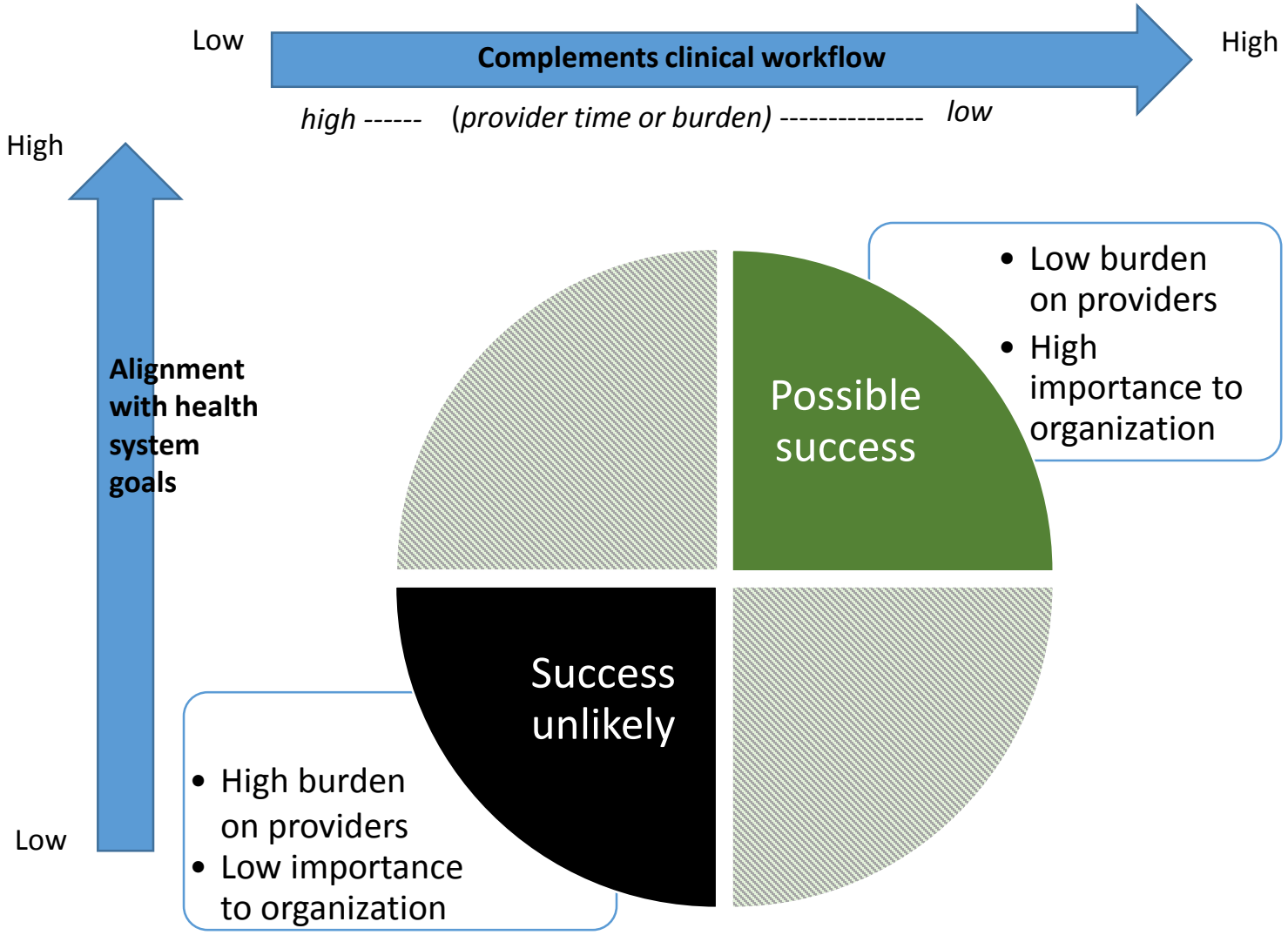
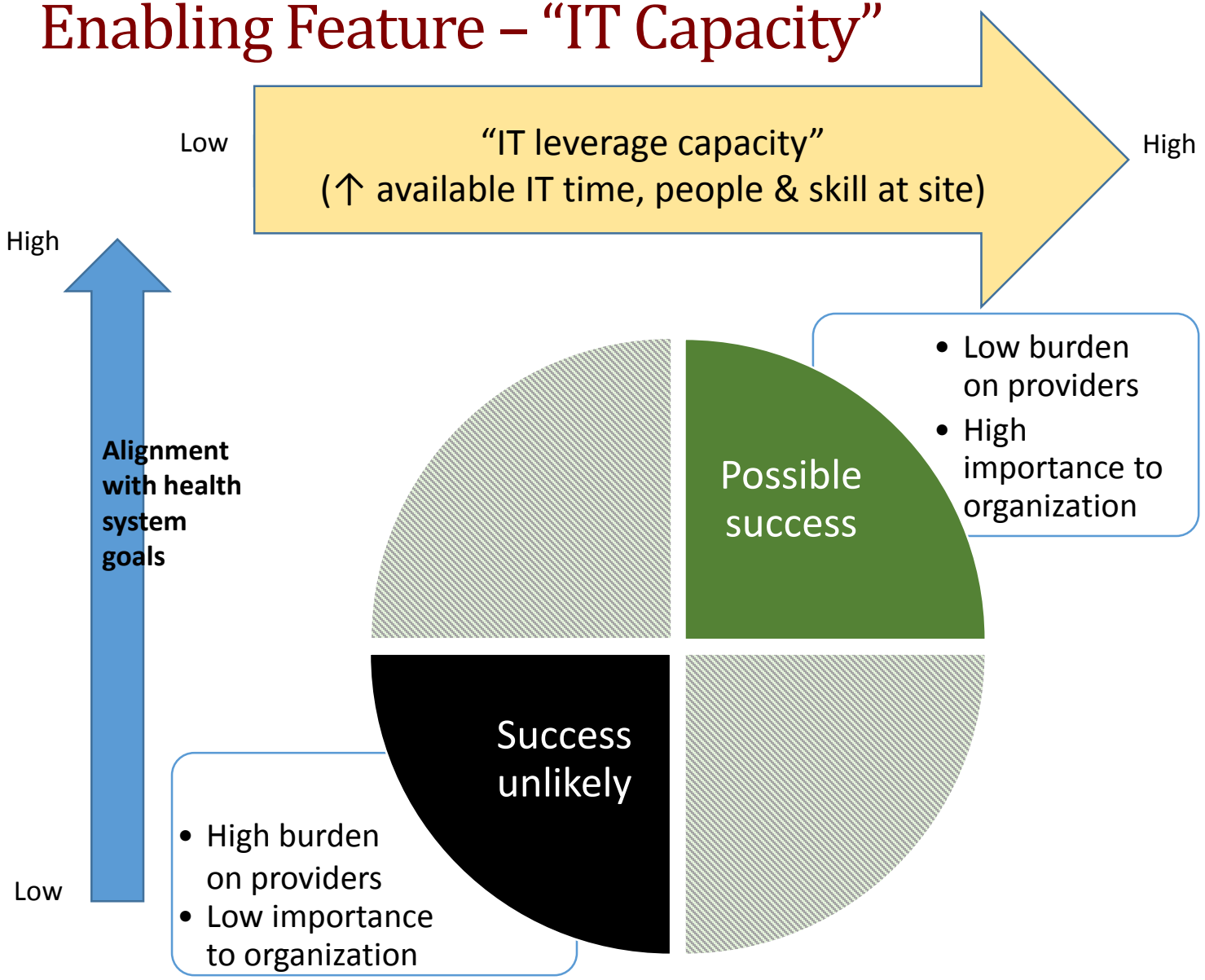


Enabling Feature – "IT Capacity"



Other success factors for PCTs

- Dedicated and or research-trained data operations staff
- Existence of resources that can support multiple research and QI studies
- 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.

Final Thoughts

- Good practices for using clinical data in PCTs are based upon scientific principles
- Data, data standards and tools can support research and clinical goals
- Creating LHS environments that can support rapid generation of PCTs requires research / health system / operations collaboration
  - … and shared resources
  - … might be able to quantify or measure
- Deliberate strategy to advance needs of multiple stakeholders

Future directions & areas of influence

- Future Collaboratory PCTs
- Health care organizations / research ecosystem
- Documentation of data collection, management, and quality assessment
- Integration of data standards & research functions into commercial EHR systems can enhance organizational and national capacity for PCTs

Acknowledgements

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All products from the core are posted on the NIH Collaboratory website.

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LIVING TEXTBOOK
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