Health Care Systems Research Collaboratory

A Virtual Home for Knowledge about Pragmatic Clinical Trials using Health Systems: nihcollaboratory.org
Perspective

• Approaching the one year mark of the Collaboratory
• First batch of projects finishing planning phase
• Cores/Working Groups in motion
• Second batch of projects will be in play over the course of this year
• Beyond that point, our job is:
  • Execute the 2 batches of projects
  • Determine what we are learning
  • Disseminate the knowledge
What Should the Collaboratory Produce for its Deliverables?
Origins of the Common Fund

2004: NIH Roadmap is launched

December 9, 2006: Congress unanimously passes a reauthorization bill affirming importance of NIH and its vital role in advancing biomedical research to improve the health of the Nation

Establishes the Division of Program Coordination, Planning, and Strategic Initiatives (DPCPSI) within Office of the Director and the NIH Common Fund to provide a dedicated source of funding to enable trans-NIH research
Criteria for Common Fund Programs

- **Transformative**: Must have high potential to dramatically affect biomedical and/or behavioral research over the next decade.
- **Catalytic**: Must achieve a defined set of high impact goals within 5-10 years.
- **Synergistic**: Outcomes must synergistically promote and advance individual missions of NIH Institutes and Centers to benefit health.
- **Cross-cutting**: Program areas must cut across missions of multiple NIH Institutes and Centers, be relevant to multiple diseases or conditions, and be sufficiently complex to require a coordinated, trans-NIH approach.
- **Unique**: Must be something no other entity is likely or able to do.
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Interest in PCTs

- The public is somewhat surprised when informed about how little we know about routine decisions in clinical care and organization of care delivery
- Providers resonate to it
- FDA and EMA are hungry for reliable answers on simple questions
- Healthcare administrators warm up after exchange of information
- The NIH response has been amazing
- Linkage to “Big Data to Knowledge”
- Feeling of a “tipping point”
Preliminary Insights from Recent Clinical Trial Polling Data

Mary Woolley, Research!America

June 10, 2013
“...public sentiment is everything. With public sentiment, nothing can fail; without it nothing can succeed.”

President Abraham Lincoln
Less Than Half Believe Health Care Services Based on Recent Research

Do you believe that the health care services you receive are based on the best and most recent research available?

Sources: National public opinion polls, 2005-2013
Seven in 10 Say Doctors Don’t Talk About Medical Research

Has your doctor or other health care professional ever talked to you about medical research?

- Yes: 22%
- No: 70%
- Not sure: 8%

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
Americans are Interested in Clinical Trials

Do you agree or disagree with each of the following statements?

1. I am interested in finding out more about taking part in clinical trials
   - Strongly agree: 29
   - Somewhat agree: 32
   - Somewhat disagree: 17
   - Strongly disagree: 11
   - Not sure: 12

2. I would take part in a clinical trial if I was asked by someone I trust
   - Strongly agree: 28
   - Somewhat agree: 36
   - Somewhat disagree: 13
   - Strongly disagree: 7
   - Not sure: 17

3. Taking part in a clinical trial is as valuable to our health care system as giving blood
   - Strongly agree: 21
   - Somewhat agree: 46
   - Somewhat disagree: 16
   - Strongly disagree: 7
   - Not sure: 11

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
Patients Willing to Share Health Information

Please indicate your willingness to share your personal health information, assuming that appropriate privacy protections are in place.

- So researchers can better understand diseases and develop new ways to prevent, treat and cure them: 74 Yes, 12 No, 14 Not sure.
- To advance medical research: 73 Yes, 11 No, 16 Not sure.
- So health care providers can improve patient care: 72 Yes, 14 No, 14 Not sure.
- So public health officials can better track disease and disability and the causes: 67 Yes, 17 No, 17 Not sure.

Source: A Research!America poll of U.S. adults conducted in partnership with Zogby Analytics in May 2013.
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Distributed Analysis Example: Mini-Sentinel

1. User creates and submits query (a computer program)

2. Data partners retrieve query

3. Data partners review and run query against their local data

4. Data partners review results

5. Data partners return results via secure network

6. Results are aggregated
Mini-Sentinel’s Common Data Model:

**Enrollment**
- Person ID
- Enrollment start & end dates
- Drug coverage
  - Medical coverage

**Demographic**
- Person ID
- Birth date
- Sex
- Race
- Etc.

**Dispensing**
- Person ID
- Dispensing date
- National drug code (NDC)
- Days supply
- Amount dispensed

**Encounter**
- Person ID
- Dates of service
- Provider seen
- Type of encounter
- Facility
- Etc.

**Lab Result**
- Person ID
- Dates of order, collection & result
- Test type, immediacy & location
- Procedure code & type
- Test result & unit
- Abnormal result indicator
- Etc.

**Vital Signs**
- Person ID
- Date & time of measurement
- Height
- Weight
- Diastolic & systolic BP
- Tobacco use & type
- BP type & position

**Death**
- Person ID
- Date of death
- Source
- Confidence
- Etc.

**Cause of Death**
- Person ID
- Cause of death
- Diagnosis code & code type
- Source
- Confidence
- Etc.

**Diagnosis**
- Person ID
- Date
- Principle diagnosis flag
- Encounter type & provider
- Diagnosis code & type
- Etc.

**Procedure**
- Person ID
- Dates of service
- Procedure code & type
- Encounter type & provider
- Etc.

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NIH Distributed Research Network Coordinating Center

- Network Management
- Query Support
- Research Support
- Query Tool Development
- Knowledge Database
- Software Development
- Project Management
- Data Models & Standards
- Consultation
- Health System Expertise

NIH DRN Secure Portal

Knowledge Management System
Cross project lessons learned, query tracking, meta-data capture, search functions, etc

PROJECTS
- LIRE

Query Tools
- Modular Programs
- Summary Tables
- Query Interface
- SAS, SQL, menu-driven
- Analytic Tools
- Reporting Tools

Administration
- Security / Access Control
- File / Query Repository
- User Administration
- Workflow Management

Mayo Clinic
Group Health Research Institute
Health Partners
KP Northwest
University of Washington

Data Source: HMORN Virtual Data Warehouse + selected clinic data
Query Health: Distributed Population Queries

“Send questions to the data!”
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THE ESSENTIAL ISSUE

The “Tower of Babel” of data from databases, EHRs, literature, and clinical trials: without controlled vocabulary and data standards we are somewhat lost!
Core Components and Deliverables

- Phenotype validation
- Consulting
- Synthesis of practice
- Environmental scanning

- Iterative Data Quality Assessment Survey and Report

- Lit. Search Guidelines
- Phenotype Definition Template
- Evaluation criteria
- Phenotype Resource Library

- Table 1 Project
- ID existing standards
- Nomination for standardization
- Definition and metadata

Demonstration Project Support

Standard Data Elements

Data Quality

Phenotype Definitions

The Collaboratory
Why These Are in One Core

**Quality** = Measuring “fitness for use” of data
Used to measure aspects of data, e.g., accuracy, completeness, currency that impact it’s fitness for use.

**Phenotype** = Data Elements + Logic
Used to describe data necessary to answer a clinical question.

**Data Element** = atomic unit of data exchange and use
Used to collect, store, and exchange data.

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Data Quality Assessment

Phenotypes

Data Elements
Evidence of How Work to be Done

- Many EHRs cannot accurately characterize race and ethnicity
- Depending on the definition used rates of diabetes in an EHR based population can vary by almost two-fold
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Fundamental Informatics Infrastructure--Matrix Organizational Structure

Disease Registries—Granular, Detailed

Electronic Health Records
Adaptable to all!

PRO’s as an integrating factor

Primary Care
Mental Health
Cancer
Cardiovascular
Etc...

NIH Collaboratory
Health Care Systems Research Collaboratory

Rethinking Clinical Trials
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In a learning health care system, research influences practice and practice influences research.

**EVALUATE**
Collect data and analyze results to show what works and what doesn’t.

**IMPLEMENT**
Apply plan in pilot and control settings.

**DESIGN**
Design care and evaluation based on evidence generated here and elsewhere.

**ADJUST**
Use evidence to influence continual improvement.

**DISSEMINATE**
Share results to improve care for everyone.

**INTERNAL AND EXTERNAL SCAN**
Identify problems and potentially innovative solutions.
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Collaboratory Coordinating Center and Stakeholder Organizations
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Ethics of Learning Health—the Opportunity for this Year

- IOM Learning Health Systems
- SUPPORT Trial Controversy—HHS hearings
- Major PCORI investment
- Multiple investments by other countries
- Continued pressure on NIH budget
- Continued pressure on industry R&D budgets

- The fundamental issue is how to balance our newfound understanding of ignorance and how to fix it through “big data” and the rights and preference of people to understand and make autonomous choices
- This will require wisdom and empirical research
7 Proposed Moral Obligations in a Learning Health Care System

- Respect the rights and dignity of patients
- Respect clinician judgments
- Provide optimal clinical care to each patient
- Avoid imposing nonclinical risks and burdens on patients
- Address health inequalities
- Conduct continuous learning activities that improve the quality of clinical care and health care systems
- Contribute to the common purpose of improving the quality and value of clinical care and health care systems
- Introduction and 7 commentaries
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Why Engage Stakeholders?

- Wide range of barriers to metamorphosis from health care delivery system to research partner
  - Technical, operational, regulatory, financial, cultural
- Health systems and research community don’t have all necessary expertise, authority, resources, insights
  - Optimal “implementation methods and best practices” may require actions by other agents
- Stakeholder Engagement Core provides forum to engage broader healthcare community
Thoughts on the Path Forward

• There is a lot of public education needed to build greater support for the necessity of more efficient learning
  • Uncertainty and risks in clinical care
  • Potential harms of not learning
  • Risk of overprotection/under-protection with current approach
• Understanding clinical trials and randomization is really complicated.
  • Need the best materials possibly to explain these concepts using multiple media.
• Progress is possible within current regulatory environment
  • Use cases; adopting best current practices across IRBs
• Modified consent: Greater disclosure/explanation may be viable alternative to standard consent for selected experimental studies
• Acceptance of alternative methods of consent may vary based on the extent to which the provider/system upholds commitments to patients that “learning” will actually translate into improved care.
Stakeholder Feedback on the Overall Goals of the Collaboratory and Learning Healthcare Systems

- The notion that a learning will lead to better patient care in not in itself sufficient justification for major reductions in research oversight or regulation.
- Sense of group: in the rush to learn more quickly, we must also remain respectful of rights to be fully informed, and protected from potential harms.
- SAG feedback provided good reality check on degree to which reduced oversight would be acceptable.
Feedback on Collaboratory Goals and LHS

- Need to further raise public awareness that healthcare systems are not currently learning systems
- Note that even those who recognize gaps in knowledge often don’t think this is true of their own providers
- Stakeholders support of learning activities depends heavily on being convinced of commitment to use evidence to change
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Collaboratory Deliverables

- Demonstration projects
  - Practical clinical trials this year
  - Future FOA’s could cover other parts of research spectrum per NIH decisions
- Knowledge repository (nihcollaboratory.org)
  - Portal
  - Living text
  - Products of cores/working groups
- Distributed research network
# Preliminary Deliverables

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<th>Key Guidance’s &amp; Best Practices for Year 1</th>
<th>Working Group</th>
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<td>Criteria for assessing utility of phenotypes from EHRs in PCTs</td>
<td>Phenotype</td>
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<td>Table 1 Project (see below)</td>
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<td>Assessing the power of cluster randomized trials (CRTs)</td>
<td>Design/Statistics</td>
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<td>Well-catalogued issues and challenges across Demonstration Projects</td>
<td>Health Systems Interactions</td>
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<td>Strategies to maximize engagement with health care systems</td>
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<td>Strategies to minimize disruption when implementing PCTs and CRTs</td>
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<td>Updated &amp; generalized HMORN Toolkit resources for Knowledge Repository</td>
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<td>Overcoming barriers to conducting PCTs within health care delivery systems: Perspectives of the Stakeholder Advisory Group to the HSR Collaboratory</td>
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</table>
- Leverages existing networks’ data and analysis tools
  - Can use many data types, e.g., EHR, claims, registries
  - Can use many data models, e.g., Mini-Sentinel, i2b2, OMOP
  - Can use existing querying tools, e.g., Mini-Sentinel modular programs
- Every use requires the agreement of the data steward
“To win the future, America needs to out-educate, out-innovate, and out-build the rest of the world.”

— President Barack Obama, Weekly Address February 5, 2011