

PCOR Methodology

Sharon-Lise T. Normand

Department of Health Care Policy, Harvard Medical School
Department of Biostatistics, Harvard Chan School of Public Health

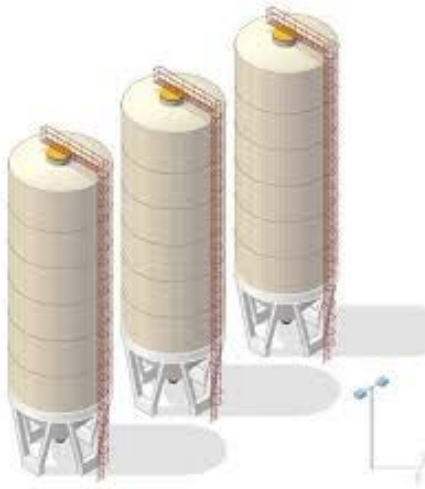
24/05/2021

PCOR Data Capacity Workshop



Data Silos

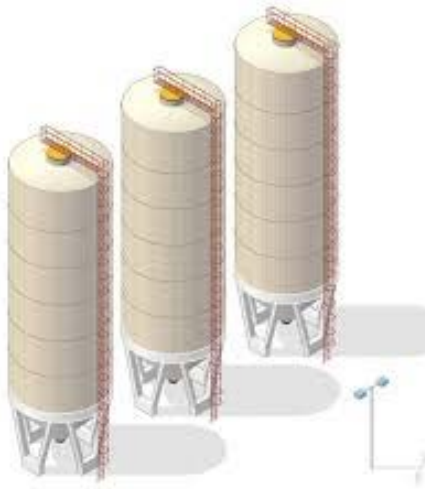
depositphotos.com



depositphotos.com

Data Silos

- ▶ Too many silos
- ▶ Unique IDs questionable
- ▶ Too many rules preventing linkages

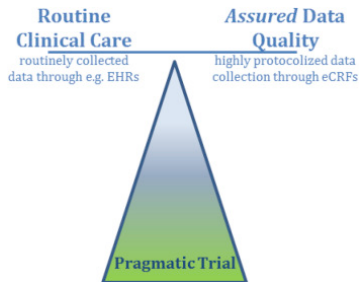


Data Silos

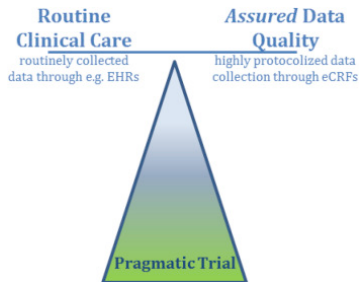
- ▶ Too many silos
- ▶ Unique IDs questionable
- ▶ Too many rules preventing linkages

depositphotos.com

Learning what works requires **longitudinal** observation

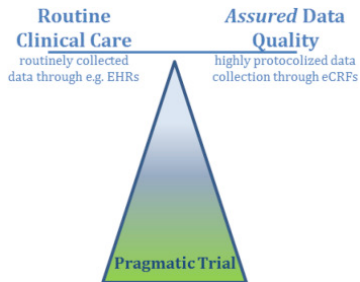


Clinical Trials



Clinical Trials

- ▶ Pragmatic Trials
 - ▶ Often lack of blinding
 - ▶ Usual care arms
 - ▶ Small effect size, heterogeneous populations
- ▶ Underuse of hybrid designs



Clinical Trials

- ▶ Pragmatic Trials
 - ▶ Often lack of blinding
 - ▶ Usual care arms
 - ▶ Small effect size, heterogeneous populations
- ▶ Underuse of hybrid designs

Learning what works requires **valid** statistical approaches



Missing Data



Missing Data

- ▶ When are data missing (e.g., EHR)?
- ▶ Irregularly spaced data
- ▶ Combining more datasets increases likelihood of missingness



Missing Data

- ▶ When are data missing (e.g., EHR)?
- ▶ Irregularly spaced data
- ▶ Combining more datasets increases likelihood of missingness

Learning what works requires **addressing** missing data



Uncertainty



Uncertainty

- ▶ Selective inference
- ▶ Heterogeneous data sources
- ▶ Propagation of error more complicated



Uncertainty

- ▶ Selective inference
- ▶ Heterogeneous data sources
- ▶ Propagation of error more complicated

Learning what works requires an **honest reflection** of uncertainty and **transparency** of decisions

Opportunities

- ▶ Clinical trials
 - ▶ Streamline approaches for adaptive trials
 - ▶ Develop parallel randomized & prospective observational studies
 - ▶ Develop adjustments for non-blinding in outcomes reporting

Opportunities

- ▶ Clinical trials
 - ▶ Streamline approaches for adaptive trials
 - ▶ Develop parallel randomized & prospective observational studies
 - ▶ Develop adjustments for non-blinding in outcomes reporting
- ▶ Causal inference & experimental thinking
 - ▶ Develop/encourage use in pragmatic trials
 - ▶ Study implication of missing data in sparse data settings
 - ▶ Understand & propagate error

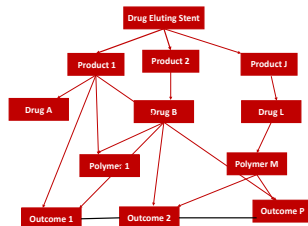
Opportunities

- ▶ Clinical trials
 - ▶ Streamline approaches for adaptive trials
 - ▶ Develop parallel randomized & prospective observational studies
 - ▶ Develop adjustments for non-blinding in outcomes reporting
- ▶ Causal inference & experimental thinking
 - ▶ Develop/encourage use in pragmatic trials
 - ▶ Study implication of missing data in sparse data settings
 - ▶ Understand & propagate error
- ▶ Borrowing information
 - ▶ Exploit connectedness of information

Opportunities

- ▶ Clinical trials
 - ▶ Streamline approaches for adaptive trials
 - ▶ Develop parallel randomized & prospective observational studies
 - ▶ Develop adjustments for non-blinding in outcomes reporting
- ▶ Causal inference & experimental thinking
 - ▶ Develop/encourage use in pragmatic trials
 - ▶ Study implication of missing data in sparse data settings
 - ▶ Understand & propagate error
- ▶ Borrowing information
 - ▶ Exploit connectedness of information

Longitudinal multi-task approaches



ASPE's Role



**Notice of Intent to Publish
Funding Opportunity for the
NIH Common Fund's Bridge2AI program**

Integration, Dissemination, and Evaluation (BRIDGE) Center (NOT-RM-21-021)

- 01**
Generating new flagship biomedical and behavioral data sets that are ethically sourced, trustworthy, well-defined, and accessible
- 02**
Developing software and standards to unify data attributes across multiple data sources and across data types
- 03**
Creating automated tools to accelerate the creation of FAIR (Findable, Accessible, Interoperable, and Reusable) and ethically sourced data sets
- 04**
Providing resources to disseminate data, ethical principles, tools, and best practices
- 05**
Creating training materials and activities for workforce development that bridges the AI, biomedical, and behavioral research communities

ASPE's Role



**Notice of Intent to Publish
Funding Opportunity for the
NIH Common Fund's Bridge2AI program**

Integration, Dissemination, and Evaluation (BRIDGE) Center (NOT-RM-21-021)

- 01**
Generating new flagship biomedical and behavioral data sets that are ethically sourced, trustworthy, well-defined, and accessible
- 02**
Developing software and standards to unify data attributes across multiple data sources and across data types
- 03**
Creating automated tools to accelerate the creation of FAIR (Findable, Accessible, Interoperable, and Reusable) and ethically sourced data sets
- 04**
Providing resources to disseminate data, ethical principles, tools, and best practices
- 05**
Creating training materials and activities for workforce development that bridges the AI, biomedical, and behavioral research communities

- ▶ Data on demand
- ▶ Invest in trial infrastructure
- ▶ Invest in statistical methodology