Program Policies and Guidance Documents

Project Onboarding Meeting
November 1, 2023

Rich Platt, MD, MSc
Gina Uhlenbrauck

NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

Data Quality Guidance and Data Sharing Policy

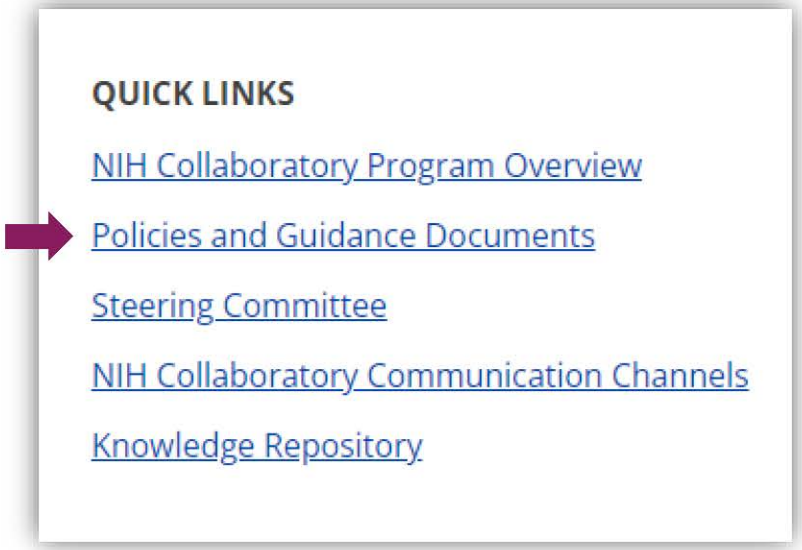
Rich Platt, MD, MSc

NIH PRAGMATIC TRIALS COLLABORATORY

Rethinking Clinical Trials®

NIH Collaboratory Governance Policies & Guidance Documents

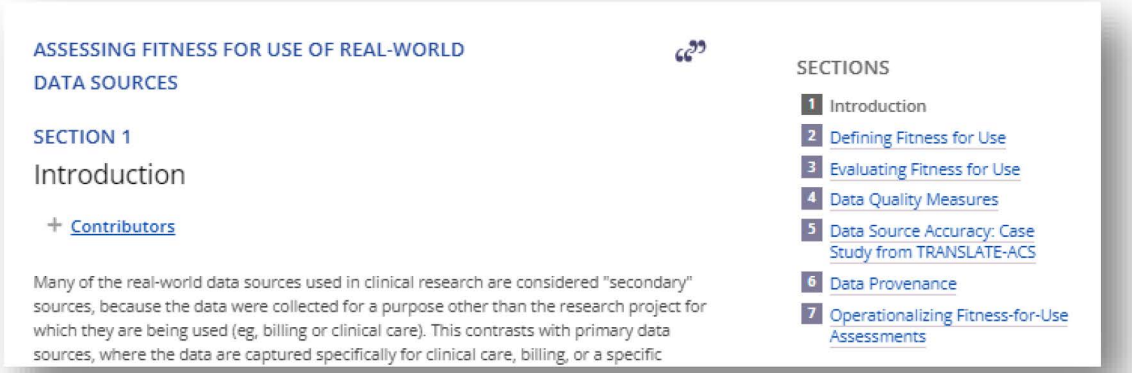
- Available on website and in meeting e-Binder
  - https://rethinkingclinicaltrials.org/nih-collaboratory-policies-and-guidance-documents/



NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

Data Quality Guidance

- **Assessing Fitness for Use of Real-Word Data**
  - Describes approaches to determine whether real-world data are fit for their intended use prior to their use in research settings
- **Quick Reference Handout**
  - Describes data quality checks and recommendations for assessments


ASSESSING FITNESS FOR USE OF REAL-WORLD DATA SOURCES

SECTION 1

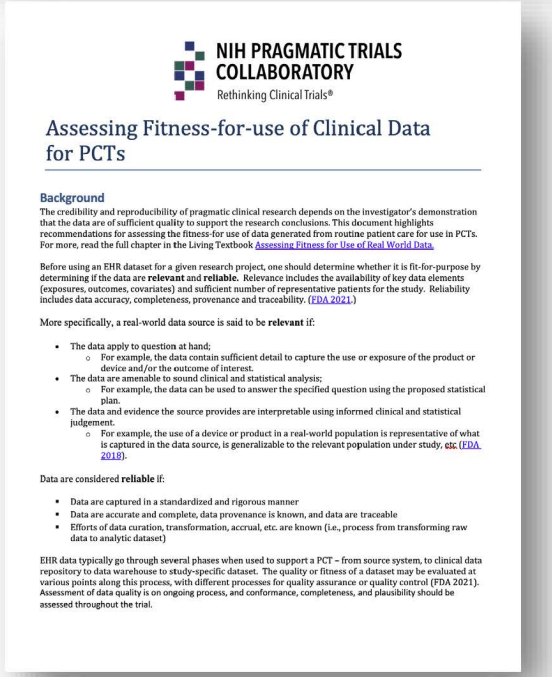
Introduction

+ Contributors

Many of the real-world data sources used in clinical research are considered "secondary" sources, because the data were collected for a purpose other than the research project for which they are being used (eg, billing or clinical care). This contrasts with primary data sources, where the data are captured specifically for clinical care, billing, or a specific

SECTIONS

1 Introduction
2 Defining Fitness for Use
3 Evaluating Fitness for Use
4 Data Quality Measures
5 Data Source Accuracy: Case Study from TRANSLATE-ACS
6 Data Provenance
7 Operationalizing Fitness-for-Use Assessments


NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

Assessing Fitness-for-use of Clinical Data for PCTs

Background

The credibility and reproducibility of pragmatic clinical research depends on the investigator's demonstration that the data are of sufficient quality to support the research conclusions. This document highlights recommendations for assessing the fitness-for use of data generated from routine patient care for use in PCTs. For more, read the full chapter in the Living Textbook Assessing Fitness for Use of Real World Data.

Before using an EHR dataset for a given research project, one should determine whether it is fit-for-purpose by determining if the data are **relevant** and **reliable**. Relevance includes the availability of key data elements (exposures, outcomes, covariates) and sufficient number of representative patients for the study. Reliability includes data accuracy, completeness, provenance and traceability. (FDA 2021.)

More specifically, a real-world data source is said to be **relevant** if:

- The data apply to question at hand;
  - For example, the data contain sufficient detail to capture the use or exposure of the product or device and/or the outcome of interest.
- The data are amenable to sound clinical and statistical analysis;
  - For example, the data can be used to answer the specified question using the proposed statistical plan.
- The data and evidence the source provides are interpretable using informed clinical and statistical judgement.
  - For example, the use of a device or product in a real-world population is representative of what is captured in the data source, is generalizable to the relevant population under study, etc. (FDA 2018).

Data are considered **reliable** if:

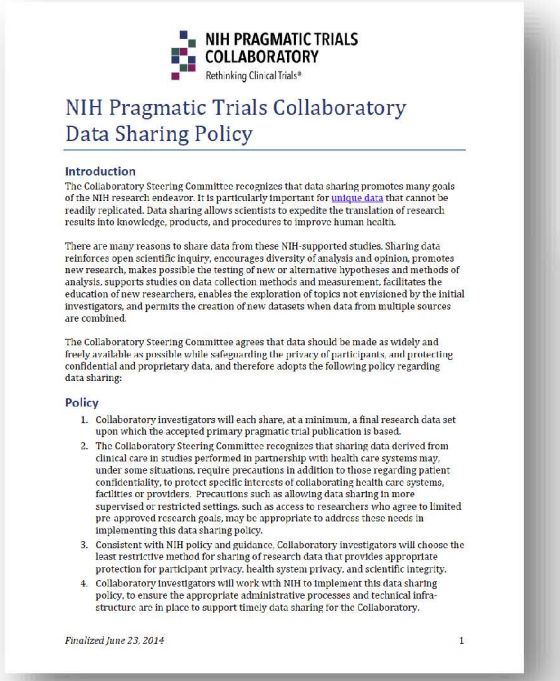
- Data are captured in a standardized and rigorous manner
- Data are accurate and complete, data provenance is known, and data are traceable
- Efforts of data curation, transformation, accrual, etc. are known (i.e., process from transforming raw data to analytic dataset)

EHR data typically go through several phases when used to support a PCT – from source system, to clinical data repository to data warehouse to study-specific dataset. The quality or fitness of a dataset may be evaluated at various points along this process, with different processes for quality assurance or quality control (FDA 2021). Assessment of data quality is on ongoing process, and conformance, completeness, and plausibility should be assessed throughout the trial.

NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

Data Sharing Policy and Considerations

- **Data Sharing Policy**
  - States the 4 policies adopted by the NIH Collaboratory for data sharing
- **Data Sharing Considerations**
  - Describes considerations for use of healthcare system data, methods and tools for data sharing, and expectations for NIH Collaboratory trials



NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

NIH Pragmatic Trials Collaboratory
Data Sharing Policy

Introduction

The Collaboratory Steering Committee recognizes that data sharing promotes many goals of the NIH research endeavor. It is particularly important for unique data that cannot be readily replicated. Data sharing allows scientists to expedite the translation of research results into knowledge, products, and procedures to improve human health.

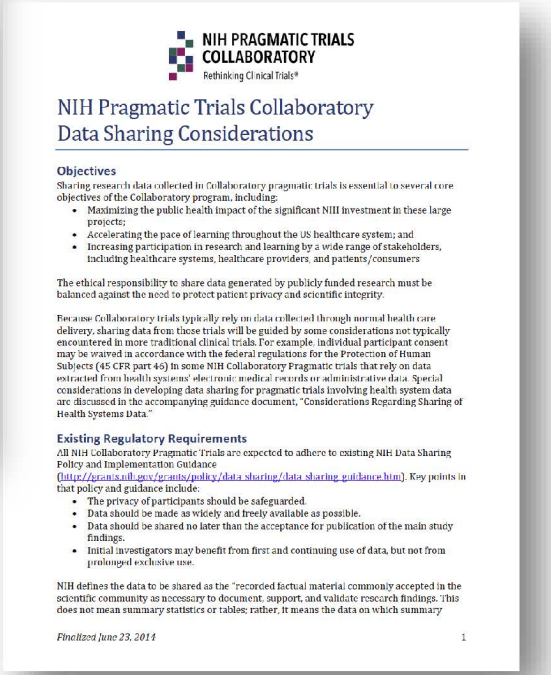
There are many reasons to share data from these NIH-supported studies. Sharing data reinforces open scientific inquiry, encourages diversity of analysis and opinion, promotes new research, makes possible the testing of new or alternative hypotheses and methods of analysis, supports studies on data collection methods and measurement, facilitates the education of new researchers, enables the exploration of topics not envisioned by the initial investigators, and permits the creation of new datasets when data from multiple sources are combined.

The Collaboratory Steering Committee agrees that data should be made as widely and freely available as possible while safeguarding the privacy of participants, and protecting confidential and proprietary data, and therefore adopts the following policy regarding data sharing:

Policy

1. Collaboratory investigators will each share, at a minimum, a final research data set upon which the accepted primary pragmatic trial publication is based.
2. The Collaboratory Steering Committee recognizes that sharing data derived from clinical care in studies performed in partnership with health care systems may, under some situations, require precautions in addition to those regarding patient confidentiality, to protect specific interests of collaborating health care systems, facilities or providers. Precautions such as allowing data sharing in more supervised or restricted settings, such as access to researchers who agree to limited pre approved research goals, may be appropriate to address these needs in implementing this data sharing policy.
3. Consistent with NIH policy and guidance, Collaboratory investigators will choose the least restrictive method for sharing of research data that provides appropriate protection for participant privacy, health system privacy, and scientific integrity.
4. Collaboratory investigators will work with NIH to implement this data sharing policy, to ensure the appropriate administrative processes and technical infrastructure are in place to support timely data sharing for the Collaboratory.

Finalized June 23, 2014 1



NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

NIH Pragmatic Trials Collaboratory
Data Sharing Considerations

Objectives

Sharing research data collected in Collaboratory pragmatic trials is essential to several core objectives of the Collaboratory program, including:

- Maximizing the public health impact of the significant NIH investment in these large projects;
- Accelerating the pace of learning throughout the US healthcare system; and
- Increasing participation in research and learning by a wide range of stakeholders, including healthcare systems, healthcare providers, and patients/consumers

The ethical responsibility to share data generated by publicly funded research must be balanced against the need to protect patient privacy and scientific integrity.

Because Collaboratory trials typically rely on data collected through normal health care delivery, sharing data from these trials will be guided by some considerations not typically encountered in more traditional clinical trials. For example, individual participant consent may be waived in accordance with the federal regulations for the Protection of Human Subjects (45 CFR part 46) in some NIH Collaboratory Pragmatic trials that rely on data extracted from health systems' electronic medical records or administrative data. Special considerations in developing data sharing for pragmatic trials involving health system data are discussed in the accompanying guidance document, "Considerations Regarding Sharing of Health Systems Data."

Existing Regulatory Requirements

All NIH Collaboratory Pragmatic Trials are expected to adhere to existing NIH Data Sharing Policy and Implementation Guidance (http://grants.nih.gov/grants/policy/data_sharing/data_sharing_guidance.htm). Key points in that policy and guidance include:

- The privacy of participants should be safeguarded.
- Data should be made as widely and freely available as possible.
- Data should be shared no later than the acceptance for publication of the main study findings.
- Initial investigators may benefit from first and continuing use of data, but not from prolonged exclusive use.

NIH defines the data to be shared as the "recorded factual material commonly accepted in the scientific community as necessary to document, support, and validate research findings. This does not mean summary statistics or tables; rather, it means the data on which summary

Finalized June 23, 2014 1

NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

Reasons to share:

- Advancing public health
- Maximizing investment
- Accelerating learning
- Fostering collaboration (pay it forward)

*Ultimately… transparency, reproducibility, and secondary use of medical research are **good for society**.*

NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

Annals of Internal Medicine

IDEAS AND OPINIONS

Data Sharing and Embedded Research

Gregory E. Simon, MD, MPH; Gloria Coronado, PhD; Lynn L. DeBar, PhD, MPH; Laura M. Dember, MD; Beverly B. Green, MD, MPH; Susan S. Huang, MD, MPH; Jeffrey G. Jarvik, MD, MPH; Vincent Mor, PhD; Joakim Ramsberg, PhD; Edward J. Septimus, MD; Karen L. Staman, MS; Miguel A. Vazquez, MD; William M. Vollmer, PhD; Douglas Zatzick, MD; Adrian F. Hernandez, MD, MHS; and Richard Platt MD, MS

- The ethical responsibility to share data generated by publicly funded research must be balanced against the need to protect patient privacy and scientific integrity
- Data sharing policies *must not dissuade healthcare system participation*

NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

Annals of Internal Medicine®

Ideas and Opinions | March 2023

Moving From Idealism to Realism With Data Sharing

Keith A. Marsolo, PhD, Kevin P. Weinfurt, PhD, Karen L. Staman, MS, and Bradley G. Hammill, DrPH

- Data sharing should be more than just a box-checking exercise to meet a mandate
- Technologies are making data more available and “useful”
- Opportunity >>>> Current practices

NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

Data and Resource Sharing Process for Demonstration Projects

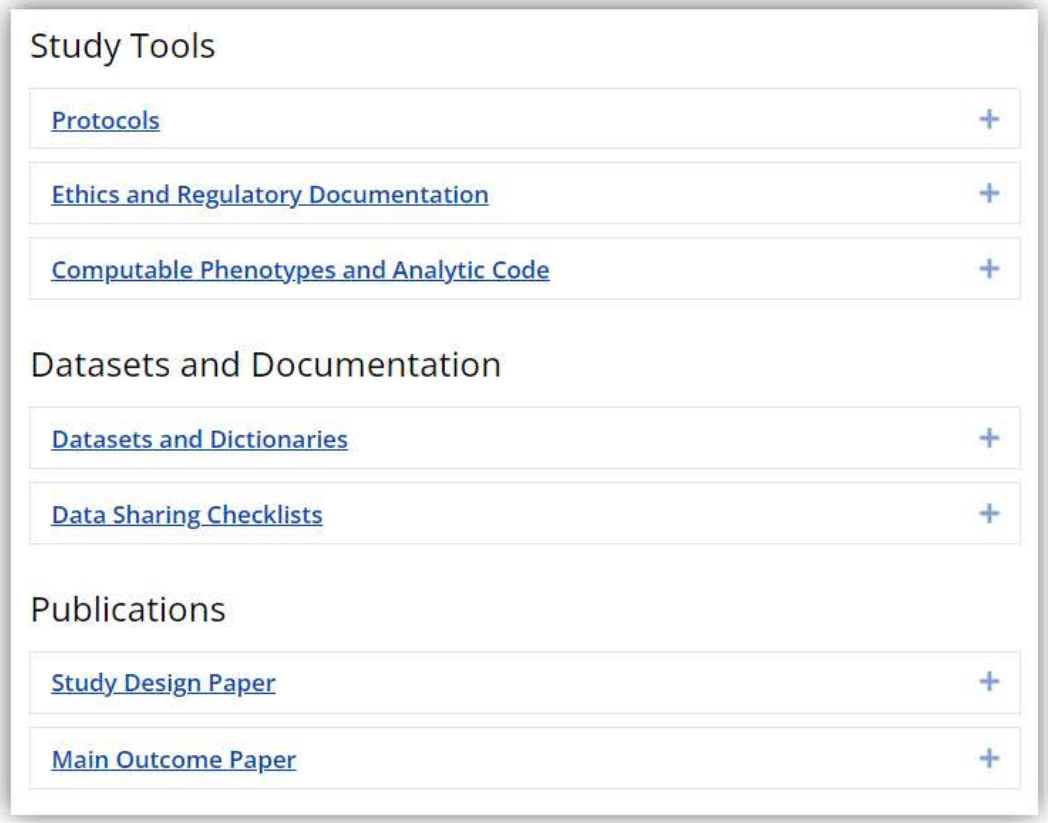
Gina Uhlenbrauck

NIH PRAGMATIC TRIALS COLLABORATORY

Rethinking Clinical Trials®

Coordinating Center Facilitates Projects' Data and Resource Sharing

- Completed projects expected to share data and resources publicly
  - Study tools
  - Datasets and documentation
- Materials added to NIH Collaboratory website at project closeout



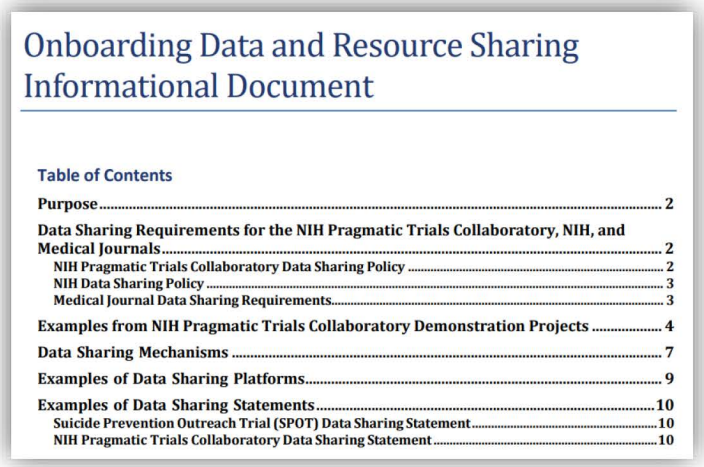
rethinkingclinicaltrials.org/data-and-resource-sharing/

NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

Data and Resource Sharing Preparations

Consult Informational Document

- Data sharing examples
  - From NIH Collaboratory Demonstration Projects
  - Mechanisms
  - Platforms
  - Statements

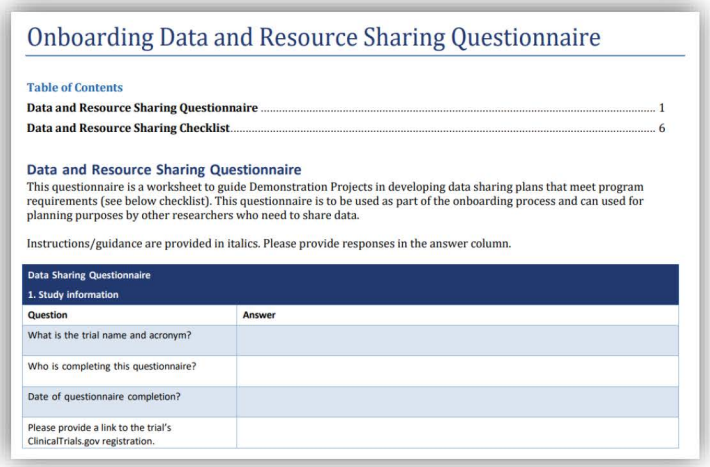


Onboarding Data and Resource Sharing Informational Document

Table of Contents

Complete Onboarding Data and Resource Sharing Questionnaire

- Planning tool for researchers
- Worksheet guides in development of data sharing plan



Onboarding Data and Resource Sharing Questionnaire

Table of Contents

Data and Resource Sharing Questionnaire

This questionnaire is a worksheet to guide Demonstration Projects in developing data sharing plans that meet program requirements (see below checklist). This questionnaire is to be used as part of the onboarding process and can used for planning purposes by other researchers who need to share data.

Instructions/guidance are provided in italics. Please provide responses in the answer column.

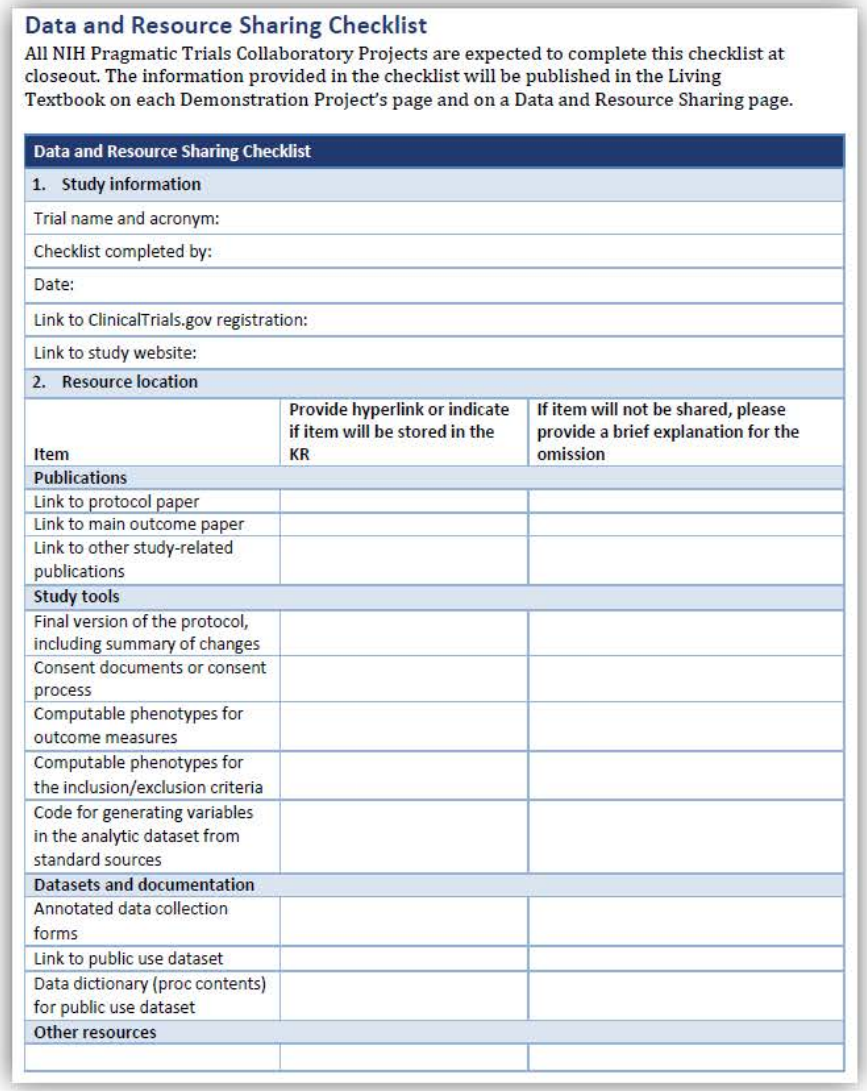
Data Sharing Questionnaire 1. Study information	
Question	**Answer**
What is the trial name and acronym?	
Who is completing this questionnaire?	
Date of questionnaire completion?	
Please provide a link to the trial's ClinicalTrials.gov registration.	

NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

At Project Closeout

Complete Closeout Data and Resource Sharing Checklist

- Provide links or files for posting on the program website
- Share everything requested or indicate why an item cannot be shared
- Coordinating Center will initiate this process



Data and Resource Sharing Checklist

All NIH Pragmatic Trials Collaboratory Projects are expected to complete this checklist at closeout. The information provided in the checklist will be published in the Living Textbook on each Demonstration Project's page and on a Data and Resource Sharing page.

Data and Resource Sharing Checklist		
1. Study information		
Trial name and acronym:		
Checklist completed by:		
Date:		
Link to ClinicalTrials.gov registration:		
Link to study website:		
2. Resource location		
Item	**Provide hyperlink or indicate if item will be stored in the KR**	**If item will not be shared, please provide a brief explanation for the omission**
Publications		
Link to protocol paper		
Link to main outcome paper		
Link to other study-related publications		
Study tools		
Final version of the protocol, including summary of changes		
Consent documents or consent process		
Computable phenotypes for outcome measures		
Computable phenotypes for the inclusion/exclusion criteria		
Code for generating variables in the analytic dataset from standard sources		
Datasets and documentation		
Annotated data collection forms		
Link to public use dataset		
Data dictionary (proc contents) for public use dataset		
Other resources		

NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

Publications, Presentations, and Products Policy

Gina Uhlenbrauck

NIH PRAGMATIC TRIALS COLLABORATORY

Rethinking Clinical Trials®

Publications Committee

- Coordinating Center and NIH program leaders and Demonstration Project representatives
- Oversees the program's publication activities, in accordance with the Publications and Presentations Policy

Publications, Presentations, and Products Policy

- Outlines procedures for Coordinating Center review of publications from the Demonstration Projects and Core Working Groups
- Includes required funding acknowledgment language

NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

Must-Dos for Demonstration Projects

- **Before submission**:
  - Include the appropriate funding acknowledgment in your manuscript
  - Send your manuscript to the Coordinating Center for review; turnaround is 1 week maximum, and usually much quicker
- **After submission**:
  - Notify the Coordinating Center of submission(s)
  - Notify the Coordinating Center of acceptance
  - Ensure your work meets NIH public access requirements, such as inclusion in PubMed Central

Publication Tracking

- Coordinating Center staff will contact you **quarterly** for updates about your publications and presentations

Publications

Treating persistent pain: a nurse co-led, interdisciplinary model for primary care

Economic evaluation: a randomized pragmatic trial of a primary care-based cognitive behavioral intervention for adults receiving long-term opioids for chronic pain

PPACT Closeout Snapshot

Graded chronic pain scale revised: mild, bothersome, and high-impact chronic pain

PPACT Main Outcome Paper

Validating pain communication: current state of the

Presentations

2023 NIH Workshop Panel 1: DeBar, Glassenberg

Lynn DeBar Sharing Study Results Presentation 2023 Steering Committee Meeting

Lynn DeBar Presentation 2023 Steering Committee Meeting

Primary Care-Based Behavioral Treatment for Long Term Opioid Users with Chronic Pain: Primary Results and Lessons Learned from the PPACT Pragmatic Trial

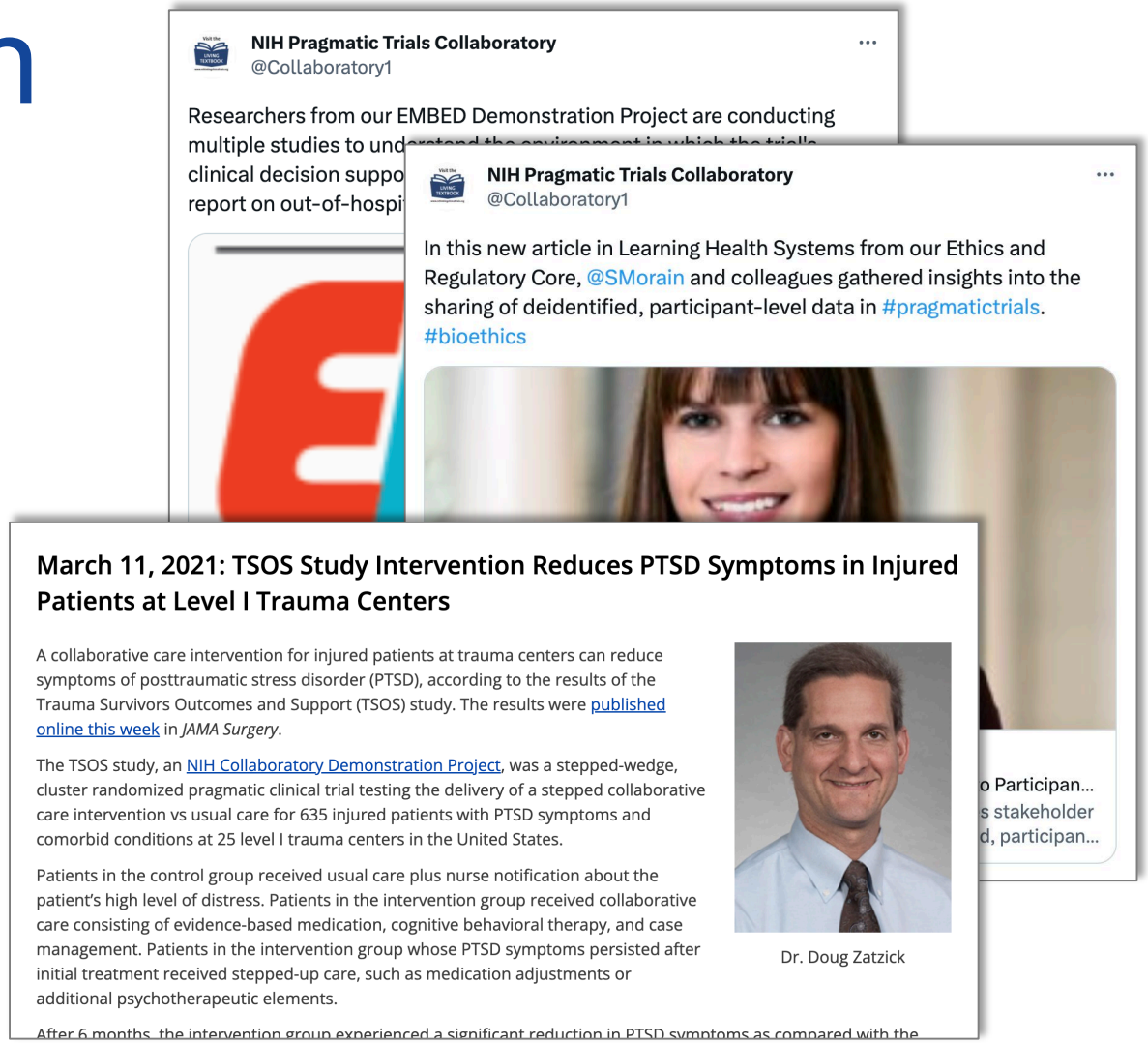
Primary Care-Based Behavioral Treatment for Long Term Opioid Users with Chronic Pain: Primary Results and Lessons Learned from the PPACT Pragmatic Trial

NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

Publication Promotion

Keep us informed so we can track and help promote your NIH Collaboratory work!

nih-collaboratory@dm.duke.edu



NIH PRAGMATIC TRIALS COLLABORATORY
Rethinking Clinical Trials®

Demonstration Project Publication Types

- Many opportunities for study teams to publish throughout the lifecycle of their projects
- Pilot studies, secondary outcomes, lessons learned, and more—in addition to the study design paper and main outcome paper

NIH PRAGMATIC TRIALS COLLABORATORY

Rethinking Clinical Trials®