CRDSA

Clinical Research Data Sharing Alliance

FDA External Controls Guidance Roundtable

- ASCO CancerLinQ
- Duke Clinical Research Institute
- Muscular Dystrophy Association
- Cystic Fibrosis Foundation
- ACRO
- Critical Path Institute
- Project Data Sphere
- Novartis
- COTA Healthcare
- Duke-Margolis
- TransCelerate
- GSK
- UCB

July 19, 2023

Roundtable Recording

The roundtable is available on CRDSA's YouTube channel: https://youtu.be/1WGWTbMU83g

Chapter Locations:

- 00:00 Overview and Objectives
- 11:30 Why alternative control designs?
- 40:00 Overview: Regulatory Challenges
- 41:30 Source Data Access
- 1:07:50 Trust and Provenance
- 1:15:30 Guidance Scope
- 1:34:15 RCT Benchmarking
- 1:48:00 Final Thoughts

Housekeeping (1/2)

This meeting is being recorded.

Please post your questions through the Q&A function.

All users can also upvote and comment on questions.

Please note that questions and comments are *not anonymous*.

Housekeeping (2/2)

All attendees will be muted.

If you need assistance during the webinar, please direct your questions to Jessika via the “Host and Panelists” chat option.

After the meeting we will send a link to the recording and slides. We will also be preparing and sharing a summary report.

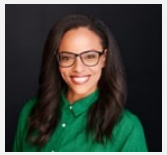
Today's Agenda

- Welcome
- Overview and Objectives
- External and Supplemental/Hybrid Trial Design
- Key Regulatory Challenges
- Actions/Next Steps
- Audience Q&A

Roundtable Panel

Ruthie Davi
Senior Vice President, Data Science, Medidata
Association of Clinical Research Organizations

Laura Fernandes
Senior Statistical Director
COTA



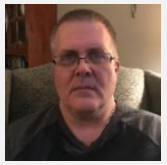
Rachele Hendricks-Sturrup
Research Director of Real-World Evidence
Duke-Margolis Center for Health Policy

Natanya Kerper
Policy Manager, Research and Development
Cystic Fibrosis Foundation

Jessica Lim
Director, Statistics
GSK

Shu Chin Ma
Vice President, Model-Informed Drug Development and
Quantitative Medicine, C-Path

Jon McDunn
Executive Director
Project Data Sphere



Peter Mesenbrink
Executive Director of Biostatistics
Novartis

Paul Melmeyer
Vice President, Public Policy and Advocacy
Muscular Dystrophy Association

Estelle Michael
RWE Engagement and Policy Lead
UCB

Robert Miller
Chief Medical Science Officer
ASCO CancerLinQ

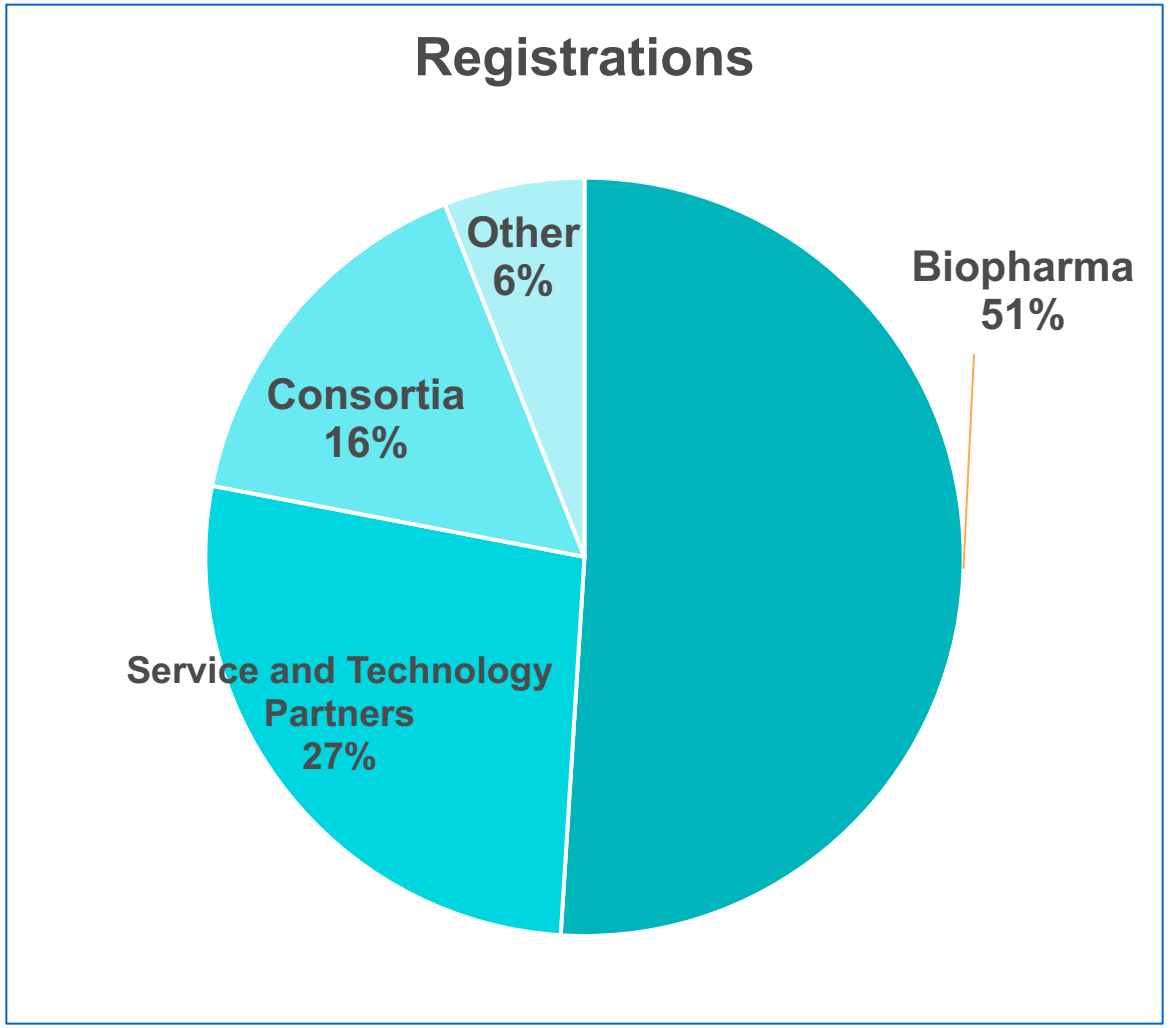
Jagdeep Podichetty
Senior Director of Predictive Analytics, Quantitative Medicine
Program, C-Path

Frank W. Rockhold
Professor of Biostatistics and Bioinformatics
Duke Clinical Research Institute | Duke University Medical Center

Overview and Objectives

Overview

- The FDA posted the draft guidance on January 31, 2023, and comments closed on May 2, 2023
  - https://www.regulations.gov/docket/FDA-2022-D-2983/document
  - The guidance encompasses both prior clinical trial and real-world data
- 205 comments were received / 182 have been publicly posted
  - Approximately 2/3 are from individual patients
- Today's agenda has been informed by:
  - An analysis of over 30 organizational comment documents
  - The pre-meeting registrant survey

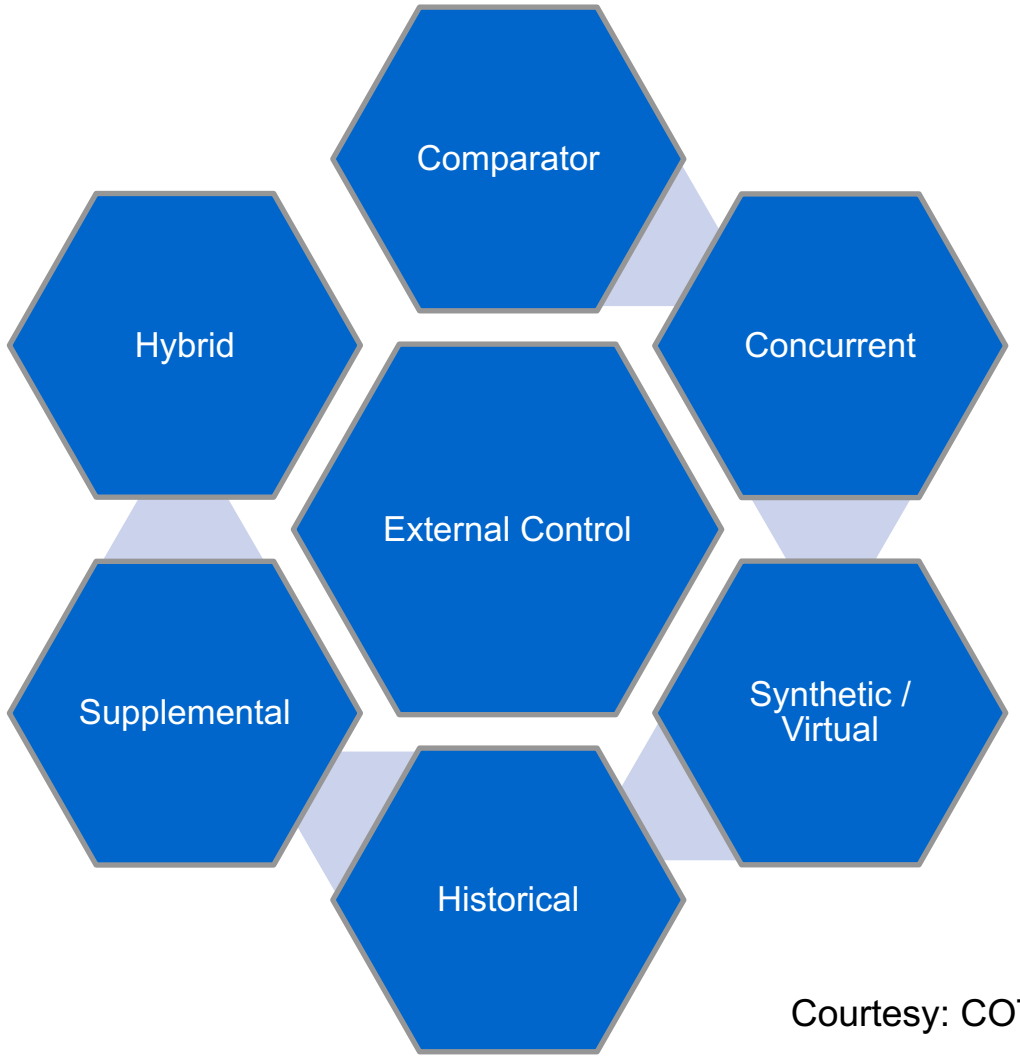


Objectives

1. Identify key regulatory barriers to the adoption of trials using external and supplemental/hybrid control designs
2. Determine collaborative actions to address those barriers, supporting FDA and other regulatory agencies to ensure a regulatory environment that supports alternative control trial designs
   - Promote awareness, participation, and collaboration with current and planned initiatives

Definitions

- Many commenting organizations asked for clarity on various terms
- Definitions are out of scope for today's discussion, but alignment is needed



Courtesy: COTA

Audience Poll

Have you been or are you currently involved in clinical trials using either fully external or supplemental/hybrid controls?

- Yes
- No
- No, but we are actively exploring future use

Why alternative control trial designs?

video timestamp: 11:30

Key Regulatory Challenges

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Discussion: Challenge Areas

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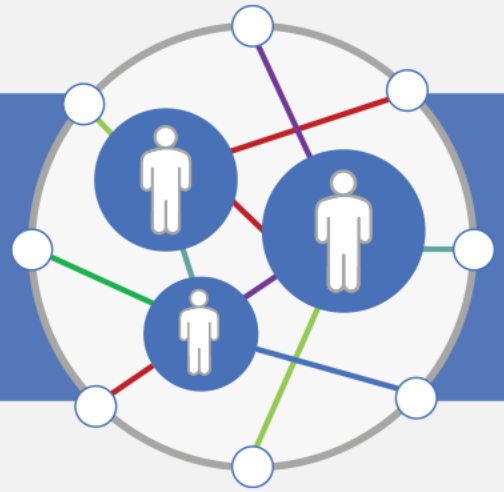
- Access to Source Data
- Data Provenance (Traceability)
- Selection Methodologies
- Guidance Scope
- RCT Benchmarking
- Additional Considerations

Today is about understanding, not problem-solving!



Source Data Access

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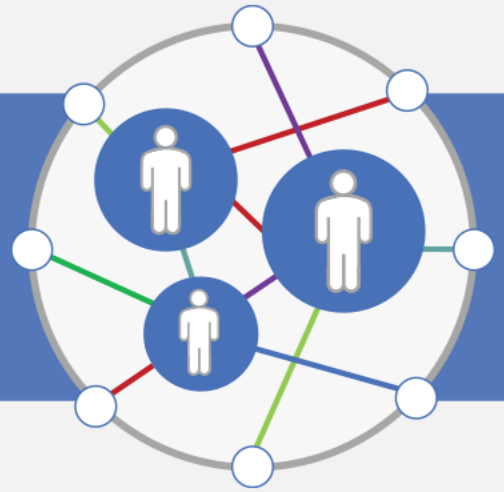

Trust and Provenance

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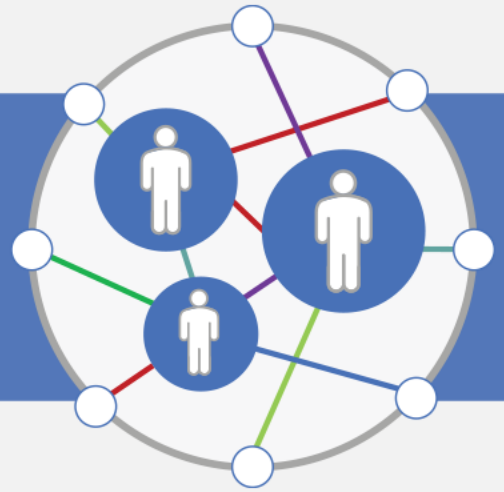

Guidance Scope

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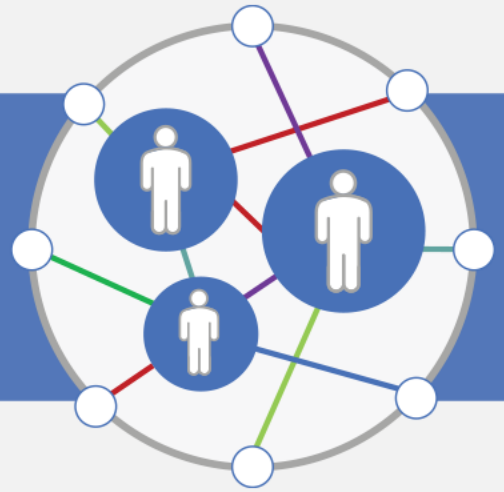

RCT Benchmarking

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Final Thoughts

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Thank you!

For additional resources and information, please visit:

https://crdsalliance.org/resources/#itd