CRITICAL PATH INSTITUTE

Welcome to the 2026 Clinical Outcome Assessment Program Annual Meeting

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April 16-17, 2026
Marriott Metro Center
Washington, D.C.

FDA Acknowledgement

Critical Path Institute is supported by the Food and Drug Administration (FDA) of the Department of Health and Human Services (HHS) and is 43% funded by the FDA/HHS, totaling $20,724,703, and 57% funded by non-government source(s), totaling $27,346,613. The contents are those of the author(s) and do not necessarily represent the official views of, nor an endorsement by, FDA/HHS or the U.S. Government.

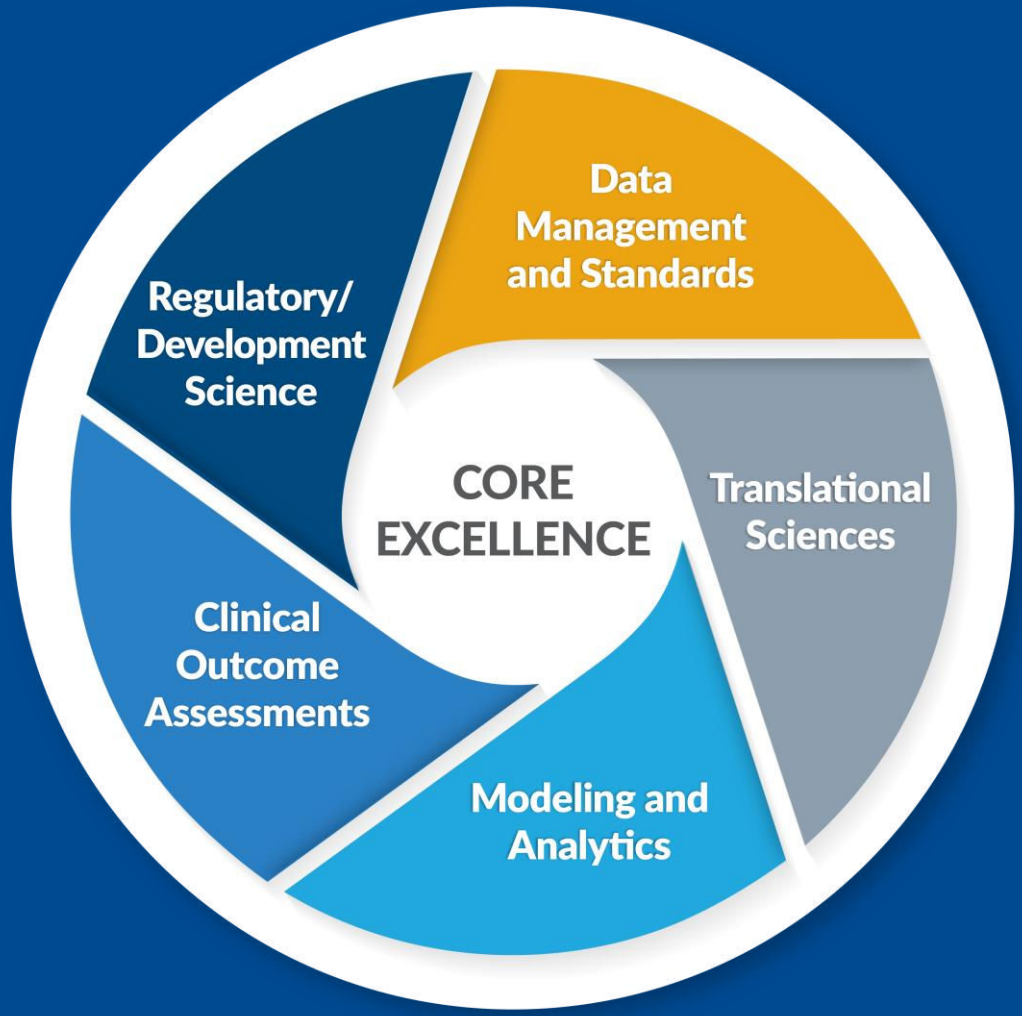
Last updated on 11/20/2025 and based on C-Path FY2025 annual audit.

Disclaimer

The views and opinions expressed in the following slides are those of the individual presenters and should not be attributed to their respective organizations/companies, the U.S. Food and Drug Administration, or the Critical Path Institute.

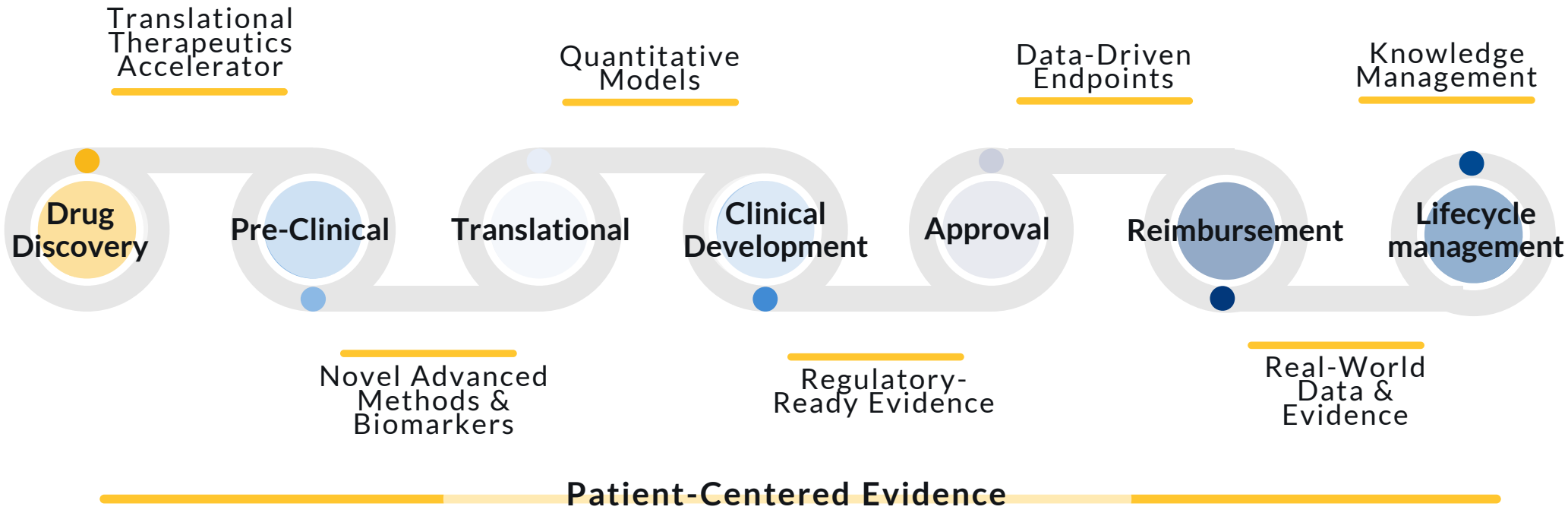
C-Path's Mission

C-Path leads collaborations that accelerate drug development, advancing better treatments for people worldwide.



Our Reach: Advancing a seamless clinical development and product lifecycle

Drug Development Lifecycle Enhanced with Integrated, Patient-Centered C-Path tools



C-Path's tools and data-driven methods aren't limited to one stage or one problem—
they're built to improve decision making across the entire drug development lifecycle.

C-Path Scales What Works Across Therapeutic Areas

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Deep disease expertise provides real-world context and scientific credibility

- Neurology
- Rare/Orphan
- Pediatrics
- Infectious Disease
- + **New Areas of Unmet Need**

Global regulatory engagement from the start enables impact for patients on a global scale

- FDA
- EMA
- PMDA
- + regional health authorities

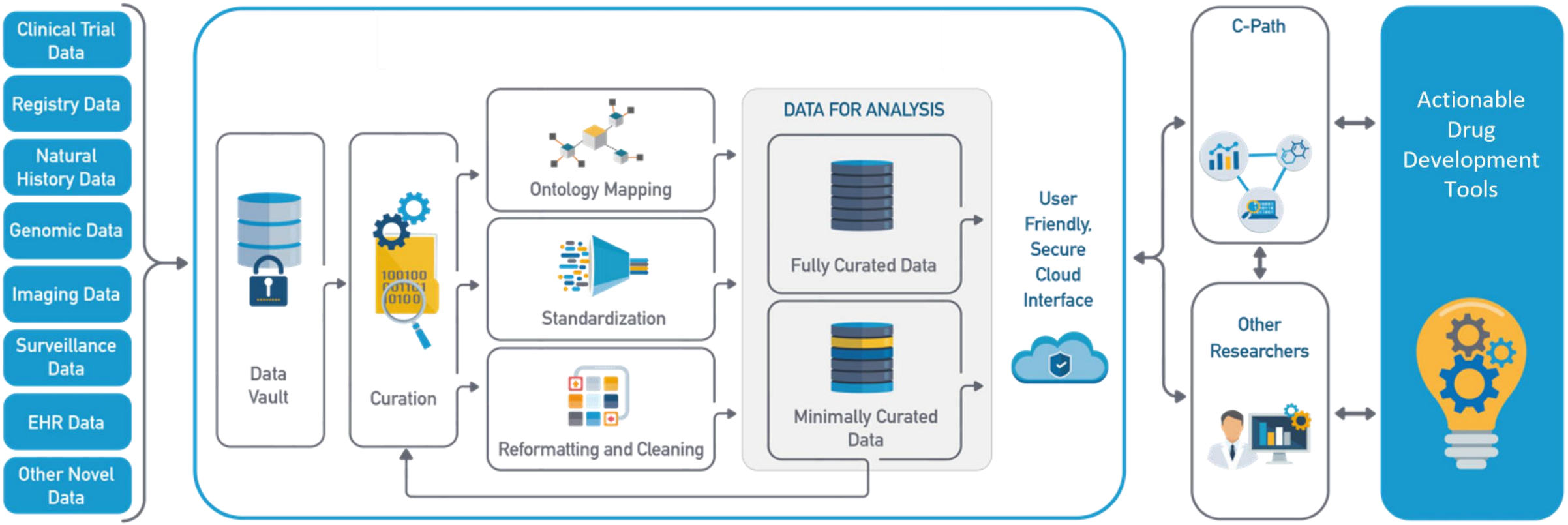
Core competencies turn disease evidence into reusable tools

- Data Management & Standards
- Translational Sciences
- Modeling & Analytics
- Clinical Outcome Assessments
- Regulatory & Development Science

Maximized learnings across programs

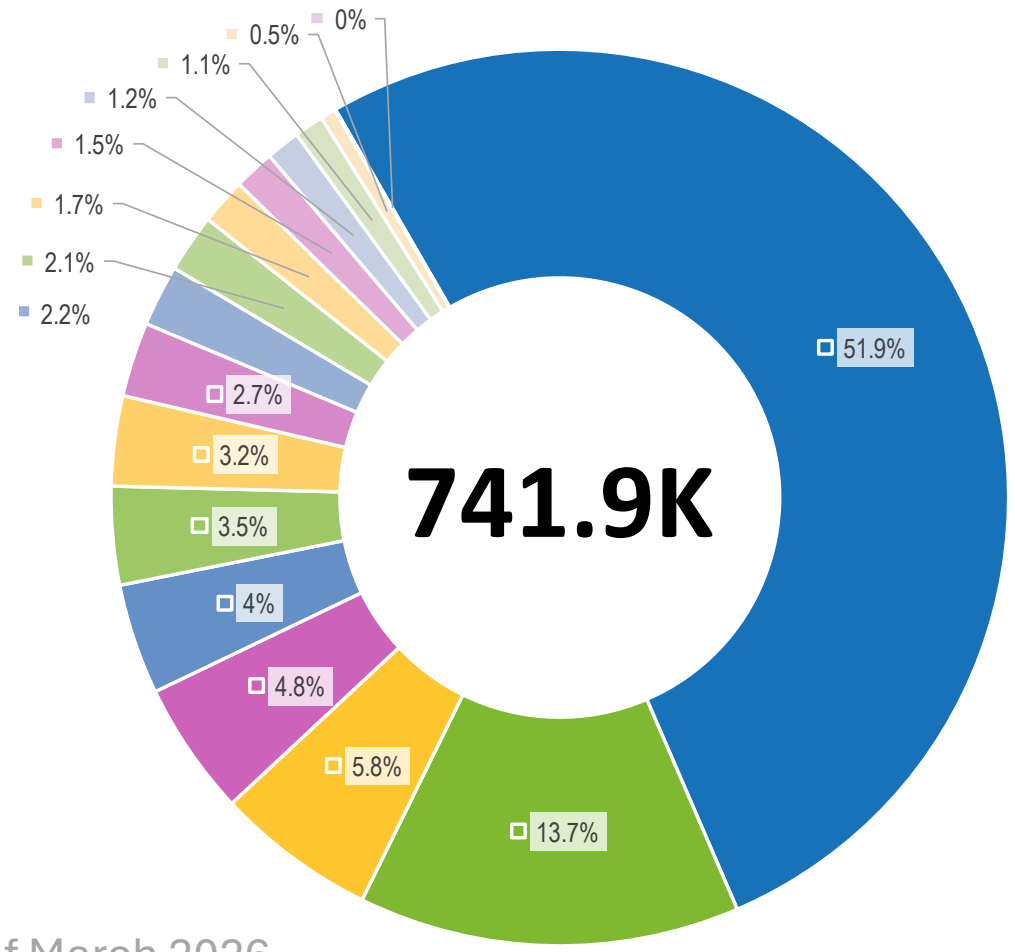
Approaches proven in one area can be adapted and deployed in another

Transforming Data into Actionable Knowledge



C-Path Subject Data and Access

Subjects by Disease Area



as of March 2026

Subject Data Access

132,554 Individual subject records available to external researchers through current data sharing agreements

Public Resources

9 Online databases and platforms available for public research applications

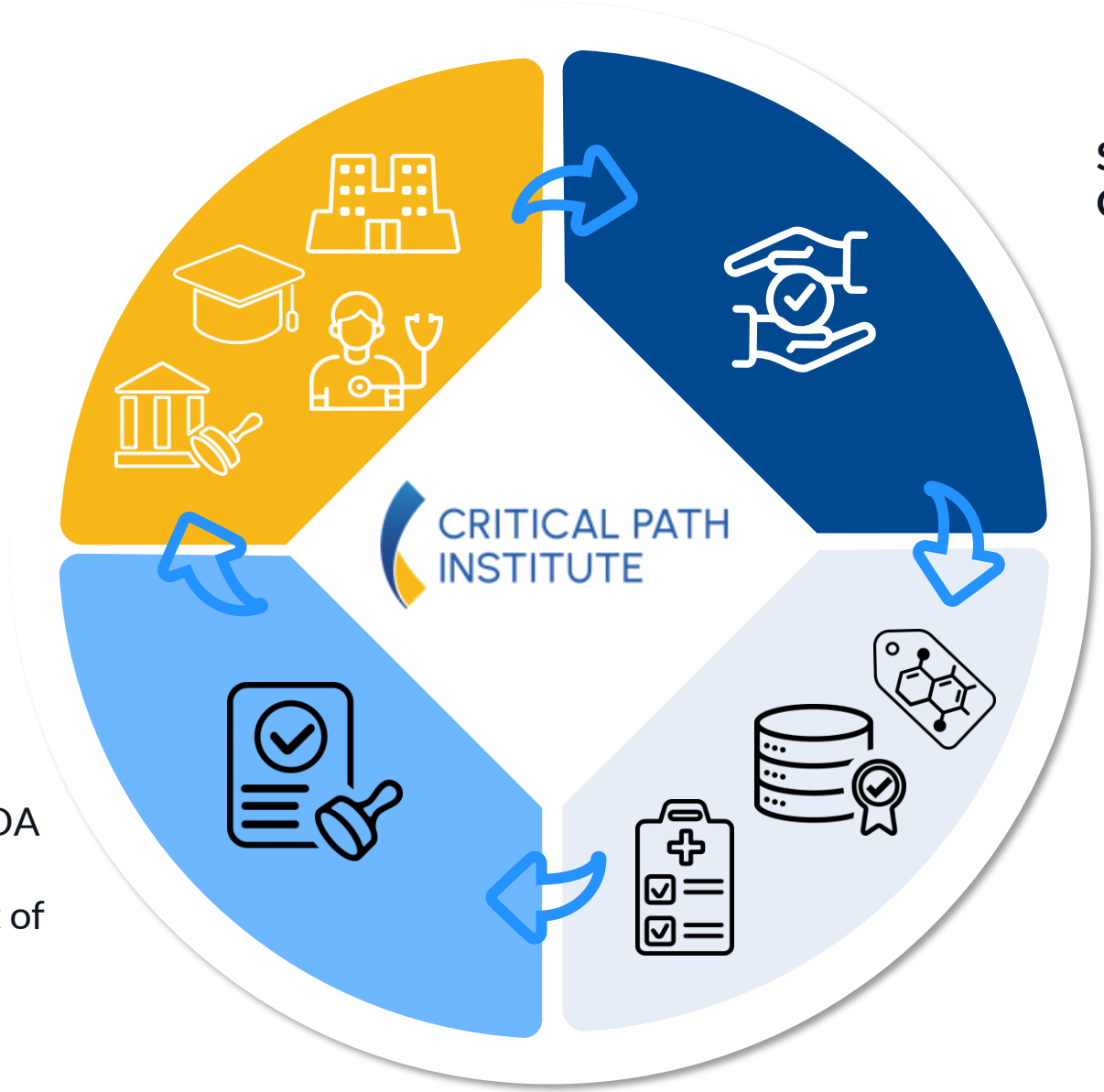
Groups and Subject Counts

- Neonatal Conditions, 384.7K
- Type 1 Diabetes, 42.9K
- COVID-19, 29.6K
- Rare Diseases, 24.1K
- Parkinson's Disease, 16.4K
- Safety & Biomarkers, 12.4K
- Polycystic Kidney Disease, 9.1K
- Amyotrophic Lateral Sclerosis, 4K
- Alzheimer's Disease, 101.9K
- Tuberculosis, 35.8K
- Kidney Transplant, 26.3K
- Huntington's Disease, 19.9K
- Multiple Sclerosis, 15.6K
- Duchenne Muscular Dystrophy, 10.9K
- Sickle Cell Disease, 7.9K
- Lysosomal Diseases, 0.3K

How We Work

Our Public-Private Partnership Model

A Pre-Competitive Neutral Environment
Bringing Industry, Patients, Academia, and Regulators



Structured, Inter-Disciplinary Collaborations

- Consensus-based development
- Shared investment and accountability
- Integrated scientific expertise

Regulatory & Practice Adoption → Patient Impact

- Iterative FDA/EMA/PMDA engagement
- Regulatory endorsement of tools
- Adoption

Regulatory-Grade Tools & Methods

- New evaluation tools
- Endpoints and models
- Data standards and analytics

Agenda – Day 1 Morning

9:00–9:15 am	Welcome
9:15–10:15 am	Update on FDA's COA Qualification Program, ISTAND Program, and Patient-Focused Drug Development Initiatives
10:15–10:30 am	Spotlight: Best Practice Recommendations and Considerations for Designing and Electronically Implementing Event-Driven Diaries in Clinical Trials
10:30–11:00 am	Break
11:00–12:15 pm	Incorporating the Patient Voice into Clinical Trial Design: More Than Just Checking the Box
12:15–1:30 pm	Lunch – Second Floor Junior Ballroom Foyer

Agenda – Day 1 Afternoon

1:30–1:45 pm	Patient-Centered Evidence Consortium Update
1:45–3:00 pm	Pulling Back the Curtain: Multi-stakeholder Insights on Electronic Clinical Outcome Assessment Implementation
3:00–3:30 pm	Break
3:30–3:45 pm	Rare Disease Clinical Outcome Assessment Consortium Update
3:45-4:45 pm	Expanding the Toolbox to Interpret the Meaningfulness of Treatment Effects
4:45–4:50 pm	Day 1 Closing Remarks
4:50–5:30 pm	Open
5:30–7:00 pm	Networking Reception – Ballroom Level Foyer

Agenda – Day 2

7:30–8:30 am	Registration and Breakfast – Ballroom Level Foyer
8:30–8:45 am	Welcome and Electronic Clinical Outcome Assessment Consortium Update
8:45–10:00 am	Using Patient Experience Data to Inform Value Assessment for Access Decision Making
10:00–10:30 am	Break
10:30–11:30 am	Navigating Methodological Challenges in Rare Disease Clinical Trials: Lessons from Real-World Case Studies
11:30–12:30 pm	How Multidisciplinary Teams Form a Comprehensive Approach to Digitally-derived Endpoints
12:30–12:35 pm	Annual Meeting Wrap Up
12:35–1:30 pm	Lunch – Ballroom Level Foyer

We’re paperless!

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Scan this QR code with your phone’s camera app to access event materials via Cvent

Active Participation During the Q&A Portion of Each Session Is Encouraged

If you have a question, please raise your hand and a microphone will be brought to you

The workshop is being audio recorded

Please turn off cell phones or set to vibrate

Thank You!

Advancing Drug Development. Improving Lives. Together