2025 Clinical Outcome Assessment Program Annual Meeting Recap

C-Path's Clinical Outcome Assessment (COA) Program recently held its Annual Meeting in Rockville, MD. Roughly 300 attendees gathered for two days of sessions and discussions on the methodologies and strategies that will advance drug development and regulatory approaches in COAs across a wide range of therapeutic areas. Full meeting recordings and slides will be posted to the C-Path website by the end of May 2025, but in the meantime, key highlights are summarized below:

Current thinking on the accessibility and burden of Electric Clinical Outcome Assessment (eCOA) and Digital Health Technology (DHT) system use

- Language should be chosen that resonates with stakeholders. E.g., "burden" might be the right word to describe what we're trying to reduce in electronic administration, but it may cause internal teams to be wary of using COAs at all because the word itself unnecessarily conveys some level of risk.

- The lack of published or publicly available evidence and guidance around the embedding of accessibility features into eCOA and DHTs is a problem, and one that the eCOA Consortium and PRO Consortium are trying to address via our *Accessibility of ePROs* collaborative project. The call for

sponsors to share evidence and experiences – of both successes and failures – was clear.

- The combination of sensor-based DHTs and eCOA presents clear opportunities for more detailed perspectives on the trial participant's health status and experience, with the added potential to increase engagement and reduce "burden."

The potential for AI for COA development and deployment in clinical trials

- Human oversight is necessary when using AI to analyze transcripts or prepare patient facing documents.

- More immediate efficiencies can be gained using AI for administrative tasks, such as preparing documents.

- There are still many unknowns around leveraging AI in the development and implementation of COAs; collaborative research is needed to create best practices.

Regulatory pathways for using patient experience data: Navigating benefit-risk assessment pre-approval and communications consistent with labeling post-approval

- Incorporating patient perspectives in structured benefit-risk (sBR) assessment can help to ensure that patients have access to treatments that best meet their needs, leading to better treatment outcomes and improved lives.

- Although both regulators and sponsors have been increasingly considering the patient perspective, more transparency is needed to address how to incorporate and communicate such data in sBR assessments.

- Companies have an opportunity to leverage the "Consistent with FDA-Required Labeling (CFL)" pathway as an alternate communication strategy, other than the product label and publications, for sharing important patient experience data (PED) about medical product characteristics to key stakeholders.

Implementation insights in Rare Disease COA

- People and caregivers with lived experiences with a rare disease frequently have to make large sacrifices to be able to participate in clinical trials, especially when the therapy may be disease modifying.

- Collaborating and educating trial participants and their caregivers on how and why certain COAs and COA-based endpoints are being included in the trial is key to study compliance.

- Thoughtful and early internal discussions between outcomes researchers and clinical operation teams are essential to optimizing COA data collection and interpretation.? Trial design and plans for interpreting COA data to support COA-based efficacy endpoints need to be planned *a priori* to maximize the utility and usability of COA data.

Using patient-centered evidence to inform decision making across the drug development lifecycle: navigating a varied landscape

- Stakeholders throughout the drug development lifecycle use patient-centered evidence to answer different questions, and therefore, the evidence needs to be packaged differently to address their specific needs. For example, regulatory agencies focus on benefit-risk assessment of efficacy and safety of treatments, while health technology assessment agencies are concerned about value and cost-effectiveness of treatments, and different evidence is needed in each case.

- It is essential to start planning an evidence strategy early in drug development to ensure the necessary evidence is assembled throughout the process, and this early planning needs to involve key internal stakeholders including outcomes measurement scientists, medical affairs, regulatory affairs, as well as clinical teams to ensure the right information is collected in time for the relevant submissions.

Overall, the meeting underscored the importance of forming COA strategies that incorporate all stakeholder perspectives and needs, particularly those of the patients participating in trials and the regulators reviewing evidence packages. A multidisciplinary approach to COA development and implementation will allow for COA evidence to be fully leveraged in telling the story of the patient experience with a new therapy to help inform regulatory, reimbursement, and treatment decisions.