

## Recommended Approaches for Analyzing Clinical Outcomes Assessment Data from Oncology Trials for Different Stakeholders

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**OBJECTIVES:** When making decisions about evaluation, approval, reimbursement, and use of oncology treatments, various stakeholders such as health technology assessment agencies, payers, oncologists, and medical journal editors have different questions that must be answered to determine the efficacy of treatments. Each stakeholder requires different evidence that is informed by different approaches to clinical outcomes assessment (COA) measurement and analysis.

**METHODS:** A review was undertaken in September 2020 to identify recommended COA analytic strategies in stakeholders' guidance documents. Strategies were compared between stakeholders to provide an 'easy reference guide' for the comprehensive analysis of COA data in oncology clinical trials.

**RESULTS:** Three important considerations in the selection, application, and analysis of COAs were identified in the guidance documents. First, COA instruments should be "fit for purpose" to produce valid, unambiguous, and interpretable data. COA instruments may not be considered if they are not "fit for purpose," even if all outcome analytic recommendations are followed. Second, implementation and analysis of COA-based endpoints should be done in the context of the estimated framework, which is essential for specifying analytic plans. Finally, study protocols should consider COA data as central to determining efficacy and/or safety of treatments. COA-based endpoints will not be used to inform regulator and payer decision-making without pre-specification, alpha-control, and appropriate positioning (i.e., endpoint hierarchy). Endpoints need to be supplemented with descriptive analyses and sensitivity analyses to provide data on group- and individual-level treatment response. An easy reference guide has been developed including evidence that is required for regulators, payers, and "others," such as journal editors, clinicians, or patients to structure analysis plans and analytic methods for COAs, to help meet the needs of multiple stakeholders.

**CONCLUSIONS:** The reference guide will supplement current recommendations from the SISAQOL Consortium by specifying different analytic approaches for various stakeholders.