

# The SISAQOL-IMI Recommendations on Standardising the Use of Thresholds for Interpretation of Patient-Reported Outcome Results in Cancer Clinical Trials

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## OBJECTIVE

To develop international guidelines for standardisation of analysis and interpretation of patient-reported outcome (PRO) data in cancer trials, including guidance on terminology and appropriate use of PRO score interpretation thresholds

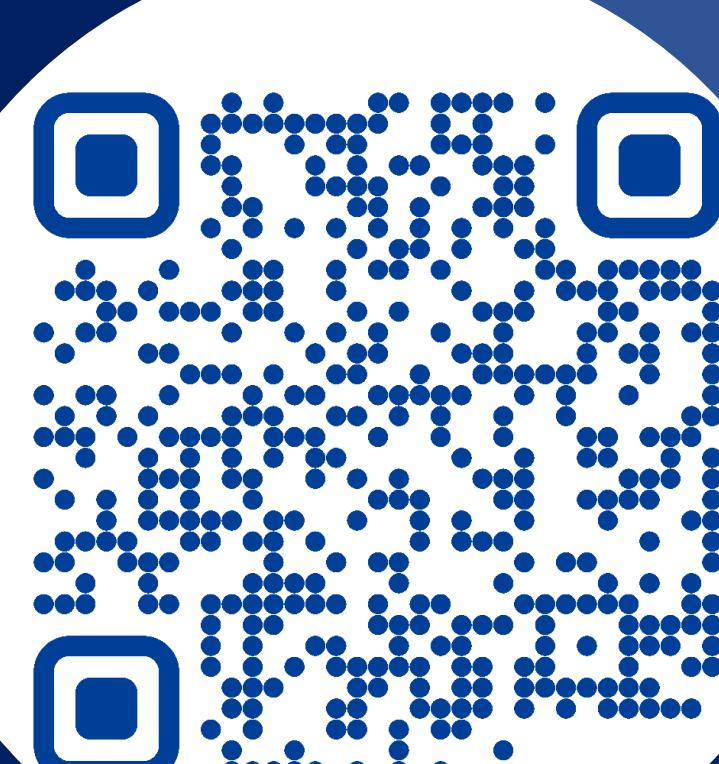
## METHODS

The SISAQOL-IMI Recommendations

- Were developed by a multi-stakeholder consortium of 41 organisations representing clinicians, patients, academics, industry, regulators, and HTA bodies
- Were derived from a four-year consensus process including, e.g., five in-person consensus rounds, virtual meetings, a patient workshop, and stakeholder surveys
- Built on the estimands framework
  - Are readily accessible through various resources

## RESULTS

- Development of a harmonised terminology with precise definitions for PRO score interpretation thresholds
- Clarification and support of key criteria for selecting thresholds, including the use of patient-centered anchors
- Clear distinction between patient-level and group-level thresholds
- Guidance on linking specific threshold types to appropriate statistical analysis methods
  - A total of 27 recommendations concerning PRO score interpretation thresholds in cancer clinical trials
  - Detailed information available at [www.sisagol-imi.org](http://www.sisagol-imi.org)



### EXAMPLE RECOMMENDATIONS ON REPORTING

THE TRIAL PROTOCOL, STATISTICAL ANALYSIS PLAN, OR TRIAL PUBLICATION SHOULD:

- Provide a rationale for choosing a certain PRO score interpretation threshold
- Report on what type of methodology the patient- and group-level PRO score interpretation thresholds are based on (e.g., anchor-based, or distribution-based)
- State clearly if the PRO score interpretation thresholds are used to interpret meaningful within-patient change, meaningful within-group change, meaningful between-group difference, or meaningful between-group difference in change
- Include a short description of the patient population in which the PRO score interpretation thresholds were established (e.g., diagnosis, or treatment type)



### EXAMPLE TERMS AND DEFINITIONS

**TERM:**  
Threshold for 'meaningful within-patient change'

**DEFINITION:**  
'Thresholds for a meaningful within-patient change are reference scores for interpreting the magnitude of change of a PRO score of an individual patient that indicates what would be viewed as meaningful improvement or deterioration to an average patient.'

## CONCLUSION

The SISAQOL-IMI recommendations and terminology for PRO score interpretation thresholds will support the ANALYSIS, INTERPRETATION and the REPORTING of PRO data in cancer clinical trials, leading to a more valid and critically appraisable understanding of PRO-based endpoints.

## LITERATURE

Setting International Standards in Analyzing Patient-Reported Outcomes and Quality of Life Endpoints in Cancer Clinical Trials-Innovative Medicines Initiative (SISAQOL-IMI): stakeholder views, objectives, and procedures. Pe M, Alanya A, et al. Lancet Oncol. 2023 Jun;24(6):e270-e283. doi: 10.1016/S1470-2045(23)00157-2  
International standards for the analysis of quality-of-life and patient-reported outcome endpoints in cancer randomised controlled trials: recommendations of the SISAQOL Consortium. Coens C, Pe M, et al. Lancet Oncol. 2020 Feb;21(2):e83-e96. doi: 10.1016/S1470-2045(19)30790-9.

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