EORTC
European Organisation for Research and Treatment of Cancer
The future of cancer therapy

The SISAQOL initiative

SISAQOL

Establishing International Standards and Recommendations for the Analysis of Patient-Reported Outcomes and Quality of Life Data in Oncology Randomized Clinical Trials

L Dorme[1], M Pe[1], C Coens[1], E Basch[2], M Calvert[3], A Campbell[4], C Cleeland[5], K Cocks[6], L Collette[1], N Devlin[7], A Dueck[8], HH Flechtner[9], C Gotay[10], I Griebsch[11], M Grønvold[12], LL Johnson[13], M King[14], PG Kluetz[13], M Koller[15], DC Malone[16], F Martinelli[1], SA Mitchell[17], JZ Musoro[1], D O'Connor[18], K Oliver[19], E Piault-Louis[20], M Piccart[21], C Quinten[22], JC Reijneveld[23], C Schürmann[24], J Sloan[25], AW Smith[17], KM Soltys[26], R Sridhara[13], MJB Taphoorn[27], G Velikova[28], A Bottomley[1]

BACKGROUND

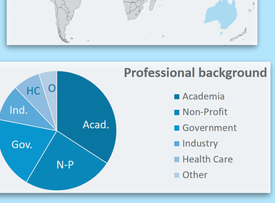
SISAQOL — **S**etting **I**nternational **S**tandards in **A**nalyzing Patient-Reported Outcome (PRO) and **Q**uality **o**f **L**ife Endpoints Data

AIM — Standardize PRO analysis in oncology randomized clinical trials

WHY — Heterogeneity in analysis, interpretation & reporting of PRO data (Ref 1)

WHO — International expert consortium

The SISAQOL consortium *(N=41)*



Professional Role	N*
Researcher	24
PRO expert advisor	14
Statistician	12
Clinician	10
Trials methodologist	9
Regulator	5
Industry representative	3
Health psychologist	3
Health economist	2
Reviewer	2
Patient representative	1
Journal editor	1

**One consortium member can hold multiple roles*

METHODS

Priorities

Objectives — Specify <u>well-defined</u> **PRO research objectives**

Methods — Recommend <u>appropriate</u> **methods** for -PRO data analysis -Missing data

Terminology — Standardise **terminology** -Objectives -PRO Statistical terms -Missing data

Process

Development of consensus recommendations through:

- Literature review
- Surveys
- Meeting discussions
- Consensus voting

Consensus recommendations for PRO analysis

RESULTS

Consensus recommendations for PRO analysis

- Taxonomy of well-defined PRO research objectives (Ref 2)
- Key features of good statistical approaches for PRO analysis
- Appropriate statistical methodology according to PRO research objective
- Standardized reporting of missing data rates

CONCLUSION

- First set of robust **PRO analysis recommendations**
- Needs and requirements of **diverse international stakeholders** addressed
- Strong **foundation for widespread endorsement** of recommendations

Future direction

- **Finalize discussion on methods for remaining objectives**
- **Further standardize statistical terminology**
- **Standardize data collection for reasons for missingness**
- **Extend analysis recommendations for ordinal outcomes**
- **Extend recommendations for dealing with missing data**
- **Translate recommendations to estimands framework**

AFFILIATIONS & DISCLAIMER

[1]*European Organisation for Research and Treatment of Cancer, Belgium,* [2]*Lineberger Comprehensive Cancer Center; University of North Carolina, USA,* [3]*Centre for Patient Reported Outcomes Research, University of Birmingham, UK,* [4]*Patient Relevant Evidence, USA,* [5]*Dept. Of Symptom Research, MD Anderson Cancer Center; University of Texas, USA,* [6]*Adelphi Values, UK,* [7]*University of Melbourne, Australia,* [8]*Alliance Statistics and Data Center; Mayo Clinic, Arizona, USA,* [9]*Clinic for Child and Adolescent Psychiatry and Psychotherapy; University of Magdeburg, Germany,* [10]*School of Population and Public Health; University of British Columbia, Canada,* [11]*Boehringer-Ingelheim, Germany,* [12]*Dept. Of Public Health; Bispebjerg Hospital, University of Copenhagen, Denmark,* [13]*US Food and Drug Administration, USA,* [14]*School of Psychology and Sydney Medical School; University of Sydney, Australia,* [15]*Center for Clinical Studies; University Hospital Regensburg, Germany,* [16]*College of Pharmacy, University of Arizona, USA,* [17]*National Cancer Institute, USA,* [18]*Medicines and Healthcare products Regulatory Agency, UK,* [19]*International Brain Tumour Alliance, UK,* [20]*Genentech, USA,* [21]*Institut Jules Bordet; Université Libre de Bruxelles, Belgium,* [22]*European Centre for Disease Prevention and Control, Sweden,* [23]*VU University Medical Center, Dept. of Neurology & Brain Tumor Center, The Netherlands,* [24]*Institute for Quality and Efficiency in Health Care, Germany,* [25]*Alliance Statistics and Data Center; Mayo Clinic, Minnesota, USA,* [26]*Health Canada, Canada,* [27]*Leiden University/Haaglanden Medical Center, The Netherlands,* [28]*Leeds Institute of Cancer and Pathology; University of Leeds, St. James's Hospital, UK*

Disclaimer: The views here reflect that of the individual presenter and should not be construed to represent official views or policies of the US Food and Drug Administration, US National Cancer Institute, Medicines and Healthcare products Regulatory Agency, Institute for Quality and Efficiency in Health Care, Germany, European Centre for Disease Prevention and Control, Health Canada or any other agency or organization.
EORTC received an unrestricted education grant from Boehringer Ingelheim GmbH to initiate the SISAQOL work, an unrestricted education grant from Genentech to continue the SISAQOL work, and additional financial support provided by the EORTC Cancer Research Fund.

Ref 1. Bottomley A, Pe M, Sloan J, Basch E, Bonnetain F, Calvert M, Campbell A, Cleeland C, Cocks K, Collette L, Dueck AC, Devlin N, Flechtner HH, Gotay C, Greimel E, Griebsch I, Groenvold M, Hamel JF, King M, Kluetz PG, Koller M, Malone DC, Martinelli F, Mitchell SA, Moinpour CM, Musoro J, O'Connor D, Oliver K, Piault-Louis E, Piccart M, Pimentel FL, Quinten C, Reijneveld JC, Schürmann C, Smith AW, Soltys KM, Taphoorn M, Velikova G, Coens C. (2016). Analysing data from patient-reported outcome and quality of life endpoints for cancer clinical trials: a start in setting international standards. *The Lancet Oncology*, 17(1), e510-e514.

Ref 2. Pe M, Dorme L, Coens C, Hamel JF, Basch E, Bonnetain F, Calvert M, Campbell A, Cleeland C, Cocks K, Collette L, Dueck AC, Devlin N, Flechtner HH, Gotay C, Greimel E, Griebsch I, Groenvold M, Johnson LL, King M, Kluetz PG, Koller M, Malone DC, Martinelli F, Mitchell SA, Musoro J, O'Connor D, Oliver K, Piault-Louis E, Piccart M, Pimentel FL, Quinten C, Reijneveld JC, Schürmann C, Sloan J, Wilder Smith A, Soltys K, Sridhara R, Taphoorn MJB, Velikova G, Bottomley A. Improving standards of patient reported outcomes analysis: developing a consensus taxonomy of key research objectives – a SISAQOL initiative. 25th Annual Conference of the International Society for Quality of Life Research, Dublin, Ireland, October 2018. *Qual Life Res* 27, 1, ab203.4, p:35-36.

Icons: www.thenounproject.com - background: Designed by starline / www.freepik.com

ISPOR EU 2019
ID: 96614 - PCN457 - U18