The European Regulatory Issues on Quality of Life Assessment (ERIQA) Project

Health Outcomes Conference 1999

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Background

Exploratory meetings on Health-related Quality of Life and Regulatory Issues

- Vienna, November 4-5th, 1997:

  Mapi Research Institute

- Milano, October 1997:

  Mario Negri Institute, GlaxoWellcome

Background (cont'd)

- Need to rationalise the field of HRQL and to make it credible as a criterion of evaluation to the regulatory authorities.
- Issue to be only resolved through a better collaborative effort between key players: regulatory authorities, HRQL researchers and pharmaceutical companies.

➔ Creation of the ERIQA Group

Mission Statement

« Establishing principles and practices for the integration of health-related quality of life outcomes in the regulatory process »

Main Objective

To provide European regulatory authorities (RA) with guidance on:

- how to assess the quality of HRQL studies in clinical trials,
- how to evaluate the validity of HRQL claims

Target

- EMEA
  - Permanent Staff: Human Products Unit
  - CPMP Members: 30 people (2 per country)

  ➔ Registration
- Country-Specific Agencies
  - Registration
  - Pricing, Reimbursement
  - Promotion

Contributors

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Sponsors

- Mapi Research Institute, Lyon, France
- Astra Hässle, Mölndal, Sweden
- Bayer, Leverkusen, Germany
- GlaxoWellcome, Verona, Italy
- Janssen Research Foundation, NJ, USA
- Rhône Poulenc Rorer, Antony, France
- Schering AG, Berlin, Germany

Organisation: 2 Phases

Phases/Steps	Deliverables
Phase I	**for Phase I**
step 1: Search and review of existing guidelines	1. Report
step 2: Production of a reference document	2. Reference document
step 3: Decide target areas and draft guidance : COPD/asthma, cancer as pilot areas	3. Draft guidance on COPD/asthma and cancer
step 4: Presentations to RA	**ALL 3 materials**
Phase II	**for Phase II**
Consensus meetings with all key players	consensus guidance on specific diseases

Ph.I - St. 1: Report

- review and synthesis of existing guidance
- special focus on the EMEA documents

Ph.I - St. 1: Conclusions

- No documents directly focused on HRQL measures
- A few explicit recommendations (1 generic + 11 conditions/drugs)
- Most of the times, generic recommendations
- Only one example with recommendations regarding dimensions to be included or criteria to be satisfied, ...

Ph.I - St. 1: Conclusions (cont 'd)

- Only 2 questionnaires cited
- No comment on generic/specific issue
- Only few warnings on "cultural problems"
- Absence of recommendations in expected disease/drugs (ie, hypertension)

Ph.I - St. 2: Reference Document

- basic guidance for RA to evaluate the quality of HRQL evaluation in RCTs and the validity of HRQL claims.
  - 1. basic statements on HRQL issues;
  - 2. checklist of key issues to be addressed/analysis grid
  - 3. examples taken from specifc pathologies or areas.
- applicable to all areas

Checklist on reporting on HRQoL in RCTs *(main issues)*

- objective
- implementation of HRQoL
- justification of sample size
- clear handling of missing data
- description of follow-up
- correct presentation of results
- interpretation of results

Ph.I - St. 3: Specific Draft Guidances

- pilots with two indications
  - cancer
  - COPD - asthma
- body of evidence important

Phase II

- development of consensus guidance for specific diseases

- using:
  - analysis grid developed in phase I
  - consensus meetings

Other initiatives

- Food and Drug Administration **(FDA)**
- Health Outcomes Work Group of Pharmaceutical Research and Manufacturers of America **(PhRMA HOWG)**
- International Society for Pharmacoeconomics and Outcomes Research **(ISPOR)**

FDA suggestions to industry

- Determine CLAIMS before study initiation.
- Ensure that

  the INSTRUMENT,

  its DEVELOPMENT AND VALIDATION history,

  the PROTOCOL, and

  the DATA ANALYSIS PLAN are adequately developed to support the desired claims.
- If an agency advisory opinion or action is sought, or if responding to an agency inquiry regarding promotional materials, submit all of above

PhRMA Health Outcomes Work Group (HOWG)

- FDA- HRQL guidance in conjunction with Laurie Burke at FDA:
  - Identification of 5 contentious HRQL issues
  - development of recommendations for key issues at industry conference sponsored by HOWG in March 99
  - collaboration with other groups working with FDA (ERIQA)

ISPOR (www.ispor.org)

- Development of a Consensus Document as a Supporting Document for FDA and Other Health Regulatory Authorities on HRQL guidances
- Comments received to be reviewed by the ISPOR Ad Hoc Quality of Life Guidance Committee and included, if appropriate, in the final working paper

Conclusion: Perspectives for the future

- Promote collaboration between all initiatives

- → to improve consistency between regulatory agencies (EMEA vs FDA)