

# **Health-related Quality of Life and Regulatory Issues**

## **The ERIQA Project and other initiatives**

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

# Background

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

- 1 Vienna, November 4-5th, 1997:  
*Mapi Research Institute*
  
- 1 Milano, October 1997:  
*Mario Negri Institute, GlaxoWellcome*

## Background (cont'd)

- 1 Need to rationalise the field of HRQL and to make it credible as a criterion of evaluation to the regulatory authorities.
  - 1 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

## 1 EMEA

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

## 1 Country-Specific Agencies

- Registration
- Pricing, Reimbursement
- Promotion

# Contributors

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# Sponsors

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

# Organisation: 2 Phases

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 Decide target areas and draft guidance : COPD/asthma, cancer as pilot areas	3. Draft guidance on COPD/asthma and cancer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

# Ph.I - St. 1: Report

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

# Ph.I - St. 1: Conclusions

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

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

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

# Ph.I - St. 2: Reference Document

- 1 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 specific pathologies or areas.
- 1 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

1 pilots with two indications

– cancer

– COPD - asthma

1 body of evidence important



# Phase II

1 development of consensus guidance for specific diseases

1 using:

- analysis grid developed in phase I
- consensus meetings

# Other initiatives

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

# FDA suggestions to industry

- 1 Determine CLAIMS before study initiation.
- 1 Ensure that
  - the INSTRUMENT,
  - its DEVELOPMENT AND VALIDATION history,
  - the PROTOCOL, and
  - the DATA ANALYSIS PLAN are adequately developed to support the desired claims.
- 1 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)

- 1 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 (ERIQAGroup)

# ISPOR ([www.ispor.org](http://www.ispor.org))

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

# Perspectives for the future

- 1 Promote collaboration between all initiatives
  - è to improve consistency between regulatory agencies (EMEA vs FDA)