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.

 création 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
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<tr>
<td>Neil Aaronson</td>
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<td>Harry Burns</td>
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<td>Margaret Rothman</td>
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<td>Suzanne Wait</td>
<td>Novartis, Switzerland</td>
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Sponsors

1. Mapi Research Institute, Lyon, France
2. Astra Hässle, Mölndal, Sweden
3. Bayer, Leverkusen, Germany
4. GlaxoWellcome, Verona, Italy
5. Janssen Research Foundation, NJ, USA
6. Rhône Poulenc Rorer, Antony, France
7. Schering AG, Berlin, Germany
## Organisation: 2 Phases

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Ph.I - St. 1: Report

1. review and synthesis of existing guidance
2. special focus on the EMEA documents
Ph.I - St. 1: Conclusions

1. No documents directly focused on HRQL measures

2. A few explicit recommendations (1 generic + 11 conditions/drugs)

3. Most of the times, generic recommendations

4. 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

2. No comment on generic/specific issue

3. Only few warnings on "cultural problems"

4. 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

Development of consensus guidance for specific diseases using:
- analysis grid developed in phase I
- consensus meetings
Other initiatives

1. Food and Drug Administration (FDA)
2. Health Outcomes Work Group of Pharmaceutical Research and Manufacturers of America (PhRMA HOWG)
3. International Society for Pharmacoeconomics and Outcomes Research (ISPOR)
FDA suggestions to industry

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

1. Promote collaboration between all initiatives

2. To improve consistency between regulatory agencies (EMEA vs FDA)