



Formation sur la Qualité de Vie liée à la Santé dans les Essais Cliniques

Educational Programme on Health-Related Quality of Life (HRQL) in Clinical Trials

Vendredi 24 janvier 2003

Paris

Dr Olivier Chassany

Chargé de Mission, Délégation Régionale A la Recherche Clinique, Hôpital Saint Louis, Paris

Dr Juliette Longin

Chef de Projets, Mapi Research Institute, Lyon

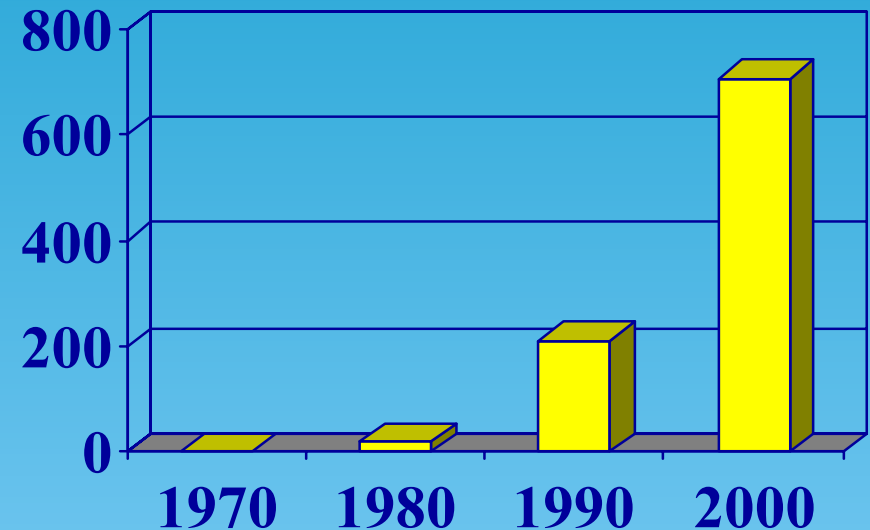
Evolution of HRQL assessment in Clinical Trials since 1966

★ Literature search (Medline)

“Quality of Life” Matched with
“Clinical Trials”:

Results: number of references

- ★ 1966 to 1970: 0
- ★ 1966 to 1980: 20
- ★ 1966 to 1990: 210
- ★ 1966 to 2000: 708



Considerable increase
of HRQL assessment
in Clinical Trials

Cochrane Health-Related Quality of Life Methods Group



Objective:

- To advise Cochrane reviewers about when and how to incorporate HRQL data into systematic reviews of health care interventions
- Convenors: Catherine Acquadro, MD
 - Prof. Dick Joyce, PhD, FBPoS
 - Prof. Donald L. Patrick, MSPH, PhD
- Coordinator: Lucile Lapalus (llapalus@mapi.fr)
- Active members : 37 from 12 countries

Objectives

- **To help reviewers of clinical trials to acquire the skills needed to assess HRQL outcomes included in regulatory files and publications**
- **To facilitate decisions made by Health Authorities and health care providers**

Presentation of the Workmats (1/2)

■ **Workmats :**

- ◆ large worksheets
- ◆ contain concise information: background
- ◆ present various assignments

■ **Workbook :**

- ◆ reference source
- ◆ additional information on HRQL

Presentation of the Workmats (2/2)

- **Interactive learning method**

- **Participants**
 - ◆ **Small group discussions** and interactions
 - ◆ To understand the new information
 - ◆ To complete the assignments through group discussions (writing material)
 - ◆ Group answers have to be discussed by all the groups to reach a consensus

Content

Workmats	Content
1	How do disease and treatment affect Health-Related Quality of Life (HRQL)?
2	Deciding which domains to include in a HRQL instrument
3	Developing a HRQL questionnaire 1 st step: Development of items and item reduction
4	Choosing an appropriate existing HRQL measure
5	Analysis of HRQL data
6	Presentation and interpretation of HRQL outcomes included in clinical trials?

WORKMAT 1

How do disease and treatment affect HRQL?

*Educational Programme on Health-Related Quality of Life (HRQL)
for Reviewers of Clinical Trials*

WORKMAT 1 : (9h30 – 10h00)

■ Learning objectives

- ◆ **To identify the impact of health conditions and treatment on HRQL**
- ◆ **To distinguish the different ways diseases and treatment can affect HRQL**
- ◆ **To create an awareness that treatments can affect HRQL**

*Educational Programme on Health-Related Quality of Life (HRQL)
for Reviewers of Clinical Trials*

WORKMAT 1

■ Learning points

- ◆ Diseases and treatments can affect a person's quality of life in different ways
- ◆ HRQL is multi-dimensional and subjective

WORKMAT 2

Deciding which domains to include in HRQL instrument

*Educational Programme on Health-Related Quality of Life (HRQL)
for Reviewers of Clinical Trials*

WORKMAT 2 : (10h15 – 10h45)

■ Learning objective

- ◆ To define the relevant HRQL domains depending on the conditions studied

WORKMAT 2

■ Learning points

- ◆ The relative burden of disease and treatment on population can be measured through HRQL domains
- ◆ At a minimum, HRQL consists of physical, psychological, and social domains
- ◆ The patient plays an important part in the questionnaire development

WORKMAT 3

Developing a HRQL questionnaire

**1st step: Development of items and item
reduction**

WORKMAT 3 : (11h00 – 11h45)

■ Learning objective

- ◆ To describe the process of HRQL instrument development

WORKMAT 3

■ Learning points

- ◆ Instrument development is a scientific rigorous process
- ◆ No single *right* way to develop an instrument although best practices available for steps in the process
- ◆ The instrument should have empirical evidence of validity

WORKMAT 4

Choosing an appropriate existing HRQL measure

*Educational Programme on Health-Related Quality of Life (HRQL)
for Reviewers of Clinical Trials*

WORKMAT 4 : (13h30 – 14h15)

■ **Learning objectives**

- ◆ **To explore the process for selecting appropriate health status instrument for use in specific clinical trial scenario**
- ◆ **To examine the trade-offs in the selection process**
- ◆ **To review the criteria necessary for appropriately evaluating an HRQL instrument**
- ◆ **To identify and evaluate established questionnaires for use in specific patient group**

WORKMAT 4

■ Learning points

- ◆ The first step is to ask yourself Key Questions
- ◆ The choice of domains and the selection of an HRQL instrument is influenced by severity and nature of the disease and the expected benefits and side effects of treatment

WORKMAT 5

Evaluating techniques for HRQL data analysis

*Educational Programme on Health-Related Quality of Life (HRQL)
for Reviewers of Clinical Trials*

WORKMAT 5 : (14h30 – 15h15)

■ Learning objectives

- ◆ To identify the issues and potential problems in designing a statistical analysis plan for HRQL data
- ◆ To understand the different methods of treating missing data
- ◆ To gain the knowledge and skills needed to analyse differences in HRQL outcomes between two or more treatments

WORKMAT 5

■ Learning points

- ◆ To pre-specify hypotheses and to establish a rigorous analysis plan with a special focus on multiple test and missing data
- ◆ The type of missing data should be specified (missing items / missing questionnaires)

WORKMAT 6

**Presentation and interpretation of HRQL
outcomes included in clinical trials**

WORKMAT 6 : (15h30 – 16h30)

■ **Learning objective**

- ◆ **To critically evaluate published literature describing HRQL surveys**
- ◆ **To interpret HRQL data that are reported in the published literature**

WORKMAT 6

■ Learning points

- ◆ There are several ways to interpret, all have advantages and disadvantages : Effect Size (ES), Minimal Clinically Important Difference (MCID), Number of Patients to Treat (NNT)
- ◆ As the experience of interpreting HRQL outcomes is minor, raw HRQL scores may be difficult to interpret at the moment
- ◆ Attempts to interpret in different ways HRQL scores in a file are recommended
- ◆ The evaluating techniques for HRQL data analysis are still in development but are valuable techniques