



# **Educational Programme on Patient-Reported Outcomes (PRO) for Reviewers of Clinical Trials**

## **Pilot Meeting**

*Thursday 23<sup>rd</sup> May, 2002*

**Food and Drug Administration, Rockville, USA**

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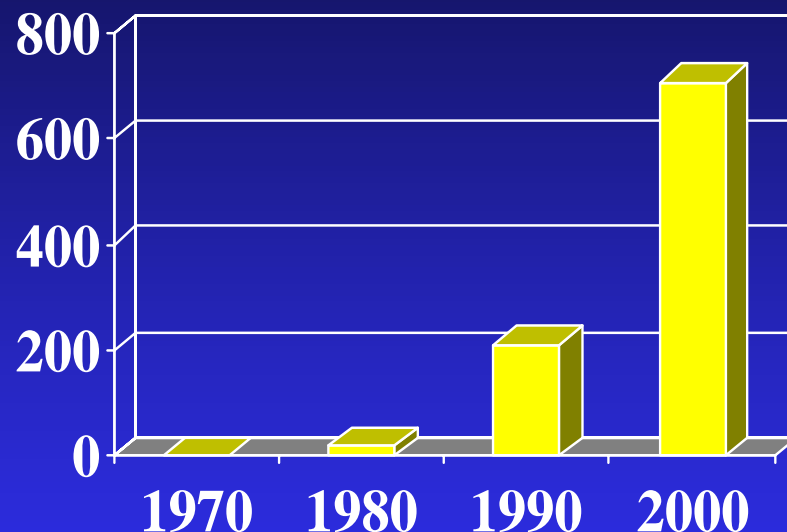
# Evolution of HRQL assessment in Clinical Trials since 1966

## ★ 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

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# 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

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➤ [www.cochrane.co.uk](http://www.cochrane.co.uk) or [www.cochrane.org](http://www.cochrane.org)

# Objectives of the Workmats

- To help reviewers of clinical trials to acquire the skills needed to assess PRO included in regulatory files and publications
- To facilitate decisions made by health authorities and health care providers

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# Presentation of the Workmats (1/2)

## ■ **Workmats :**

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

## ■ **Workbook :**

- ◆ reference source
- ◆ additional information on PRO

# 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 impact patients' lives— from the patients' perspective
2	Deciding which items and domains to include in an instrument designed to measure the impact of disease and treatment from the patient's perspective
3	Developing a questionnaire
4	Choosing an appropriate existing measure
5	Analysis of PRO data : example of HRQL data
6	Presentation and interpretation of PRO included in clinical trials : example of HRQL

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# WORKMAT 1

**How do disease and treatment impact patients' lives – from the patients' perspective ?**

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# WORKMAT 1 : (9:30 – 10:15)

## ■ Learning objectives

- ◆ To identify the impact of health conditions and treatment from the patient's perspective
- ◆ To distinguish the different ways diseases and treatment can affect patient's life
- ◆ To create an awareness that treatments can affect patient's life

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# WORKMAT 1

## ■ Learning points

- ◆ Diseases and treatments can affect patient's life in different ways
- ◆ HRQL, as a PRO, is multi-dimensional and subjective

# WORKMAT 2

**Deciding which items and domains to include  
in an instrument designed to measure the  
impact of disease and treatment from the  
patient's perspective**

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# WORKMAT 2 : (10:45 – 11:15)

- Learning objective

- ◆ To define the relevant domains and items 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

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# WORKMAT 3

## Developing a questionnaire

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# WORKMAT 3 : (11:45 – 12:15)

- Learning objective

- ◆ To describe the process of PRO 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



**Lunch**  
**(12:45 – 13:45)**

# WORKMAT 4

## Choosing an appropriate existing measure

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# WORKMAT 4 : (13:45 – 14:15)

## ■ Learning objectives

- ◆ To explore the process for selecting appropriate PRO 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 a PRO instrument
- ◆ To identify and evaluate established questionnaires for use in specific patient group

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# WORKMAT 4

## ■ Learning points

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

# WORKMAT 5

**Analysis of PRO data : example of  
HRQL data**

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# WORKMAT 5 : (14:45 – 15:30)

## ■ Learning objectives

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

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# 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 PRO  
included in clinical trials: example of  
HRQL**



# WORKMAT 6 : (16:00 - 16:30)

## ■ 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

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# Conclusion