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Patient-Centered Outcome Measures

Patient-Centered Outcome Measures (PCOM) aim to place patients, their family and carers at the heart of decisions concerning the health assessment

Types of clinical outcome assessment

- ▶ Patient-reported outcomes
- ▶ Clinician-reported outcomes
- ▶ Observer-reported outcomes
- ▶ Performance reported outcomes
- ▶ Biomarkers

Conclusions I: Clear Definition

In the development of PCOM, the following should be clearly defined:

- ▶ What to measure, based on qualitative research
- ▶ Why we measure, for what purpose
- ▶ Where the measuring takes place
- ▶ Who is qualified to measure
- ▶ How to measure

Conclusions III: Common Data Elements

Use common data elements (CDE) when possible:

- ▶ Use validated / existing CDE if possible
- ▶ Use validated instruments when possible
- ▶ Plan ahead to update CDE
- ▶ Do not work alone; work with others
- ▶ Engage the full range of needed expertise
- ▶ Gain support of the user community

Conclusions V: Support to be provided

Support to be provided through funding and collaboration

- ▶ Non-competitive area: appropriate PCOM will benefit all categories of stakeholders
- ▶ Promote good practices by training modules, tool kits and publications
- ▶ Consider a funding pot to adapt / establish PCOM: public / private funding process

Aims of the Patient-Centered Outcome Measures Task Force

- ▶ Identify what can be done to support the development of PCOM for rare diseases in order to improve the feasibility and quality of trials
- ▶ Identify what can be done to provide relevant data for the patient community and other decision makers

Conclusions II: Look for validated instruments

Several databases and guidelines are available to search and evaluate existing instruments

Databases

- ▶ COMET
- ▶ COSMIN
- ▶ PROMIS
- ▶ PROQOLID
- ▶ ...

Guidelines

- ▶ COSMIN
- ▶ FDA
- ▶ EMA
- ▶ ISPOR
- ▶ ...

Conclusions IV: Tools

- ▶ Adapt existing tools rather than developing new ones
- ▶ Ensure content validity through qualitative research
- ▶ If new PCOM has to be developed:
 - Start early in the R&D process
 - Share the workload with other partners if possible
 - Awareness of best practices is necessary
 - Publication of new PCOM is highly recommended

