Treatment-Related Health Outcomes: Adaptation to Lebanese Patients

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Introduction

- Growing movement to focus the evaluation of health care on the assessment of the end results, or outcomes.
- Outcomes are associated with medical care delivery systems and specific medical interventions.
- Objective: maximize the net health benefit derived from the use of finite health care resources.
- Serious lack of information all over the world.
Types of Health Outcomes

- Five D’s: death, disease, disability, discomfort, and dissatisfaction.

- More comprehensive conceptual framework model (the ECHO model): economic, clinical, and humanistic outcomes.
  - Clinical: medical events occurring as a result of disease or treatment (clinical practice & clinical pharmacy)
  - Economic: direct, indirect and intangible costs compared with the consequences of a medical intervention (health economics & pharmacoeconomics)
  - Humanistic: patient satisfaction, self-assessed function and well-being → Health related quality of life (QOL)
### Phases of Clinical Drug Development

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<th>Phase</th>
<th>Disadvantage</th>
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<td>Phase 0: preclinical testing; laboratory and animals studies; assess safety and biologic activity</td>
<td>Selected Healthy/Diseased population. Ideal setting</td>
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<td>Phase I: 20-80 healthy volunteers; determine safety and dosage range</td>
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<td>Phase II: 100-300 patient volunteers; evaluate efficacy; look for side effects</td>
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<td>Phase III: 1000-3000 patient volunteers; confirm efficacy, monitor adverse reactions from longer term use</td>
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<td>Phase IV: Additional post-marketing testing required (Pharmaco-epidemiology)</td>
<td>Extended population Real Life setting</td>
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Usefulness of Pharmacoepidemiology

- When monitoring long term or rare side effects
- Effect on populations not introduced in clinical trials: pregnant women, elderly, children...
- Observational long term beneficial effects of drugs (support practice guidelines)
- Possibility of assessing Health Outcomes (5Ds & ECHO model)
Pharmacoeconomic Value

Balance of economic, humanistic and clinical outcomes:

- **Economic**: Cost compared with the consequences of treatment alternatives
- **Humanistic**: Consequences of disease or treatment on patient functional status or QOL along several dimensions (physical, social, general health and well-being, and life satisfaction)
- **Clinical**: Medical events that occur as a result of disease or treatment (safety and efficacy endpoints)
Pharmacoeconomic Methodologies

- Cost-of-illness evaluation
- Cost minimization analysis
- Cost-benefit analysis
- Cost-effectiveness analysis
- Cost-utility analysis → Quality of life assessment
Quality of Life Assessment (QOL)

- Physical, social, and emotional aspects of patients’ well-being that are relevant and important to the patient
- Examines drug effects in areas not covered by laboratory or physiologic measurements
- Outcome unit = QOL score, patient preferences, patient satisfaction...
Definition of QOL

- No consensus on its definition
- “Multidimensional concept referring to a person’s total well-being, including his or her psychological, social and physical health status”, Schron and Shumaker.
- “The value assigned to duration of life as modified by the impairments, functional states, perceptions, and social opportunities that are influenced by disease, injury, treatment or policy”, Patrick and Erickson.
- Commonly measured dimensions:
  - Physical health and functioning
  - Psychic health and functioning
  - Social function and role
  - Perception of overall well-being
QOL Measure: Instrument Taxonomy

- Generic (general) instruments: applicable to all conditions, interventions and populations
  - Health profile (e.g. SF36, SIP, HUI-Mark III)
  - Measures based on patient’s preference (economic: e.g. QALY)

- Specific instruments
  - Disease (e.g. asthma, diabetes...)
  - Population (e.g. elderly, children...)
  - Function (e.g. sexual, social...)
  - Condition or problem (e.g. pain, personal satisfaction...)
QOL Measure: Properties

- QOL increasingly seen as a result of a treatment (outcome): subjective impact
- Problem: subjective measure
- *Physiological measurement can change because of an intervention without change of QOL, and vice versa.*
- Psychometry: Measurement of psychological constructs such as QOL.
- Developed instruments must be tested → confidence in measurement
Psychometric Properties

- Conceptual and measurement models
- Internal validity (measures what it is supposed to measure)
- Reproducibility (reliability/external validity)
- Responsiveness to change
- Interpretability (cost to respondent and administration)
- Alternatives forms
- Cultural and linguistic adaptation
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