Introduction to Outcomes Research

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Introduction

- Outcomes are the Pharmaco- part of Pharmacoeconomics.
- The manner of assessing outcomes drives PE analysis.
Learning Objectives

- Define and contrast outcomes and outcome surrogates with examples
- Discuss different classification systems for characterizing health outcomes
- Contrast the quality of outcomes collected from different sources and levels of evidence
- List questions to consider in choosing outcomes data for Pharmacoeconomics studies
What are Health Outcomes?

Defined as “the end result of antecedent health care”
Treatment
Treatment

Success

- Good Outcome
  - Survival,
  - BP at goal,
  - Pain control,
  - Ability to walk a distance, Satisfaction with treatment

Failure

- Bad Outcome
  - Death,
  - High BP,
  - Uncontrolled pain,
  - Inability to walk a distance, Dissatisfaction with treatment
Using Outcomes to Measure Effectiveness
# Effectiveness Measures

| Outcomes | • Events avoided (MI, seizures, blindness)  
|          | • Changes in outcome rates and frequencies  
|          | • Days free of symptoms or events  
|          | • Improvements in quality of life and satisfaction  
|          | • Life years saved or gained  |
| Clinical Indicators | • Improvements (% reduction, unit changes) in some lab value (FEV1, BP, LDL, Hb/Hct, HbA1c)  
|          | • Patients successfully treated or reaching treatment goal  |
| Monetary values | • Dollars  |
How are Outcomes Characterized?

1. According to outcomes measured
2. According to data used
1. Characterizing Outcomes According to the Outcomes Measured

Outcomes measured influence many aspects of the assessment
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- **FOCUS**
- **Usefulness**
- **Validity**
- **Feasibility**

Outcomes measured influence many aspects of the assessment.
1. Characterizing Outcomes According to the Outcomes Measured

Focus

VALIDITY

Usefulness

Feasibility

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Outcomes measured influence many aspects of the assessment

FEASIBILITY
Outcomes Measured: The Five Ds

Dissatisfaction
Discomfort
Disability
Disease
Death

The Five Ds

Objective

Provider Focused

Patient Focused

Death

Disease

Disability

Discomfort

Dissatisfaction

Subjective

(Patient reported)

The Five Ds

- Death
- Disease
- Disability
- Discomfort
- Dissatisfaction

(Patient reported)

The Five Ds

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(Patient reported)

The Five Ds

Disability

Death | Disease | Disability | Discomfort | Dissatisfaction

(Patient reported)

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Outcomes Measured: ECHO Model

- Outcomes fall into three categories: Economic, Clinical, and Humanistic.

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- ECHO distinguishes between
  - outcomes and
  - intermediaries.

Non-Outcome Measures

- Surrogates of the end result of health care
  - Intermediaries (ECHO model)
  - Surrogate Outcomes
  - Intermediate Outcomes
Surrogates for Desired Outcomes

- Desired Outcome
- Undesired Outcome

Perfect Surrogate
Surrogates for Desired Outcomes

Acceptable Surrogate

Desired Outcome

Undesired Outcome
Surrogates for Desired Outcomes

- Desired Outcome
- Undesired Outcome
- Unacceptable Surrogate
Acceptability of Surrogates is Treatment Specific

Obesity → Dexfenfluramine → Lowered Weight → Valvular Heart Disease
2. Characterizing Outcomes According to the Data Used

- The better the outcomes data used, the more valid the outcome assessment.
- Quality of outcomes data typically assessed by:
  - Internal or external sources
  - Hierarchies of evidence
Internal or External Data Sources?

- **Internal** -- collected from your own patient population or organization
  - medical chart, pharmacy computer systems, insurance claims databases, expert opinion
  - Patient-reported outcomes from surveys

- **External** -- Collected from external sources
  - published studies (clinical trials, epidemiologic studies, meta-analyses), Web sites, registries
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Hierarchies of Evidence

- Randomized Control Trials
  - Typical, Supplemented, Pragmatic, Meta-analyses
- Nonrandomized intervention studies
- Registries
- Administrative data for observational studies
- Health Surveys
- Expert Opinion
Hierarchies of Evidence

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- **Expert Opinion**
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Questions to ask in selecting outcomes data
What level of evidence is necessary to address the question being posed

- For making national public policy or formulary decisions?
- For publication?
- For poster presentation?
- For managerial problem solving?

Increasing Level of Evidence Needed
What outcomes measures are most relevant?

- Understand the process first by mapping out each step in the therapeutic process.
  
  Symptoms
  Diagnosis
  Treatment
  Follow-up
  Re-treatment
What is feasible?

- Are outcomes data available?
- Is the data easy to collect and analyze?
- How much will data collection and analysis cost?
Should Outcomes be Discounted?

Costs are discounted.

• A dollar now is worth more than a dollar in the future

Why not outcomes?

• Good health now is worth more than good health in the future

How Should Outcomes be Presented?

- Describe all relevant outcomes in sufficient detail. Justify the exclusion of any major outcomes.
- State the sources of data.
- Describe how outcomes were collected.
- Explain any strategies used to validate data quality.
Recommendations for Using Outcomes

- Know how outcomes data is collected.
- Provide citations and descriptions of all data collection.
- Describe how any intermediate outcomes or surrogates are related to desired final outcomes.
- Explain limitations associated with the data.
  - Characteristics of the population
  - Limitations of research design
  - Assumptions associated with data
Conclusion

- The quality of pharmacoeconomic studies relies on outcomes data used.
- Transparency of the sources and methods used are essential for decision makers to use the research.