The use of routinely collected PROMs data in economic analyses

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APERSU Webinar

**Outline**

- What PROMs are
- What PROMs are not
- Routine collection of PROMs
- Using routinely collected PROMs for measuring outcomes
- Using routinely collected PROMs for economic evaluation
- Examples
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• Using routinely collected PROMs for economic evaluation
• Examples

What PROMs are

• PROMs
  - Patient Reported Outcome Measures
  - Subjective assessment of a person’s health or wellbeing
  - Used to measure
    • Quality of life
    • Capabilities
    • Functioning
    • Symptoms
    • Symptom burden
    • Physical health
    • Mental health
    • ...
    • ...
**What PROMs are**

- PROMs are a set of tools that help us understand how health care impacts on the health and wellbeing of patients

- Generic v Condition specific

- They have many uses:
  - Economic evaluation
  - Clinical outcomes
  - Policy evaluation

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**What PROMs are**

- A set of tools that help us understand how health care impacts on the health and wellbeing of patients

- Generic v condition specific

- Preference v non-preference based

<table>
<thead>
<tr>
<th></th>
<th>With Preferences</th>
<th>Without preferences</th>
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</thead>
<tbody>
<tr>
<td>Generic</td>
<td>EQ-5D</td>
<td>KIDSCREEN</td>
</tr>
<tr>
<td>Specific</td>
<td>MSIS-8D-P</td>
<td>HAD</td>
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</tbody>
</table>

- They have many uses:
  - Economic evaluation
  - Clinical outcomes
  - Policy setting
What PROMs are

- PROMs in health economics
  - A measure of benefit
    - Different approaches to doing so (eg functioning, capability)
  - Preference based
    - We have knowledge about which states of being are preferred in relation to one another
    - Some can be used to estimate utility (those based on measures of health)
    - Others have rankings but are not measures of utility (those based on capability)
  - Focus is on those that can be used to estimate utility
What PROMs are

• Ranking
  – Describes preferences for different states
    • Combined set of responses define a set of unique health states
    • It is these unique health states that are ranked to determine preferences
    • Individuals may rank states differently (important later!)
    • Population preferences are a set of aggregated individual preferences (also important later!)
    • Must be transitive: \( A > B > C \)
    • Must be complete: Cannot leave some states unranked
  – Preferences typically determined by the ‘general’ population
    • Different methods exist, but aim is to understand rankings between states

• Utility
  – A measure of welfare, or benefit
  – Formally: The level of satisfaction a consumer gets from consuming a good
  – If :
    a) We know the complete set of possible health states
    b) Have a ranking for each of those health states
    c) Have a value for each of those health states (eg in terms of time)

Then we can estimate utility.
What PROMs are

- PROMs used in health economics
  - **EQ-5D-X / SF-X / HUI X**
    - Focus on health via functioning
    - Suitable for estimating utility values
    - Multiple versions of each
    - Value sets (ie rankings) across different populations
  - **ICECAP-X**
    - Focus on health via capabilities
    - Not suitable for estimating utilities (maybe)
    - Rankings exist for some, in smaller number of populations
    - Increasing use reflects perceived limitations of functioning based measures
What PROMs are

• EQ-5D-X
  – Different versions of the EQ-5D
    • 3-level, 5-level, Youth
    • Measure five domains of health
      – Mobility
      – Self-care
      – Usual activities
      – Pain
      – Anxiety/Depression
    • 3L = 243 unique states plus dead
    • 5L = 3,125 unique states plus dead

What PROMs are not

• They are not universal
  – No PROM can cover everything

• They are not substitutes for one another
  – one PROM is not the same as another

• They are not ‘sufficient’
  – They are part of the evidence base

• They are not substitutes for objective or clinical outcomes
  – A treatment that patients like is not the same as a good treatment
  – Must be used alongside other evidence
Outline

• Background
• What PROMs are
• What PROMs are not
• Routine collection of PROMs
• Using routinely collected PROMs for evaluation
• More research needed

Routine collection of PROMs

• PROMs have long been used in research contexts
  – Typically as a secondary measure or outcome
  – Increasingly recognised as a suitable primary outcome measure
    • Often in studies where survival not a key endpoint
    • eg in mental health studies
    • MIR trial used Becks Depression Inventory
  – EQ-5D has also been used as a primary outcome measure
    • Less common
    • What is clinically meaningful difference?
    • “Laser in Glaucoma and Ocular Hypertension (LiGHT) trial. A multicentre, randomised controlled trial: design and methodology”
Routine collection of PROMs

• Routine collection of PROMs data less common

• Some PROMs collected more than others
  – Condition specific rather than generic
  – Non-preference rather than preference based

• But! Routine collection of generic, preference based PROMs is starting to happen
  – UK NHS
    • EQ-5D-3L
    • CLINICAL AREAS
  – Alberta
    • EQ-5D-5L
    • Cancer Control Alberta, Community rehab, PCNs
    • AHS - aims to have more comprehensive coverage as part of EHR

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Evaluations

• Collect all the PROMs data you want
  – Easy
  – Quick
• What matters is what you do with it

Routine collection of PROMs

• Collecting EQ-5D or other preference based PROM does not mean you can use these as measures of individual outcome
• Remember this:
  • Individuals may rank states differently (important later!)
  • Population preferences are a set of aggregated individual preferences (also important later!)
  – Imagine a patient being followed over time with two PROMs
    • EQ-5D-3L
    • PHQ-9 (for depression)
Routine collection of PROMs

• Imagine a patient being followed over time with two PROMs
  – EQ-5D-3L
  – PHQ-9 (for depression)

• PHQ-9 is scored by adding responses together
  – Higher scores mean worse health
  – Over time possible to track changes in health and infer better/worse

• EQ-5D-3L gives 243 possible states
  – Scoring 1 on all dimensions is written as 11111
  – Scoring 2 on all dimensions is written as 22222
  – But! These are not scores, they are descriptions

• Does the patient feel better off in T1 or T2?
• Utility score is a societal preference for states – ie the utility society would get from consuming resources to achieve that state

<table>
<thead>
<tr>
<th>Domain</th>
<th>Time 1</th>
<th>Time 2</th>
</tr>
</thead>
<tbody>
<tr>
<td>Mobility</td>
<td>1</td>
<td>1</td>
</tr>
<tr>
<td>Self-care</td>
<td>2</td>
<td>1</td>
</tr>
<tr>
<td>Usual Activities</td>
<td>2</td>
<td>2</td>
</tr>
<tr>
<td>Pain</td>
<td>2</td>
<td>2</td>
</tr>
<tr>
<td>Anxiety/Depression</td>
<td>2</td>
<td>3</td>
</tr>
<tr>
<td>Utility Score</td>
<td>0.585</td>
<td>0.157</td>
</tr>
</tbody>
</table>
### Evaluations

- **UK NHS (PROMs) programme**
  - Captured PROMs for groin-hernia surgery, varicose vein surgery, and knee and hip replacement
  - Generic (EQ-5D-3L) and condition specific (e.g., Oxford Hip Score, Aberdeen Varicose Vein Questionnaire)
  - Data collected between 2009 and present
  - Data collection on G-H and VV surgery stopped in 2017
  - Data still collected on hip and knee replacement

### Evaluations: Example

- **Using PROMs data in research**
  - Routinely collected PROMs data
    - Baseline measurement at day of surgery
    - Follow-up at least 6-months post surgery
    - EQ-5D-3L and Oxford Hip/Knee Score
  - Routinely collected resource use data (Hospital Episode Statistics) for inpatient care
  - Did not try to infer causality
- Post-op EQ-5D index scores higher than pre-op for index surgery
- Average hip cost = £5,522
- Average knee cost = £6,053
- Preoperative and postoperative quality of life were higher for patients
  with high OHS/OKS scores (higher scores are better)

Could not evaluate cost-effectiveness of joint replacement
- No data on the costs and quality of life for patients without joint replacement
- This is a key shortcoming of using routinely collected data – identification of control groups
  - We only have data for those who receive treatment
  - Patients in the UK NHS only routinely complete PROMs if they are having surgery
Evaluations: Challenges

• Administrative data is “…information collected primarily for administrative (not research) purposes.”
  – No controls or randomization
  – Limited by what is being collected already
  – Legal and ethical barriers to access
  – Integrating disparate sources of data
  – Negotiating across jurisdictions
    • Departments, Provinces, International

Economic Evaluations: Challenges

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### Economic Evaluations: Challenges

- **No controls or randomization**
  - Evaluation of an intervention requires a counter-factual – that is, what would happen without intervention
  - UK NHS PROMs programme did not have this, so couldn’t be evaluated fully
  - RCTs are ideal – one group get Rx, the other not
  - What do we do when we can’t randomize?
    - Artificial counter-factual

### Economic Evaluations: Challenges

- **Creating counterfactuals**
  - Difference-in-Difference
  - Matching (including propensity score matching)
  - Instrumental variables
  - Before and after studies (Interrupted time series)
  - Regression discontinuity
### Economic Evaluations: Challenges

- Creating counterfactuals
  - Difference-in-Difference
  - Matching (including propensity score matching)
  - Instrumental variables
  - **Before and after studies (Interrupted time series)**
  - Regression discontinuity
  - **Modelling**

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- There are many established methods from economics and epidemiology for doing causal inference on admin data
  - But all have drawbacks and must be chosen with care!
- A key difference between econ and epi are measures of outcome
- Epi is interested in effect of exposure on some outcome like survival
- Economics is interested in effect of exposure on:
  - Resource use (easiest)
  - Costs (harder)
  - Health outcomes eg quality of life (hardest)
### Economic Evaluations: Examples

- Examples of ongoing research
  - Evaluation of endovascular therapy (modelling)
  - Evaluation of an online wellness program (interrupted time series)
  - Evaluation of a community support intervention (difference-in-difference)

### Policy Question

What is the health system impact of alternative models of providing endovascular therapy in Alberta while taking into consideration the following:

- patient and clinical factors;
- distance, time, and other viability thresholds for endovascular therapy; and
- capacity within existing system resources to provide coordinated care and how they are impacted, including what new resources would be required?
Technical Approach

Developed a two-stage model to assess the cost:

1. Decision tree model from stroke onset to 90 days
   - Outcome 1: medical costs
   - Outcome 2: probability of treatments
     - A patient can receive either EVT, or EVT plus tPA or tPA only

2. Markov model from 90 days to life year
   - Outcome: lifetime costs for the cohort of mRS 0 – 2 versus the cohort of mRS 3 – 5 at 90 days
   - Better outcome (i.e. mRS 0 – 2) is associated with fewer resources used
Results: Distribution of optimal strategies

The Figure shows optimal transportation methods in each DAs.

The strategies that generated maximum good outcomes (mRS 0-2)

Note that optimal strategy is unique in each DA, but is a combination of transportation methods at higher level (e.g. health zone).

Economic Evaluations: Examples

- Examples of ongoing research
  - Evaluation of endovascular therapy (modelling)
  - Evaluation of an online wellness program (interrupted time series)
  - Evaluation of a community support intervention (difference-in-difference)
Economic Evaluations: Alberta

- Economic evaluations require data on costs and benefits
- Administrative data primarily contains information on activity through contacts with the public part of the health system
  - Sometimes we have cost data (e.g. billing data, fee schedules)
  - Much of the time
- Routine collection of PROMs in Alberta is new
  - Few clinical areas (e.g. cancer, community rehab)
  - Within these, not 100% coverage yet
  - Needs historical data to help with controls