Patient reported outcome measures in clinical practice

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Objective

• To provide healthcare professionals (HCPs) with an introduction to patient reported outcomes measures (PROMs), and how they are important in developing a patient-centred approach to care
Upon completion of this module, the participant will be able to:

1. Demonstrate an increase in, or affirmation of, their knowledge of patient reported outcome (PRO) research in terms of:
   - Understanding the meaning and purpose of PRO research
   - Recognising the different types of measures used in PRO research

2. Identify and utilise PROMs for the needs of the patient and for their clinical setting

3. Understand how to synthesise findings from PRO research studies
Content overview

- The principles of PRO research
- The applications of PROMs in clinical practice
- PRO clinical case study
- Conclusions
- Self-assessment quiz
The principles of PRO research
What is PRO research?

• PRO research can be defined as:
  “Any report on the status of a patient’s health condition that comes directly from the patient, without interpretation of the response by a clinician or anybody else”\(^1,2\)

• PRO research takes into consideration, and stresses the importance of, research that is informed by the perspectives, interests and values of the patient,\(^2\) and are used to evaluate the effect of treatment on how a patient feels or functions\(^3\)

• PROs are used when measuring a concept that is best known by the patient or measured from the perspective of the patient\(^4\)
  - Other than reporting of outcomes by the patients themselves, a **proxy-reported outcome** is a measurement based on a report by someone other than the patient, reporting as if he or she is the patient\(^5\)
  - An observer (e.g. caregiver) may give a measure based on their opinion, referred to as an **observer-reported outcome**\(^5\)

• PROMs are typically based on questionnaires, referred to as PRO instruments, that are based on health and quality of life\(^6\)

• PROs are often used to evaluate change in health status over time with PRO instruments administered at baseline and during or after treatment, or when a certain milestone has been reached\(^7\)

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What instruments are used to assess PROs?

- PRO instruments collect data across several areas of health, including physical, psychological and social functioning, and are generally classed as either ‘generic’ or ‘disease-specific’

**Generic PRO instruments**

- **Overall state of the condition**
  - e.g. angina

- **Overall feelings about a condition or treatment**
  - e.g. worry about the condition deteriorating

- **General disease concepts**
  - e.g. physical functioning

**Disease-specific PRO instruments**

- **Specific disease concepts**
  - e.g. frequency/severity of symptoms

- **Treatment side effects**
  - e.g. severity of adverse events

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Advantages and disadvantages of PRO instruments

**Generic PRO instruments**¹

**Advantages**
- ✓ Suitable for the general public and for comparisons across populations
- ✓ Suitable for comparisons with disparate conditions/disease groups
- ✓ Captures commonly experienced health domains

**Disadvantages**
- ✗ Content may be redundant for certain conditions/illnesses
- ✗ Not sensitive to detecting disease-specific illnesses

**Disease-specific PRO instruments**¹

**Advantages**
- ✓ Specific for a given group (disease/population)
- ✓ Sensitive enough to detect clinically significant changes
- ✓ Captures clinically-relevant information that is applicable to a specific target group

**Disadvantages**
- ✗ Cannot compare with the general population

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Examples of PRO instruments

- Generic PRO instruments measure health across several areas, examples described below are the short-form health questionnaire (SF-36) and the EuroQOL five dimensions questionnaire (EQ-5D)\textsuperscript{1,2}
- A large number of disease-specific PRO instruments have been developed, for example over 110 PRO instruments have been developed in gastroenterology alone\textsuperscript{1}

**SF-36**
- Includes 36 items that are organised into eight discrete domains\textsuperscript{1,3}

**EQ-5D**
- Measures health-related quality of life (HRQoL) in five distinct domains\textsuperscript{2}

Responses to PRO instruments can be gathered through a number of different channels\(^1,2,3\):

- **Telephone**
  - Personal
  - Convenient
  - Only requires auditory proficiency
  - Impersonal if using automated systems
  - Resource intensive

- **Interview**
  - Personal
  - Doesn’t require literacy
  - Can be more in-depth and allows for in-person explanation
  - Expensive
  - Invasive

- **Mail**
  - Low tech and cost
  - Can lose data
  - Resource intensive

- **Paper**
  - Low technology and cost
  - Not for the illiterate
  - Can lose data
  - Resource intensive

- **Computer/Internet**
  - Efficient
  - May encourage patients to report sensitive information
  - Can be linked to electronic health records
  - Immediate scoring and presentation is possible
  - May allow for real-time data interpretation and timely interventions
  - Larger recruitment catchment area, less restricted by geographies and boundaries
  - Smaller investment in staff time and cost savings
  - Requires trained personnel and software
  - High technical costs and initially expensive
  - May require training of patients
  - Data privacy is an issue

Why do we need PRO studies?

- Patient-centred care is an emerging concept in healthcare and can be defined as:
  “Understanding of the patient’s thoughts, feelings, and expectations in the context of their illnesses in order to reach a common ground for integrated management”¹

- PROMs can be used to gauge patient opinion on their care and improve clinical practice²

- PROMs can be used to quantify consequences of interventions and quality of life following an intervention³

- Multiple stakeholders including, patients, payors, government bodies and the general public are interested in measuring the effects of a medical intervention⁴

- PROs are also important from a pharmacoeconomic perspective and are used when calculating quality-adjusted life years⁵

The applications of PROMs in clinical practice
How are PROMs used in clinical practice?

- PRO research generates ‘measures’, referred to as PROMS, that can be used to inform clinical practice.
  - A one-time assessment may help to identify problems that would otherwise have gone unnoticed.
  - Multiple assessments can track patient outcomes over time and may help to modify treatments, as required.
  - Linking PROMs to disease registries offers a cost-effective way to gather real-world data on disease progression and treatment outcomes.

- As a screening and treatment monitoring tool:
  - Helps to facilitate discussions between patients and clinicians regarding patient issues, including concerns and priorities for care.
  - Helps patients understand the treatment options that are available and assists in the selection of a treatment that is most suitable to the individual patient.

- Part of a patient-centred approach to care

- Linking to disease registries offers a cost-effective way to gather real-world data on disease progression and treatment outcomes.

Using PROMs as a treatment monitoring tool

- Linking PROMs to treatment outcomes provides an opportunity to monitor quality of life while undergoing treatment\(^1\)

Example: Monitoring treatment and HRQoL using PRO instruments in patients with metastatic breast, genitourinary, gynaecologic, or lung cancer

Objectives
- Use PRO instruments to monitor symptoms and HRQoL during cancer treatment

Patients
- Patients were randomised to report symptoms via an electronic device or means at the discretion of clinicians

Assessments
- HRQoL was evaluated using the EQ-5D PRO instrument at 6 months

Outcomes
- Patients who reported symptoms via an electronic device improved HRQoL compared with means at the clinicians discretion

Linking PROMs to disease registries

- Linking PROMs to disease registries allows the practitioner to gather real-world data in real time\(^1\)

**Example - The electronic Patient-reported Outcomes from Cancer Survivors (ePOCS) system**

### Objectives
- Link PROMs to disease registries for recently diagnosed patients with breast, colorectal, and prostate cancer

### Assessments
- Six different PRO instruments were administered at 6, 9 and 15 months, post diagnosis

### Participation
- Participation was considerably higher among patients approached face-to-face (61.4%, 490/798) compared with telephone (48.8%, 21/43) or by letter (41.0%, 125/305)

### Outcomes
- Patient participation was 55.21% (636/1152) overall, although this varied by mode of approach
- The PROMs were successfully linked with cancer registry data for 100% of patients (N=636)

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Implementing PROMs into clinical practice typically involves the consideration of a number of factors:\(^1\)

1. Identifying the goals for collecting PROMs
2. Selecting patients, settings and timings of assessments
3. Determining which PRO instrument to use
4. Mode for administration and scoring PROMs
5. Reporting results
6. Interpreting scores
7. Responding to issues identified by the PROMs
8. Evaluating the impact that PROMs may have on clinical practice

Challenges associated with PRO research

• There are some difficulties associated with developing PRO research.

**Poor literacy and understanding of PRO instruments**

**Challenge:**
• Patients with low literacy may fail to understand some questions

**Solution:**
• Format the PRO instrument so that it is easy to follow and use illustrations where appropriate

**Additional costs**

**Challenge:**
• Additional costs required to implement PRO research, such as training, administration and data interpretation

**Validation:**
• It would be expected that the additional costs are offset by the benefits to the patient and the practitioner

PRO clinical case study:

Northumbria NHS Healthcare Foundation Trust: The use of PROMs to assess the impact of knee replacement surgery
The programme

An NHS programme that began in April 2009 in the United Kingdom (UK) that used PRO instruments to evaluate health before and after elective knee replacement, hip replacement, varicose vein and groin hernia surgeries.
Implementing PROMs in clinical research

The objectives of the study
• To use PROMs to inform service changes and enable providers and other stakeholders to improve quality of care
• To empower patients and clinicians to make an informed choice on the course of treatment

Selecting patients
• The programme selected patients that were to undergo elective knee replacement, hip replacement, varicose vein and groin hernia surgery

Timing of assessment
• Pre-operative and post-operative PRO instruments were used to assess whether patient health improved following surgery; post-operative questionnaires were sent out 3 and 6 months after treatment

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1. Basser MR. Health and Social Care Information Centre. Can be accessed from: http://www.hscic.gov.uk/media/16547/full-PROMs-benefits-case-study/pdf/promscasestudy.pdf. Copyright © 2016, Re-used with the permission of the Health and Social Care Information Centre. All rights reserved
Selecting the PRO instrument

The generic PRO instruments that were used for all procedures were:

**EQ-5D Index**

- Patients provided responses to five questions, each covering a distinct health domain
  1. Mobility
  2. Self-care
  3. Performing usual activities
  4. Pain/discomfort
  5. Anxiety/depression
- Patients rated their health on one of three levels, ranging from ‘no problems’ to ‘severe problems’

**EQ Visual Analogue Scale (EQ VAS)**

- Patients rated their general health on an analogue linear scale ranging between 0 and 100, with 100 representing the best state of health

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1. Basser MR. Health and Social Care Information Centre. Can be accessed from: http://www.hscic.gov.uk/media/16547/full-PROMs-benefits-case-study/pdf/promscasestudy.pdf. Copyright © 2016, Re-used with the permission of the Health and Social Care Information Centre. All rights reserved
Disease-specific PRO instruments

- The disease-specific PRO instruments selected were:
  - Oxford Hip Score
  - Oxford Knee Score
  - Aberdeen Varicose Questionnaire

- 12 questions relating to the patient’s hip (Oxford Hip Score) or knee (Oxford knee score) and how it’s condition affects quality of life
- Responses combined to give an overall score, ranging from 0 (worst possible score) to 48 (best possible score)
- 13 questions relating to the patient’s varicose veins
- The responses are combined into an index, ranging from 0 (best possible score) to 100 (worst possible score)

1. Basser MR. Health and Social Care Information Centre. Can be accessed from: http://www.hscic.gov.uk/media/16547/full-PROMs-benefits-case-study/pdf/promcasestudy.pdf. Copyright © 2016, Re-used with the permission of the Health and Social Care Information Centre. All rights reserved
**Identifying an unmet need**

PROMs demonstrated that the Northumbria NHS Healthcare Foundation Trust was below the UK average for adjusted health gain following replacement knee surgery.

**Analysis of PROMs**

PROMs were linked to the National Joint Registry data for 22,691 primary total knee replacements to detect a correlation between PROMs scores and various surgical factors, including surgical implants.

**Linking PROMs to clinical outcomes**

One implant brand (brand C) returned the highest health gain for the Oxford Knee Score, compared with two other brands (brands A and B).

1. Basser MR. Health and Social Care Information Centre. Can be accessed from: http://www.hscic.gov.uk/media/16547/full-PROMs-benefits-case-study/pdf/promscasestudy.pdf. Copyright © 2016, Re-used with the permission of the Health and Social Care Information Centre. All rights reserved.
Evaluating clinical practice informed by PROMs

- The Northumbria NHS Foundation Trust moved to solely using the knee replacement implant brand C that demonstrated the superior Oxford Knee Score.
- To assess the effectiveness of the implant brand change, Northumbria Healthcare Foundation Trust analysed 1623 completed PRO instruments for the period April 2009 to March 2014.

<table>
<thead>
<tr>
<th>Brand</th>
<th>Number of primary knee replacement surgeries</th>
<th>Adjusted health gain for Oxford Knee Score</th>
</tr>
</thead>
<tbody>
<tr>
<td>Implant brand A</td>
<td>581</td>
<td>13.9</td>
</tr>
<tr>
<td>Implant brand B</td>
<td>246</td>
<td>14.2</td>
</tr>
<tr>
<td>Implant brand C</td>
<td>796</td>
<td>16.6</td>
</tr>
<tr>
<td>Total</td>
<td>1623</td>
<td>N/A</td>
</tr>
</tbody>
</table>

- The analysis showed that changing to implant brand C significantly improved adjusted health gain scores for the Oxford Knee Score.

1. Basser MR. Health and Social Care Information Centre. Can be accessed from: http://www.hscic.gov.uk/media/16547/full-PROMs-benefits-case-study/pdf/promscasestudy.pdf. Copyright © 2016, Re-used with the permission of the Health and Social Care Information Centre. All rights reserved.
Conclusions
Summary

• PRO instruments help to encourage a patient-centric approach to care

• PRO instruments are typically questionnaires that collect data across several areas of health, including physical, psychological and social functioning
  ○ PRO instruments can be either generic or disease-specific

• PROMs can be used to influence clinical practice in a number of ways, these include:
  ○ As a screening tool to identify conditions/symptoms that would otherwise have been overlooked
  ○ To monitor treatment and respond to patient outcomes over time
  ○ To aid treatment choices and assist in selecting treatments that are most suited to the patient

• Limitations of PROMs include comprehension of PRO instruments and associated costs of implementing and evaluating results
  ○ However, PRO instruments can be improved for comprehension and the clinical benefits of implementing changes as a result of PROMs can offset initial economic costs

Further reading


Self-assessment quiz
Question 1

How many health domains does the SF-36 PRO instrument evaluate?

1. Eight
2. Six
3. Four
4. Two
How many health domains does the EQ-5D PRO instrument evaluate?

1. Two
2. Four
3. Five
4. Eight
Question 3

Which of the following is not a type of PRO instrument?

1. Disease-specific
2. Individual patient-specific
3. Generic
Question 4

Which of the following is a disadvantage of disease-specific PRO instruments?

1. Specific for a given group (disease/population)
2. Sensitive enough to detect clinically significant changes
3. Captures clinically-relevant information that is applicable to a specific target group
4. Cannot compare with the general population
Question 5

Which of the following is a disadvantage of generic PRO instruments?

1. Suitable for the general public and for comparisons across populations
2. Suitable for comparisons with disparate conditions/disease groups
3. Captures commonly experienced health domains
4. Not sensitive to detecting disease-specific illnesses
Which of the following is a potential drawback to PRO research

1. PROs are a poor reflection of the patient experience
2. Exclusion of patients due to poor literacy
3. An inability to link PRO outcomes to clinical registries
Linking PROMs with clinical registries allows the clinician to establish:

1. Real world, retrospective data as it is processed
2. Prospective, randomised data
Which of the following uses for PROMs is not true?

PROMs can be used in clinical practice:
1. As a screening and monitoring tool
2. To replace randomised clinical trials to monitor the efficacy and safety of a treatment
3. As part of a patient-centred approach to care
4. As a treatment decision aid
The SF-36 and EQ-5D PRO instruments are examples of a:

1. Generic PRO instrument
2. Disease-specific PRO instrument
To compare the quality of life of a patient with a specific disease with that of healthy individuals, which of the two types of PRO instrument should be used?

1. Generic
2. Disease-specific