5 Things you need to know about patient reported outcome (PRO) measures

Selecting the appropriate PROM for your study

Keith Meadows
With the increasing prominence of the patient’s involvement in the care they receive, the assessment of outcomes based on the patient’s perspective using patient reported outcome measures (PROMs), are increasingly accompanying the traditional clinical ways of measuring health and the effects of treatment on the patient. However, with literally scores of PROMs to choose from you need to do more than base your selection on the name of the PROM, what it claims to measure or because it’s been used by others. You need to be assured that it’s appropriate for measuring the desired outcomes. You need evidence of the PROMs reliability and validity and above all you need to have a clearly defined measurement strategy that links the outcomes with the disease and treatment outcomes.

Over the past few years there has been a fundamental shift in focus to give greater emphasis to the involvement of the patient in the care they receive and which is reflected in a number of policy and national initiatives. Patients also want to be involved in the decision making process.

For more than a decade PROMs have played, and continue to have, an important role in national and international academic and clinical research including Phase III IV clinical trials, resulting in the development of many thousands of instruments to measure the patient’s self-reported outcomes of treatment.

The primary reason for using PROMs is to assess treatment effects that are known only to the patient and can provide deep insight into the feelings and experience of the patient.

Although infrequently applied in clinical practice, PROMs can be used for quality improvement for example by monitoring change in PROM scores for patients who are initiating or changing medications.

These are just some example as to the possible application of PROMs. The challenge for the user in selecting a PROM is that it is both reliable and valid, is measuring the outcomes you want to know about and provides insightful decision making information.

Selecting the most appropriate PROM is more than assuming the title of the PROM will tell you all you need to know or that the PROM has been used in numerous studies. Failure to consider key factors in the choice process will lead to the use of costly resource in the collection of invalid and unreliable information.

This paper provides you with some of the key factors in the decision making process in selecting an appropriate PROM.
1. What are PROMS and what do they measure?

What is a PROM?

PROMs are the tools we use to gain insight from the perspective of the patient into how aspects of their health and the impact the disease and its treatment are perceived to be having on their lifestyle and subsequently their quality of life (QoL). They are typically self-completed questionnaires, which can be completed by a patient or individual about themselves, or by others on their behalf.

But why should patients’ perspective be considered? Don’t the clinical outcomes inform of the effectiveness of care provided or the benefits of a new prescribed drug or treatment?

Clinical outcomes such as an HbA1c for a patient with diabetes or the peak flow rate of an asthma sufferer are useful clinical outcomes in terms of treatment effectiveness. However, as indicators of the impact the disease might be having on the patient’s quality of life, for example, due to the restrictions on their social activities as a consequence of a fear of going into a hypoglycaemic coma, clinical outcomes alone do not provide the full picture.

Access to the patients’ perspective through the use of PROMs can impact on a wide range of aspects related to the delivery of effective health care including, identifying those issues faced by patients and their families living with an illness and how this knowledge might impact on treatment.

What do PROMs measure?

A PROM should be designed to provide information around a given concept which must be made explicit by the instruments’ authors. Common concepts include, health status, health-related quality of life (HRQoL), QoL, well-being, treatment satisfaction, symptoms and functioning.

Health status - Measures of health-status such as the SF-36 and EQ-5D (EuroQol Group which are in common use, focus on the quality of health including, the biological and physiological functioning, symptoms as well as the physical e.g. the ability to climb a flight of stairs, as well as the psychological and social functional impairments.

Quality of life (QoL) - In defining QoL there is the general consensus that it is based on the individual’s subjective evaluation of the psychological, physical and social aspects of their life, which is changing over time as a result of different influences such as treatment. QoL is what the patient says it is.

Health related quality of life (HRQoL) - Often referred to as the degree to which the treatment and the disease as perceived by the individual to impact on those aspects of their life – in addition to health – which are considered important.
Well-being - Measures of psychological well-being focus on those aspects of psychological illness including, anxiety, depression, coping, positive well-being and adjustment and a sense of control and self-esteem. Typical measures of well-being include the Beck Depression Inventory (BDI) and the Hospital Anxiety and Depression Scale (HADS).

Patient satisfaction - PROMs developed to gauge the patient’s satisfaction with treatment focus on the patient’s subjective appraisal of their experiences of treatment including, side effects, efficacy and convenience.

Symptoms and functioning - Measures of symptoms can concentrate either on a range of impairments or a specific impairment such as depression or pain. PROMs assessing functioning focus on activities such as personal care and activities of daily living.

2. Selecting the appropriate PROM

Selecting the most appropriate PROM is of course the most critical aspect of the study design and in the absence of some universally agreed definition as to what the measured health concept is and its relationship to the objectives of the study, choosing the appropriate PROM can be problematic. It is not uncommon that the choice of an outcome measure such is based on the instrument been used in previous studies.

Developing a measurement strategy

An effective way to establish the link between the measured outcome such as the patient’s well-being following an intervention programme is to develop a measurement strategy, which requires a clear understanding of the disease and the outcomes relevant to the disease area and patient (Figure 1).

Figure 1. Key stages in the development of a measurement strategy
Central to the measurement strategy is the endpoint model which provides the rationale for the measurement model, by making explicit the associations among the different health outcomes, patient and domains to be measured by the PROM.

Based on the understanding of the disease and the expected treatment effects, Figure 2 illustrates a model showing the specific link between the primary clinical endpoint – of a reduction in the risk of hypoglycaemia – and the subsequent impact on patients’ QoL as the secondary endpoint to be measured by the PROM. Once the relevant endpoint(s) (outcome (s)) has been identified, the appropriate PROM can be selected or developed.

"It’s not uncommon that the choice of an outcome measure is based on whether it has been used in previous studies or the name of the instrument appears to be appropriate".

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What you need to consider when selecting a PROM

PROMs can be generally categorised as generic and disease or condition-specific, with each having their own strengths and weaknesses.

The Generic PROM: measures health concepts that are of relevance to a wide range of patient groups and the general population and as such can be used for comparison across different conditions as well as with healthy populations.

Due to the nature of their generic content, they will most likely include items that are not particularly relevant for example, to adolescents with diabetes whose concerns are more likely to be focused on dealing with the implications and complexities of the disease at a time of changing self-perception, sexual and physical development rather than questions relating to physical mobility such as ability to walk a block, climb one flight of stairs or reach for items on a shelf.

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**Conceptual model**

- Reduce recurrent hypoglycaemia
- Treatment improves blood sugar levels

**Signs and symptoms**

- Sweating, shaking, anxiety

**Disease related impact**

- Reduces blood sugar level
- Reduced risk of hypoglycaemia
- Improves QoL assessed by PROM

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*Figure 2. Endpoint model showing relationship between treatment effects and outcomes*
Also generic measures are more likely to exclude content that is of particular relevance to a specific disease group. For example, both for patients and clinicians issues around diet management and the enjoyment of food are key factors in the management of diabetes. Yet content specifically focusing on these important aspects are unlikely to be included in a generic measure, making them of less relevance to the respondent and less sensitive to detecting change in outcome.

The Disease-specific or Condition-specific PROMs: These have been developed to capture those elements of health and QoL of relevance to a specific patient disease group.

Often disease-specific PROMs are developed using qualitative research methodologies such as in-depth interviews and focus groups with people with the specific disease or condition, thus, ensuring that patients are only asked questions which are both meaningful and acceptable to them.

Disease-specific measures are also more likely to be of greater clinical relevance as well as being more responsive to detecting clinically important changes resulting from treatment intervention.

<table>
<thead>
<tr>
<th>Summary of PROM types</th>
<th>Strength &amp; weakness</th>
<th>Generic</th>
<th>Disease-specific</th>
</tr>
</thead>
<tbody>
<tr>
<td>Disease-specific</td>
<td>Suitable for general population</td>
<td>✓</td>
<td></td>
</tr>
<tr>
<td>Population-specific (e.g. elderly, children)</td>
<td>✓</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Dimension-specific (e.g. depression, pain)</td>
<td>✓</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Generic (General population)</td>
<td>✓</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Individually (Respondents choose item content)</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
</tbody>
</table>

Which type of PROM should you use?

The manner in which data from PROMs can be derived depends on the rationale behind its development and manner of scoring. For example, questionnaires that measures a single construct e.g. pain are described as uni-dimensional and the items (questions) are added to yield a single overall score. In contrast a multi-dimensional questionnaire is used to provide a profile of scores such as physical and social functioning, mental health and pain.
A cautionary note: When a PROM has been developed specifically as a multidimensional scale, it can never be assumed that an overall score can be derived simply by adding each of the items without evidence that the scale can be treated as uni-dimensional for example, through confirmatory factor analysis. Similarly, a PROM providing an overall score cannot be assumed to be measuring a single construct unless there is sound psychometric evidence to support this assumption.

3. What you need to know about Reliability & Validity

A PROM is only of value if it has been designed to strict criteria and based on a sound theoretical underpinning or conceptual model of what it is purported to measure (Figure 3).

A PROM must demonstrate satisfactory psychometric properties including, reliability, validity and sensitivity to detect change in the measured concept.

"Reliability tells how consistent our results are, whilst validity tell us whether we are measuring what we think we are".

Reliability

Because PROMs are often used in clinical trials to measure change over time, the adequacy of a PROM for a variety of clinical applications depends on its reliability or ability to provide consistent and reproducible estimates of true treatment effects. For this we need to look for evidence of good test-retest reliability.

- **Test-retest:** Can be established by the administration of the PROM at different time points at a time interval which is long enough with stable patients to minimize memory effects. This is usually obtained using correlation analysis of the two sets of scores where the coefficient should not only be statistically significant but, also high e.g. >0.80.

- **Internal consistency:** Measures whether several items that propose to measure the same general construct produce similar scores. For example, if a respondent expressed agreement with the statement "My general health is very good" and disagreed with the statement "I feel my general health could be much better", this would indicate consistency of the responses.

Another way of looking at internal consistency is that it represents an indication as to how well all the items of a PROM are measuring the concept e.g. pain depression, anxiety. Internal consistency i.e. Alpha coefficients should range between 0.70 and 0.85.

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"Validity is telling us how well we are measuring what we think we are measuring".

- **Content validity**: Content of a PROM is arguably its most important quality as without satisfactory content validity no amount of statistical manipulation will improve its measurement qualities. The measured constructs must be derived from patient interviews, clinical input and a comprehensive review of the literature.

- **Construct validity**: Is determined by evidence on the basis that the relationships among items, domains, and concepts conform to a priori hypotheses concerning relationships that should exist with other measures or characteristics of patients and patient groups. For examples, evidence from factor analyses confirming the structure and domains of the PROM or PROM scores discriminating between patients with good and poor health.

- **Criterion validity**: Criterion validity is the extent to which the scores of a PROM are related to a known measure of the same concept. For example we would expect to see a significant relationship between two PROMs measuring depression or anxiety.

- **Ability to detect change**: The ability to detect change includes evidence that the PROM is sensitive to gains and losses in the measured concept when the patient experience change.

4. **When to apply PROMs**

PROMs can be applied to obtain data from the patient’s perspective in a range of areas and applications.

- Data derived from a PROM can guide clinicians in making decisions about different clinical inputs and for monitoring the outcomes of specific interventions.

- PROMs can also provide a baseline assessment of the health status, QoL, patient satisfaction or well-being etc. of a specific population to identify need and the delivery of effective care.

- Aid communication between patient and doctor. For example, the doctor can discuss with the patient why a question was answered in a particular way or why the patient’s score has improved or declined since the previous visit.

- PROMs can also be routinely administered in clinical settings for audit and quality assurance such as in the assessment of the effectiveness of different procedures.

PROMs play an increasingly significant role in clinical research such as in randomized control Phase III trials as secondary endpoints to provide ‘added value’ to the primary biomedical outcomes.

Commercially, data derived from PROMs can for example, be used to support product differentiation, provide intelligence for the targeting of drug development and marketing and in some cases support labelling claims.
5. Ways to interpret PROM data

Interpretation of data derived from a PROM can be challenging, particularly with regard to understanding the meaning of what a change or difference in a score means clinically.

Interpretation of PROM data is linked with both the objectives of the study and the constructs measured by the PROM and as a consequence should be a key factor in the development of any measurement strategy.

Understandably, optimising the patient’s health is of primary importance for the clinician and therefore, is more likely to look for improvements in health status. However, what might be of specific interest to the clinician in terms of outcome may not always correspond to what is of relevance to the patient.

Remembering that PROMs are assessing outcomes from the perspective of the patient, any change in the score may or may not be related to a clinical outcome. As an example, patients with diabetes who have changed to a different insulin treatment considered more convenient – but, in all other respects is the same – are unlikely to report any improvement in clinical outcomes but, may well report that their QoL has been enhanced as a consequence of the greater flexibility in their lives.

Despite the many thousands of PROMs developed and their application, there is often lacking an understanding as to what a PROM score represents and what is a meaningful change in score. Should the differences be big or small and what are the implications for clinical practice and health policy?

When large samples (macro level) of patients are studied, differences in PROM scores between patient groups might be numerically small but, highly statistically significant. However, data obtained at the macro level are difficult to apply at the individual patient (micro) level.

Interpretability is also something that cannot be established from a one-off study but, is based on a body of evidence developed over time through a variety of studies, and perspectives. Nevertheless, there are a number of approaches that can aid the interpretation of PROM data and which are summarised below.

- **Minimal Important Difference (MID)** - the smallest difference in a score that is considered to be worthwhile or important.
- **Known groups** – the mean scores underlying particular clinical groups or clinical indicators which give rise to them and which can be used as a clinically based benchmark to compare other groups.
- **Normative and reference groups** – mean scores from defined large populations to provide normative data – typical scores – called norms. Mean scores from a particular study can be compared with the population norms.
- **Statistical significance** – The statistical significance of the probability of treatment (A) is better than treatment (B).
- **Effect size** – A way of quantifying the difference between two groups of patients that has many advantages over the use of statistical significance alone and emphasises the size of the difference rather than confounding this with sample size.
- **Cumulative distribution functions (CDF)** – The CDF shows a continuous plot of the proportion of patients at each point along the continuum of the scale score continuum experiencing change at that level or lower levels.

Other approaches include, reference to the PROMs content when interpreting its score as well as comparing them with known clinical parameters such as days in hospital and illness severity or the proportion of patients whose PROM score improve or get worse after intervention.
The challenges still facing us

Despite the increasing use of PROMs across a range of clinical settings and research, there are a number of important issues that remain to be addressed before we can maximise the benefits from their use such as in routine care including, for example, the need to ensure instruments, data collection and analysis is highly credible.

There must also be demonstrable evidence that PROMs have been developed with scientific rigour with proven reliability, validity and sensitivity to detect change where change is present as well as the need to make PROM data meaningful to those who need to be informed including, nurses, clinicians, commissioners, providers and policy makers. Data must be relevant, affordable and practical to collect and not affect the delivery of care in the process.

There is also a need to know more about how best PROMs can be embedded into the decision-making process and combine the derived data with other clinical information as well as explore ways to provide feedback as insightful information to enable sound clinical decision making.

A few initiatives to get you started

1. Establish buy in from all those involved in the project.
   It is essential that every person involved in the process is in agreement. This will include in particular those who will be administering the PROM or who run the service in order to minimise disruption to the delivery of care process. Any potential obstacles should be resolved at this stage.

2. Understand the big picture.
   Determine exactly the purpose of the project. This will include defining the inputs and desired outcomes to be measured which are relevant to the patient and project in order to develop the measurement strategy. Are these outcomes relevant to the patient? Can they be measured? And importantly, why these particular outcomes? Establishing the measured outcomes are essential and defining them to be ‘health status’ ‘quality of life’, ‘health-related quality of life’ etc. What action will follow once the PROM evidence is available and who are the key stakeholders involved?

3. Identify the practicalities
   Carefully define the patient group and determine whether a control group will be required. How will the PROM be administered? Who will undertake the analysis?

4. Selecting the appropriate PROM.
   This will combine a number of factors including development of the measurement strategy and a detailed and comprehensive review of the literature to define exactly what the measured outcomes are. This is the point at which selection must be based on the purpose of using a PROM, its scientific and measurement qualities and whether it’s possible to easily interpret the scores as to really what they mean. Selection must not be based on the name of the PROM or because it has been used in other studies.

Ultimately, we are only scratching the surface here when it comes to the implantation of a PROM. The key comes down to this: You have to plan for measurement. It is almost impossible to measure patient reported outcomes without a clear measurement strategy defining exactly the inputs and desired outcomes.

Questions? Comments? 1 Hour free consultation Click here
References


About the Author

Keith Meadows is Founder and Director of DHP Research, which specialises in the development and implementation of patient reported outcome and patient experience measures. Keith has held academic posts at the Universities of London, Newcastle and Hull, undertaking research across much of Europe, including Russia, Spain, Scandinavia and the low countries. He participated in the European Research Group on Health Outcomes with the BIOMED I project ‘Harmonised approaches to ambulatory care outcome measurement in Europe’ and the EU-funded project (SCOPE) on providing a quality culture in health care for older people. He has wide experience in health services research, with particular emphasis on the assessment of the psychosocial impact of living with diabetes, health-related quality of life, health survey research patient reported outcome measures, patient experience and engagement. Keith has over 90 research communications and publications and is a reviewer for a number of scientific journals. He also lectures at undergraduate and post graduate level at the Universities of Brighton, London and City. Between 2003 and 2008 Keith was Associate Director of the North East London Consortium For Research & Development (NELCRAD), where he was responsible for strategy development, capacity building and increasing primary care research across east London.

Keith is author of the Diabetes Health Profile (DHP), a diabetes-specific questionnaire designed to identify the psychological and behavioural impact of living with diabetes.

For more information on the DHP visit: http://www.diabeteshealthprofile.com

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