Chapter 1

Pre-test Assessment of Urinary Dysfunction, Using Patient-Centred Questionnaires

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1.1 The History of the Development of Patient-Centred Questionnaire Assessment

Patient-centred questionnaires and patient-reported outcome (PRO) measures are terms that are used interchangeably to reflect an instrument that provides evaluation of the lived experience of symptoms from the patients' perspective. PRO use has grown significantly in the past 10–15 years, due to recognition of the importance of placing patients at the centre of their care [1]. It is recognised that only those individuals *experiencing* symptoms can report on the more subjective elements [2]. This is particularly important in the case of urodynamics, which is a clinical test. PROs provide a method of measuring subjective phenomena in an objective way and provide context to the data provided by clinical measurements. PROs can be used to record the presence and severity of symptoms and also to measure their impact, in particular on quality of life. This is useful when interpreting patients' priorities for treatment and understanding the most bothersome aspect of their symptoms.

PROs must be developed using a robust methodology in order to provide accurate measurements. It is therefore necessary to understand the principles underpinning their development in order to select the most appropriate questionnaires to use for a given patient, condition or clinical intervention [3]. The key characteristics to determine the robustness of a PRO are as follows (summarised in Table 1.1):

- Context of use – the intended population.
- Content validity – the process of item generation and interpretation of the PRO.
- Psychometric robustness – characteristics of the measurement properties. (FDA 2009, www.fda.gov/regulatory-information/search-fda-guidance-documents/patient-reported-outcome-measures-use-medical-product-development-support-labeling-claims)

1.1.1 Context of Use

The PRO should be developed in a population that represents the intended respondents, to promote its applicability [4]. The developmental population may be specific and the appropriateness of the PRO for the intended population should be evaluated. Where the PRO appears to be relevant but has not been developed in the specific population, it can be further evaluated in the intended population for its relevance and appropriateness. The important factor is whether input has been sought from the intended population to derive the PRO content. Only by conducting qualitative research, for example, through interviews or focus groups, can the PRO be capture issues of relevance that are pertinent to potential respondents.

Table 1.1 PRO characteristics to evaluate measurement capability and robustness

Parameter	Importance in PRO selection
Context of use	Determines appropriateness for the population in which use is intended
Content validity	Evidence of comprehensiveness and relevance to the population, and interpretation by potential respondents
Psychometric robustness • *Additional validity* • *Reliability* • *Responsiveness*	Quantitative data that provide evidence of measurement capability to provide robust and accurate data

1.1.2 Content Validity

Content validity is an evaluation of whether the PRO captures what it intends to measure and whether it can be clearly interpreted to provide these data [2,5]. Cognitive debriefing interviews are typically employed to evaluate this in which potential respondents complete the draft PRO and discuss their thoughts while completing the tool, followed by a structured interview to assess whether question items are interpreted as intended [6].

1.1.3 Psychometric Robustness

1.1.3.1 Additional Validity

Other components of validity testing include:

- Convergent validity – quantitative assessment of the relationship between findings from the PRO and measures of similar variables. Where constructs are closely related, the relationships should be stronger, and weaker relationships evident with more poorly related constructs.
- Discriminant validity – an assessment of the PRO's capability to distinguish between known patient groups, for example, those with mild, moderate, and severe symptoms.
- Criterion validity – an assessment of the relationship between the PRO and an accepted gold standard, if available. Clinical proxy measures can be used as a direct comparison with a PRO, due to its variable nature is rarely possible.

These elements are typically considered essential for the complete validation of a PRO but should be judged in context. The more components of validity evaluated will provide more evidence of the robustness of the PRO to provide accurate and reliable data.

1.1.3.2 Reliability

The ability of the PRO to measure in a consistent manner over time is vital to ensure any change detected is due to a real change in symptom status, rather than measurement error. This can be measured in two ways:

- Internal consistency – a measure of the homogeneity of the questionnaire or how well the items relate to each other, without a direct duplication.
- Test–retest reliability – a measure of the stability of responses over time when symptom status is considered to have remained stable.

1.1.3.3 Responsiveness

The PRO's ability to detect change where real symptom improvement, or deterioration, has occurred is particularly important for outcome measurement in order to evaluate the change following intervention. The magnitude of change should also be evident in order to make comparisons between treatments with anticipated different effect sizes; for example, surgical versus conservative intervention [1].

1.2 The International Consultation on Incontinence Modular Questionnaire (ICIQ)

1.2.1 An ICIQ Overview

The International Consultation on Incontinence Modular Questionnaire (ICIQ) was developed to provide a universally applicable, standardised series of self-completion assessment instruments to evaluate lower urinary tract symptoms, lower bowel symptoms and vaginal symptoms [7]. It began in 1999 and now consists of a collection of 16 high-quality questionnaires including a validated bladder diary [8,9].

The ICIQ PROs can be used by men and women irrespective of age, with varied causes for their symptoms, facilitating the use of common questionnaires in many patient groups. It was developed from a standard protocol for the development and translation of ICIQ questionnaires. The content of the questionnaires is derived from patients and clinical experts, providing a robust evidence base for inclusion.

Core modules evaluate the core symptoms of lower pelvic dysfunction, namely lower urinary tract, lower bowel and vaginal symptoms. An example of an ICIQ module, the ICIQ-UI Short Form, is provided in Figure 1.1.

Additional specialised and detailed modules are available, such as overactive bladder symptoms, nocturia, quality of life and sexual matters (Table 1.2).

The ICIQ is an international collaborative project encouraging worldwide standardisation of assessment and is available in numerous languages. Collaboration is invited to further develop questionnaires and translations.

1.2.2 Use of the ICIQ

A number of ICIQ modules have now been incorporated into The National Institute of Health and Care Excellence (NICE) guidelines with recommendations for their use when evaluating interventions and recognition of their status in the field of PRO provision [10]. In Scotland, primary care guidelines include recommendations for use of the ICIQ [11]. They are recommended as first line for PROs and global use in clinical practice and research by the triennial international consultation on incontinence [1]. Questionnaire modules are supplied free of charge for clinical practice and academic research. They can be accessed via the project website www.iciq.net.

With the need to provide electronic patient records, the requirement to move away from paper and pencil administration is increasingly recognised. The eICIQ has been fully validated to provide evidence of the equivalence of its measurement properties in the altered format and its online provision is in development [12].

Initial number

ICIQ-UI Short Form

CONFIDENTIAL

DAY MONTH YEAR

Today's date

Many people leak urine some of the time. We are trying to find out how many people leak urine, and how much this bothers them. We would be grateful if you could answer the following questions, thinking about how you have been, on average, over the PAST FOUR WEEKS.

1 Please write in your date of birth: DAY MONTH YEAR

2 Are you *(tick one)*: Female ☐ Male ☐

3 How often do you leak urine? *(Tick one box)*

never ☐	0
about once a week or less often ☐	1
two or three times a week ☐	2
about once a day ☐	3
several times a day ☐	4
all the time ☐	5

4 We would like to know how much urine <u>you think</u> leaks.
How much urine do you <u>usually</u> leak (whether you wear protection or not)?
(Tick one box)

none ☐	0
a small amount ☐	2
a moderate amount ☐	4
a large amount ☐	6

5 Overall, how much does leaking urine interfere with your everyday life?
Please ring a number between 0 (not at all) and 10 (a great deal)

0 1 2 3 4 5 6 7 8 9 **10**
not at all — a great deal

ICIQ score: sum scores 3+4+5 ☐ ☐

6 When does urine leak? *(Please tick all that apply to you)*

never – urine does not leak ☐
leaks before you can get to the toilet ☐
leaks when you cough or sneeze ☐
leaks when you are asleep ☐
leaks when you are physically active/exercising ☐
leaks when you have finished urinating and are dressed ☐
leaks for no obvious reason ☐
leaks all the time ☐

Thank you very much for answering these questions.

Figure 1.1 ICIQ-UI Short Form (© ICIQ group). See www.iciq.net for ICIQ user policy: questionnaires can be requested centrally for supply in their original format

Table 1.2 The International Consultation on Incontinence Modular Questionnaire structure

Condition	Recommended modules	Optional modules	Recommended add-on modules		
	(A) Core modules		QoL	Sexual matters	Post-treatment
Urinary symptoms	Males: ICIQ-MLUTS Females: ICIQ-FLUTS	Males: ICIQ-MLUTS LF Females: ICIQ-FLUTS LF	ICIQ-LUTSqol	Males: ICIQ-MLUTSsex Females: ICIQ-FLUTSsex	ICIQ-S[a] (satisfaction)
	ICIQ-bladder diary				
Vaginal symptoms	ICIQ-VS		ICIQ-VSqol[a]		
Bowel symptoms	ICIQ-B	ICIQ-IBD			
Urinary incontinence	ICIQ-UI SF	ICIQ-UI LF[a]	ICIQ-LUTSqol	Males: ICIQ-MLUTSsex Females: ICIQ-FLUTSsex	
Condition	(B) Specific patient groups		QoL	Sexual matters	
Nocturia	ICIQ-N		ICIQ-Nqol	Males: ICIQ-MLUTSsex Females: ICIQ-FLUTSsex	
Overactive bladder	ICIQ-OAB		ICIQ-OABqol	Males: ICIQ-MLUTSsex Females: ICIQ-FLUTSsex	
Underactive bladder	ICIQ-UAB[a]				
Long-term catheter	ICIQ-LTCqol				
Children	ICIQ-CLUTS[a]				
Absorbent pads			ICIQ-Padprom		
Cognitively impaired adults	ICIQ-Cognitively impaired adults[a]				

[a] Developmental version in process of validation.

1.3 Other Recommended Questionnaires for LUTS and Quality of Life Assessment

Whilst the ICIQ is the PRO of choice, it is recognised that these questionnaires may not be appropriate for an intended purpose [13]. Grade A recommendations are awarded where there is published evidence of validity, reliability and responsiveness according to standard psychometric testing. If additional evidence of content validity evaluation is provided, a Grade A* is awarded to reflect the extra efforts taken to ensure the most rigorous developmental processes were undertaken at the item generation stage.

1.4 Advantages and Disadvantages of Using Patient-Centred Questionnaires

PROs offer valuable patient insight into the lived experience of their symptoms and their use is advocated in clinical practice and research globally. It is important to be aware of their strengths and limitations, particularly for their inclusion in clinical practice, as identified below.

Advantages

- Quick and comprehensive assessment of symptoms and associated 'bother'.
- Complement clinical assessment of patients with lower urinary tract symptoms.
- Valuable for evaluating outcomes of treatment or interventions.
- Provides a vehicle to raise the discussion about symptoms often considered embarrassing and which patients are often reluctant to disclose.
- Provides the patients' perspective of their symptoms and a measure of their severity.
- Relatively cheap method of data collection although must consider associated costs such as administration and data entry.
- Provides a robust tool for clinical audit and research.

Disadvantages

- PROs must be used as intended and proxy completion can be inaccurate if this method of completion has not been evaluated.
- PROS can be restrictive in their remit and format and prevent patients from providing further information.
- Patients may inadvertently skew data to provide 'false positive' perspectives to conform with socially accepted norms, or 'false negative' perspectives that exaggerate the severity of their symptoms.
- Postal response rates can be poor.
- Translation availability may restrict the populations able to complete PROs.
- Data may be missing due to PRO questions being overlooked.
- Due to the subjective nature of the assessment, the current status of the patient may alter the answers provided.

1.5 Access to Patient-Centred Questionnaires

Aside from contacting individual authors, there are a number of ways to access PROs through collection databases. Larger projects such as the ICIQ can be accessed directly through the project website, given the nature of the project and number of questionnaires available. Industry developers also host database collections of their tools such as the Pfizer

Patient-Reported Outcomes platform: www.pfizerpatientreportedoutcomes.com. ProQolid is an independent database of clinical outcome assessments that is organised by symptom area to provide an overview of measures available and their characteristics in order to make informed choices and provide access routes to individual PROs (https://eprovide.mapi-trust.org/about/about-proqolid). Licences and fees may apply according to the intended use, which should be established before implementation in the clinical or research setting. *ePAQ (electronic Personal Assessment Questionnaire)* is a validated, web-based clinical assessment system, specifically designed to provide detailed self-reported symptoms and quality of life data from women with pelvic floor disorders. ePAQ enables the delivery of virtual clinics and provides valuable outcomes data for activities including audit, service evaluation, revalidation, appraisal and research [14].

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