Applying the PRO Findings in Practice: Guidance for Clinicians

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PROTEUS
Patient-Reported Outcomes Tools: Engaging Users and Stakeholders

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Patient-Reported Outcome Tools: Engaging Users & Stakeholders

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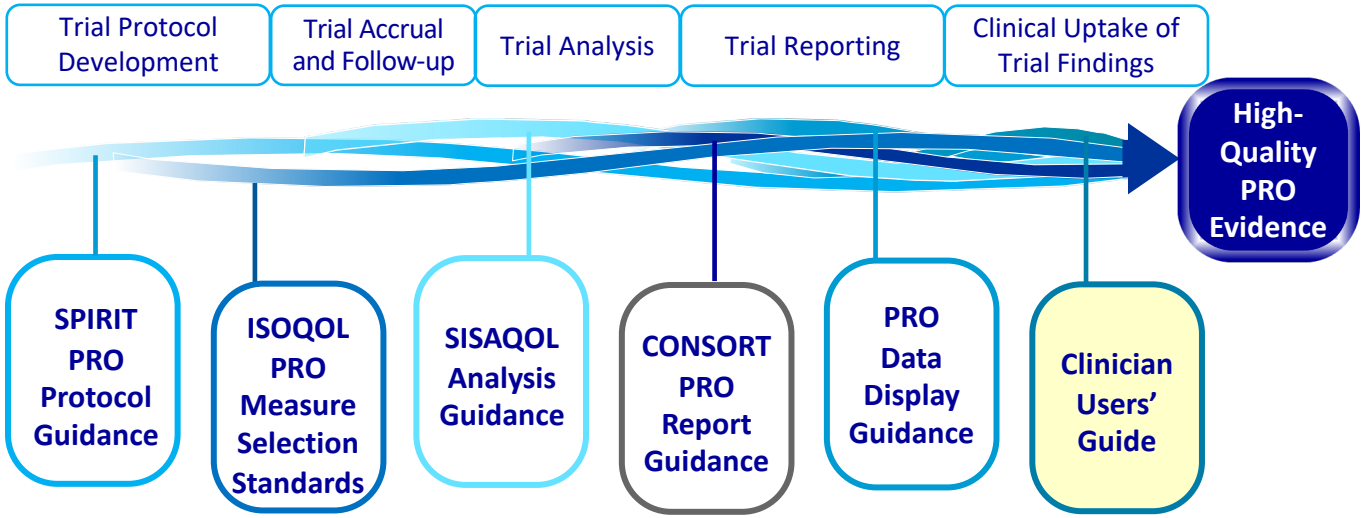
Overview of Presentations

Introduction to PROs and PROTEUS

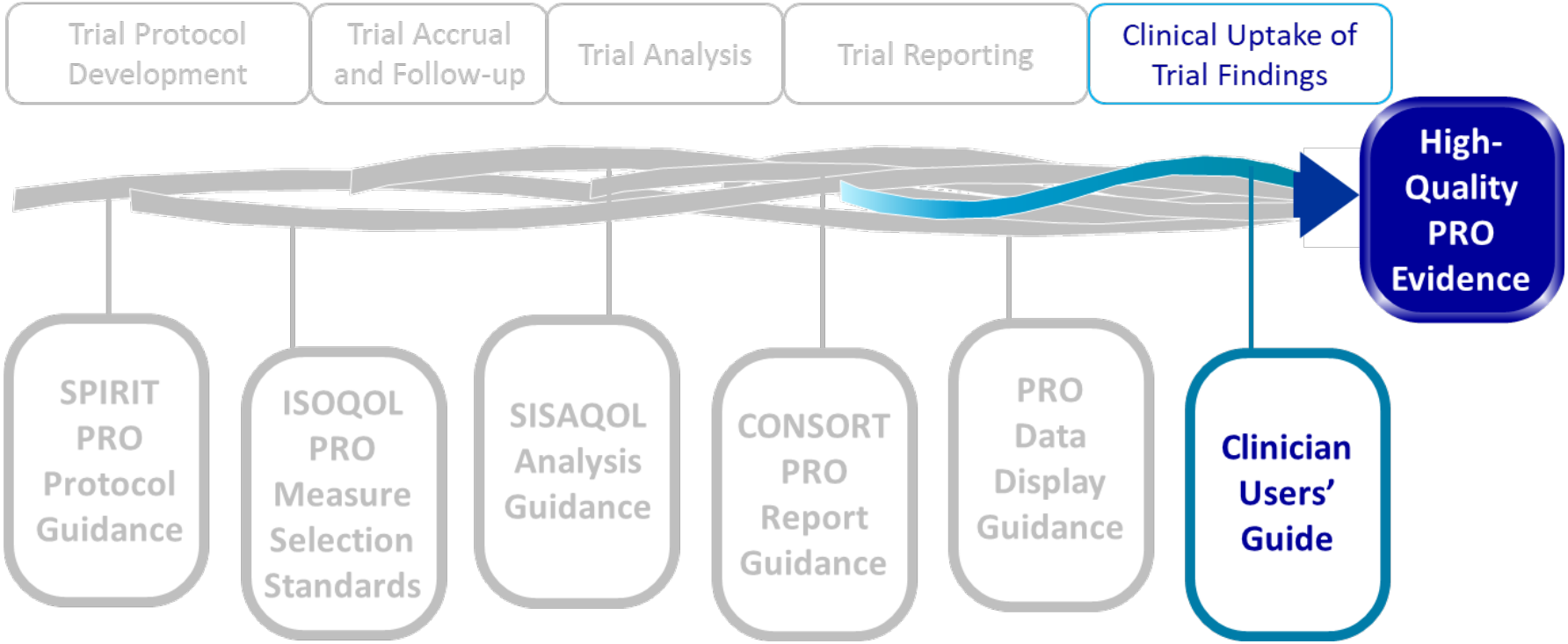
Introduction to the PROTEUS Tools



Overview of Tool Recommendations

How to Apply the Tools

Applying the PRO Findings in Practice



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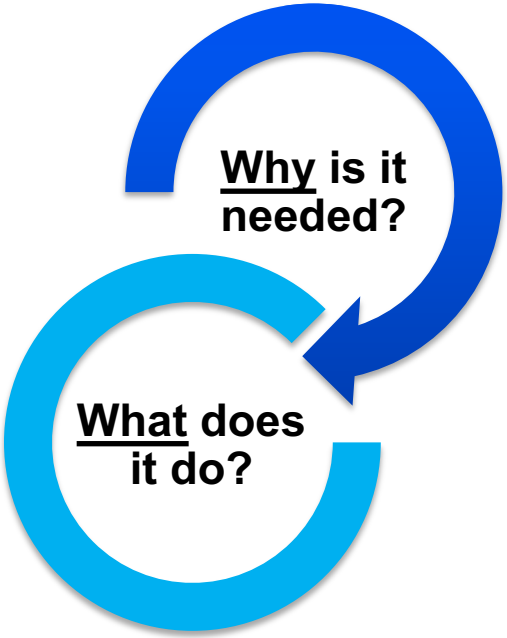
Applying PRO Findings in Practice



To help clinicians assess the quality of PRO research studies and determine whether findings are useful for clinical practice

Provides a checklist to evaluate the quality of studies that use PROs

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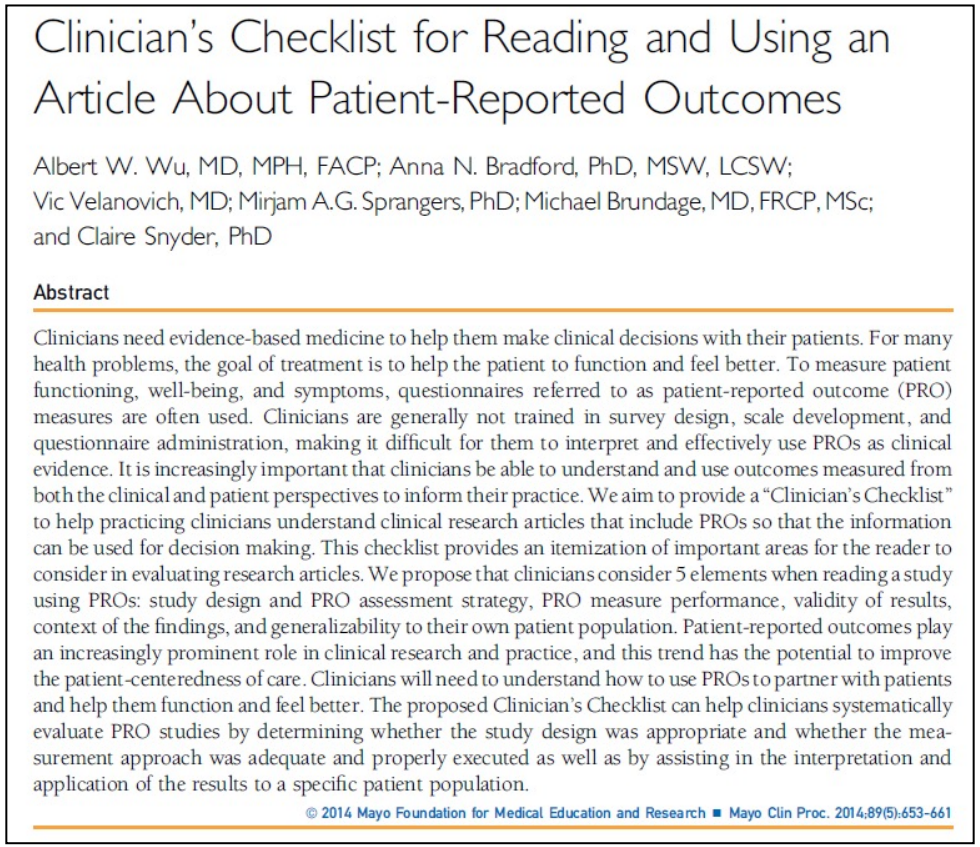
Applying the PRO Findings in Practice



Clinician's Checklist for Reading and Using an Article About Patient-Reported Outcomes

Albert W. Wu, MD, MPH, FACP; Anna N. Bradford, PhD, MSW, LCSW; Vic Velanovich, MD; Mirjam A.G. Sprangers, PhD; Michael Brundage, MD, FRCP, MSc; and Claire Snyder, PhD

Abstract

Clinicians need evidence-based medicine to help them make clinical decisions with their patients. For many health problems, the goal of treatment is to help the patient to function and feel better. To measure patient functioning, well-being, and symptoms, questionnaires referred to as patient-reported outcome (PRO) measures are often used. Clinicians are generally not trained in survey design, scale development, and questionnaire administration, making it difficult for them to interpret and effectively use PROs as clinical evidence. It is increasingly important that clinicians be able to understand and use outcomes measured from both the clinical and patient perspectives to inform their practice. We aim to provide a "Clinician's Checklist" to help practicing clinicians understand clinical research articles that include PROs so that the information can be used for decision making. This checklist provides an itemization of important areas for the reader to consider in evaluating research articles. We propose that clinicians consider 5 elements when reading a study using PROs: study design and PRO assessment strategy, PRO measure performance, validity of results, context of the findings, and generalizability to their own patient population. Patient-reported outcomes play an increasingly prominent role in clinical research and practice, and this trend has the potential to improve the patient-centeredness of care. Clinicians will need to understand how to use PROs to partner with patients and help them function and feel better. The proposed Clinician's Checklist can help clinicians systematically evaluate PRO studies by determining whether the study design was appropriate and whether the measurement approach was adequate and properly executed as well as by assisting in the interpretation and application of the results to a specific patient population.

 ■ Mayo Clin Proc. 2014;89(5):653-661

Wu et al, Mayo Clin Proc 2014, 89(5), 653-661

Acknowledgements

Collaborators: Albert W. WU, MD, FACP; Anna N. Bradford, PhD, MSW, LCSW; Vic Velanovich, MD; Mirjam A.G. Sprangers, PhD; Michael Brundage, MD, FRCP, MSc; and Claire Snyder, PhD

This article was reviewed by members of the International Society for Quality of Life Research (ISOQOL) and is endorsed by the ISOQOL Board of Directors.

Why Is This Resource Needed?

- In order to use PRO results to inform patient care, clinicians need to be able to evaluate published literature that includes PROs

- Barriers to clinicians applying PRO findings in clinical practice include:
  - lack of education and training on measurement and interpretation of PROs
  - wide variety of PRO measures
  - variation in how PRO findings are reported in the literature

Objective of this Resource

- **Purpose:** To help practicing clinicians apply results of clinical research studies that include PROs in their patient care

- **How?** By providing a brief **checklist** to help clinicians review published research studies that include PROs

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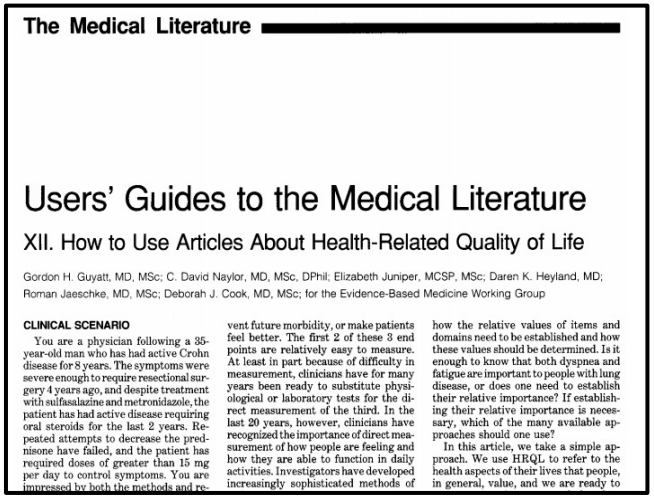
Methods

- Builds on guidelines published by Guyatt et al 1997

- Wu et al 2014, propose 5 key elements to consider when reading a published study using PROs:
  1. Assessment strategy & study design
  2. Performance of the PRO tool
  3. Validity of results
  4. Context of results
  5. Generalizability to one's own patient population

- Questions were formulated into a Clinician's Checklist to address each key element



The Medical Literature

Users' Guides to the Medical Literature

XII. How to Use Articles About Health-Related Quality of Life

Gordon H. Guyatt, MD, MSc; C. David Naylor, MD, MSc, DPhil; Elizabeth Juniper, MCSP, MSc; Daren K. Heyland, MD; Roman Jaeschke, MD, MSc; Deborah J. Cook, MD, MSc; for the Evidence-Based Medicine Working Group

CLINICAL SCENARIO

You are a physician following a 35-year-old man who has had active Crohn disease for 8 years. The symptoms were severe enough to require resectional surgery 4 years ago, and despite treatment with sulfasalazine and metronidazole, the patient has had active disease requiring oral steroids for the last 2 years. Repeated attempts to decrease the prednisone have failed, and the patient has required doses of greater than 15 mg per day to control symptoms. You are impressed by both the methods and re-

vent future morbidity, or make patients feel better. The first 2 of these 3 end points are relatively easy to measure. At least in part because of difficulty in measurement, clinicians have for many years been ready to substitute physiological or laboratory tests for the direct measurement of the third. In the last 20 years, however, clinicians have recognized the importance of direct measurement of how people are feeling and how they are able to function in daily activities. Investigators have developed increasingly sophisticated methods of

how the relative values of items and domains need to be established and how these values should be determined. Is it enough to know that both dyspnea and fatigue are important to people with lung disease, or does one need to establish their relative importance? If establishing their relative importance is necessary, which of the many available approaches should one use?

In this article, we take a simple approach. We use HRQL to refer to the health aspects of their lives that people, in general, value, and we are ready to

Guyatt et al, JAMA 1997, 277(15), 1232-1237

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Clinician's Checklist to Evaluate Studies Using PROs

1. Was the PRO assessment strategy appropriate?	
a. PRO hypothesis stated?	A priori hypothesis explicit for PROs
b. PRO measures described?	PRO measures used, and timing/follow-up of subjects
c. PRO content appropriate?	• Investigators measured aspects of patients' lives that patients consider important • PRO domains correspond to anticipated effects of disease and treatment • All important aspects of patient-reported outcomes included

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Clinician's Checklist to Evaluate Studies Using PROs

2. Did they measure PROs effectively?	
a. Evidence for reliability, validity?	The PRO instruments appear to work as intended; evidence of internal consistency and/or test-retest reliability, and construct validity are well established
b. Were missing data handled appropriately?	• Similar number of questionnaires completed by respondents in all treatment groups at every time point • Missing data management strategy described • Presence of data analysis plan for handling death, if frequent

Clinician's Checklist to Evaluate Studies Using PROs

3. Should I believe the results?	
a. Internal validity	• Findings established; observed effects likely to be caused by intervention • If non-treatment factors affect PRO, risk adjustment needed

Clinician's Checklist to Evaluate Studies Using PROs

4. Were the results placed in a clinical context?	
a. Was the clinical meaning of results explained?	• Magnitude of effect on PROs described • Clinical importance of observed differences in PRO scores demonstrated
b. Will the results help me in caring for my patients?	• Benefits and harms recognized and reconciled, including potential trade-offs between quality and quantity of life • Description of what a clinician should do with the results; study information helps clinicians communicate with patients about treatment options; applicability of group results to individual patient

Clinician's Checklist to Evaluate Studies Using PROs

5. Do the results apply to my patients?	
a. External validity to clinician's practice	• Study population is similar enough to clinician's patient population to apply to practice

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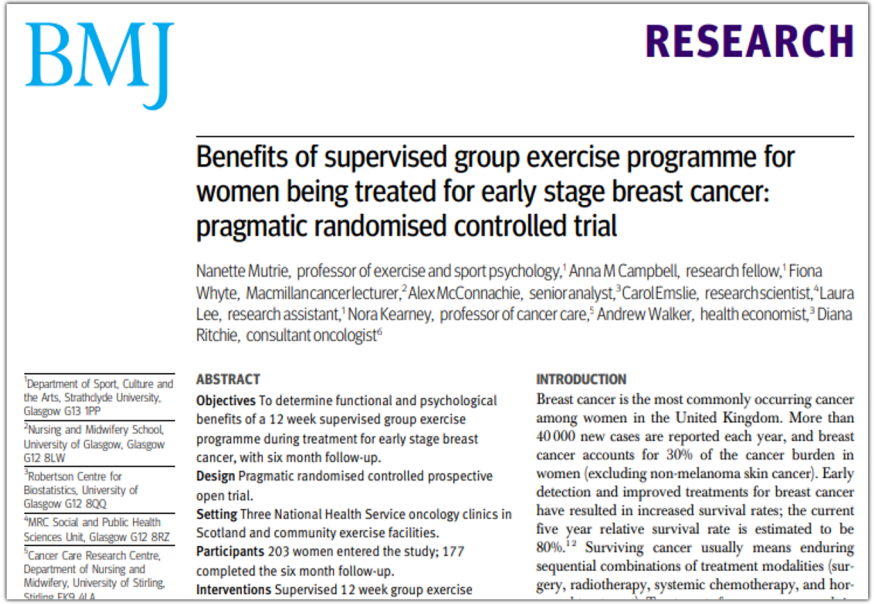
Illustrative Example

- For illustrative purposes, we apply the Clinician's Checklist to a published article to illustrate its use when evaluating clinical studies

- The article by Mutrie et al 2007, examines a randomized controlled trial of a group exercise program for women with early-stage breast cancer

  - Both primary and secondary outcomes were measured using PROs



BMJ

RESEARCH

Benefits of supervised group exercise programme for women being treated for early stage breast cancer: pragmatic randomised controlled trial

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ABSTRACT

Objectives To determine functional and psychological benefits of a 12 week supervised group exercise programme during treatment for early stage breast cancer, with six month follow-up.
Design Pragmatic randomised controlled prospective open trial.
Setting Three National Health Service oncology clinics in Scotland and community exercise facilities.
Participants 203 women entered the study; 177 completed the six month follow-up.
Interventions Supervised 12 week group exercise

INTRODUCTION

Breast cancer is the most commonly occurring cancer among women in the United Kingdom. More than 40 000 new cases are reported each year, and breast cancer accounts for 30% of the cancer burden in women (excluding non-melanoma skin cancer). Early detection and improved treatments for breast cancer have resulted in increased survival rates; the current five year relative survival rate is estimated to be 80%.[1 2] Surviving cancer usually means enduring sequential combinations of treatment modalities (surgery, radiotherapy, systemic chemotherapy, and hor-

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Mutrie et al 2007 - Evaluation Using PRO Clinician's Checklist

1. Was the PRO assessment strategy appropriate?	
a. PRO hypothesis stated?	Hypothesis stated: "12 weeks of supervised group exercise … would improve quality of life for women during treatment for early stage breast cancer … benefits maintained for six months after the intervention." Patients randomized to intervention (group exercise) or usual treatment (no group exercise) groups.

Text excerpt:

treatment for early stage breast cancer had functional and psychological benefits. We tested the hypotheses that 12 weeks of supervised group exercise, as an adjunct to usual care, would improve quality of life for women during treatment for early stage breast cancer and that benefits would be maintained for six months after the intervention.

Mutrie et al 2007 - Evaluation Using PRO Clinician's Checklist

1. Was the PRO assessment strategy appropriate? (cont.)	
b. PRO measures described?	FACT-General presented as primary outcome measure, with the 4 subscales described. Secondary PRO measures: BDI and PANAS. Other Secondary measures of physical activity, body mass index, 12-minute walk test, shoulder mobility test. References provided for FACT scales' and other PRO measures' development and validation; measures described in general terms as "appropriate for use with cancer patients." Data collected (baseline, the end of the intervention, 6 months) appropriate given the intervention and the hypothesis.
c. PRO content appropriate?	Outcomes assessed are supported by the brief literature presented; appropriate for the hypothesis.

Mutrie et al 2007 - Evaluation Using PRO Clinician's Checklist

2. Did they measure PROs effectively?	
a. Evidence for reliability, validity?	No data presented on the reliability and validity of the PRO data from this study; reliability and validity of these questionnaires previously established.
b. Were missing data handled appropriately?	A flowchart of allocation and assessment provided. The intervention group had greater loss to follow-up than the control group. Deaths were rare events in both groups. No specific discussion of analytic approaches to address missing data.

Mutrie et al 2007 - Evaluation Using PRO Clinician's Checklist

3. Should I believe the results?	
a. Internal validity	The authors acknowledge that the primary hypothesis was not supported. Presentation of the results focuses on outcome differences found between the groups assigned to exercise and usual care. Significant differences (*P*<.0001) and trends identified (eg. 12-min walk and shoulder mobility) are difficult to interpret given the failure to meet the primary end point and the lack of specification regarding which particular PRO domains were expected to differ between groups.

Mutrie et al 2007 - Evaluation Using PRO Clinician's Checklist

4. Were the results placed in a clinical context?	
a. Was the clinical meaning of results explained? Will the results help me in caring for my patients?	The authors note that it is difficult to determine what part of the exercise program was associated with the benefits and that participation in the group itself may have been valuable. The findings on the shoulder and walk tests support physical benefits.

Text excerpt:

"One weakness is that we do not know which aspect of the group exercise experience provided most benefit. Our qualitative data suggest that the group itself was an important aspect and that exercise in standard settings did not provide the same benefits. …… In addition, improvements in the 12 minute walk and shoulder mobility tests in favour of the intervention group are more directly attributable to the exercise than to the group effect".

Mutrie et al 2007 - Evaluation Using PRO Clinician's Checklist

5. Do the results apply to my patients?	
a. External validity to clinician's practice	Previous research established the potential of exercise to improve physical and psychosocial aspects of quality of life among breast cancer survivors during and after treatment. Although interpretation is difficult without statistically significant results on the primary end point, the authors promote the various positive findings from the study. This study used a group-based exercise program; generalizability therefore limited to settings in which group exercise could be implemented. "Many" participants could not attend the classes because of the required commuting time to class.

Recap

- The quality of PRO research studies affects the usefulness of the resulting PRO findings for clinical decision making
- The Clinician's Checklist can help clinicians to:
  - determine whether a PRO study was conducted with sufficient rigor for the results to be applied in practice
  - evaluate the relevance of PRO findings for their own patients and practice
- If ✓ PRO results are believable
  ✓ PRO endpoints are relevant to the specific patient population
  ✓ The magnitude of the results is clear

→ The clinician will be in a stronger position to apply PRO findings in practice

Further Reading

Wu A, Bradford A, Velanovich V, Sprangers M, Brundage M & Snyder C. Clinician's checklist for reading and using an article about patient-reported outcomes. *Mayo Clinic Proc.*89(5):653-661; 2014

Guyatt GH, Naylor CD, Juniper E, Heyland DK, Jaeschke R, Cook DJ. Users' guides to the medical literature, XII: how to use articles about health-related quality of life. Evidence-based Medicine Working Group. *JAMA.* 277(15):1232-1237; 1997

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