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Patient-reported Outcomes in Randomised Controlled Trials of Prostate Cancer: Methodological Quality and Impact on Clinical Decision Making

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EORTC Quality of Life Group (Patient Reported Outcome Measurements Over Time In ONcology-PROMOTION Project)

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Abstract

Context: Patient-reported outcomes (PRO) data from randomised controlled trials (RCTs) are increasingly used to inform patient-centred care as well as clinical and health policy decisions.
Objective: The main objective of this study was to investigate the methodological quality of PRO assessment in RCTs of prostate cancer (PCa) and to estimate the likely impact of these studies on clinical decision making.
Evidence acquisition: A systematic literature search of studies was undertaken on main electronic databases to retrieve articles published between January 2004 and March 2012. RCTs were evaluated on a predetermined extraction form, including (1) basic trial demographics and clinical and PRO characteristics; (2) level of PRO reporting based on the recently published recommendations by the International Society for Quality of Life Research; and (3) bias, assessed using the Cochrane Risk of Bias tool. Studies were systematically analysed to evaluate their relevance for supporting clinical decision making.
Evidence synthesis: Sixty-five RCTs enrolling a total of 22 071 patients were evaluated, with 31 (48%) in patients with nonmetastatic disease. When a PRO difference between treatments was found, it related in most cases to symptoms only (n = 29, 58%). Although the extent of missing data was generally documented (72% of RCTs), few reported details on statistical handling of this data (18%) and reasons for dropout (35%). Improvements in key methodological aspects over time were found. Thirteen (20%) RCTs were judged as likely to be robust in informing clinical decision making. Higher-quality PRO studies were generally associated with those RCTs that had higher internal validity.
Conclusions: Including PRO in RCTs of PCa patients is critical for better evaluating the treatment effectiveness of new therapeutic approaches. Marked improvements in PRO quality reporting over time were found, and it is estimated that at least one-fifth of PRO RCTs have provided sufficient details to allow health policy makers and physicians to make critical appraisals of results.

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Patient summary: In this report, we have investigated the methodological quality of PCa trials that have included a PRO assessment. We conclude that including PRO is critical to better evaluating the treatment effectiveness of new therapeutic approaches from the patient's perspective. Also, at least one-fifth of PRO RCTs in PCa have provided sufficient details to allow health policy makers and physicians to make a critical appraisal of results.
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1. Introduction

The global burden of prostate cancer (PCa) rose from 200 000 new cases each year in 1975 to reach an estimated 700 000 new cases in 2002 [1]. In 2013, approximately 238 000 men in the Unites States will be diagnosed with PCa, and 30 000 will be expected to die from the disease [2].

Treatments for patients with localised disease include radical prostatectomy (RP), active surveillance, and radiation therapy (RT), while hormone therapy is typically used in patients with advanced disease [3]. All of these treatments are associated with specific side effects resulting in considerable impairment in several health-related quality of life (HRQOL) domains [3,4]. Thus, the inclusion of HRQOL assessment in randomised controlled trials (RCTs) testing different interventions for PCa is crucial for understanding which approach is best from the patient's perspective.

Patient-reported outcomes (PRO) data from RCTs, which include HRQOL and other health aspects reported by patients themselves [5], are increasingly used to inform patient-centred care as well as clinical and health policy decisions [6]. Thousands of PCa patients have been enrolled in RCTs with a PRO component [7], with the ultimate goal being to provide key information on overall treatment effectiveness. Some of these RCTs have generated high-quality PRO evidence and have formed the basis for approval of drugs based on patients' subjective reports [8]. For example, Tannock et al. [9] and Osoba et al. [8], comparing prednisone with or without mitoxantrone in symptomatic patients with hormone-resistant cancer, observed significantly better and lasting HRQOL outcomes for patients treated with mitoxantrone plus prednisone. Based on this RCT and specifically on patient-reported pain, the US Food and Drug Administration (FDA) subsequently granted approval of mitoxantrone [10].

However, the number of high-quality studies in PCa with such impact, facilitating individual patient decision making or treatment policies, is low [11]. Although inclusion of PRO into clinical comparative effectiveness research and drug development has been recommended to understand the patient experience [12,13], earlier work has shown a number of methodological drawbacks in PRO reporting from RCTs, including various cancer disease sites [14–16]. In a systematic review of studies published between 1980 and 2001, Efficace et al. showed that this was also the case for RCTs of PCa [7]. However, given the increasing interest of the scientific community and stakeholders in the use and application of PRO [17], it is of paramount importance to rely on the most solid and up-to-date evidence and identify which methodological aspects are most in need of improvement.

The main objective of this study was to investigate the methodological quality of PRO assessment in RCTs of PCa conducted since the previous survey. Secondary objectives were to estimate the likely impact of these studies on clinical decision making and to evaluate whether the standard of reporting has improved over time.

2. Evidence acquisition

2.1. Search strategy for identification of studies

A systematic literature search for studies meeting the criteria was undertaken on the electronic databases PubMed/Medline, the Cochrane Library, PsycINFO, and PsycARTICLES from January 2004 to March 2012. Relevant studies listed as references were also considered. The following script was used to identify all RCTs that had a PRO component: (''quality of life'' OR ''health related quality of life'' OR ''health status'' OR ''health outcomes'' OR ''patient outcomes'' OR depression OR anxiety OR emotional OR social OR psychosocial OR psychological OR distress OR ''social functioning'' OR ''social wellbeing'' OR ''patient reported symptom'' OR ''patient reported outcomes'' OR pain OR fatigue OR ''patient reported outcome'' OR PRO OR PROs OR HRQL OR QOL OR HRQOL OR ''symptom distress'' OR ''symptom burden'' OR ''symptom assessment'' OR ''functional status'' OR sexual OR functioning) AND prostate.

The search strategy for PubMed/Medline was restricted to RCTs. No restriction in the search field description was performed, and only English-language articles were considered. We also contacted experts in the field to identify possible articles not retrieved in the electronic search. Details on the search strategy and selection process were documented according to the Preferred Reporting Items for Systematic Reviews and Meta-analysis guidelines [18].

2.2. Criteria for considering studies

2.2.1. Types of participants

Adult patients diagnosed with PCa regardless of disease stage were included. Studies on patients with benign prostatic hyperplasia and those undergoing prevention or screening procedures were not eligible.

2.2.2. Types of intervention

Interventions included any RCTs comparing conventional treatments. Studies dealing with psychosocial interventions or complementary and alternative medicine were excluded.

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