Patient Perspective on Outcomes in Rheumatology — A Position Paper for OMERACT 9

JOHN R. KIRWAN, STANTON NEWMAN, PETER S. TUGWELL, and GEORGE A. WELLS

ABSTRACT. The inclusion of patient participants at OMERACT has resulted in direct incorporation of the patient perspective in the development of outcome assessments in rheumatology. Fatigue has been adopted as a recommended measure in all studies, and further work is now under way on the assessment of sleep, effective healthcare consumers, and the effects of psychological or educational interventions. This position paper draws this work together in preparation for the Patient Perspective Workshop at OMERACT 9, and introduces the concept that other core outcomes relevant to patients might be required in assessing interventions designed to help patients live with their disease. (J Rheumatol 2009;36:2067–70; doi:10.3899/jrheum.090359)

Key Indexing Terms:
OUTCOMES SLEEP CONSUMERS
PSYCHOLOGICAL EDUCATIONAL INTERVENTIONS

The OMERACT group (Outcome Measures in Rheumatology Clinical Trials) was, with others, instrumental in developing the internationally agreed "core set" of 7 outcomes to be assessed in clinical trials of treatment for rheumatoid arthritis (RA)[1]; and at OMERACT 5 in 2000, the meeting turned its attention to the scores in the core set measures required to be considered to have truly changed in response to treatment. There were many technical arguments, but perhaps the most important development was the recognition that taking a patient perspective was required[2].

At OMERACT 6, which followed in 2002, specific provision was made for patient participants. Attending the Patient Perspective Workshop were 11 patients from 7 countries, 5 organizing group members, and 41 other participants of the OMERACT 6 meeting. The workshop consisted of 3 formal sessions each of 2 hours, working group meetings between and after the formal sessions, and an unscheduled meeting of the patient participants[3]. One factor to emerge from the workshop was the clear message that other outcomes of importance to at least some patients include a sense of well-being, fatigue, and disturbed sleep. This stimulated new work on prevalence, experience, and measurement of fatigue in RA.

Much progress was made in several research areas identified by the Patient Perspective Workshop at OMERACT 6 when it came to reporting back to OMERACT 7 two years later[4]. Perhaps the greatest progress had been made on measuring fatigue[5]. By the time of the next conference, OMERACT 8 delegates were presented with a substantial body of evidence[6], and voted overwhelmingly that fatigue should be measured in future studies of RA[7]. This work illustrates the benefits of involving patients as partners in our research endeavors and highlights fatigue as an important outcome measure, an issue now being intensively investigated.

Other topics from the patient perspective research agenda on outcome assessment are also under active development; at OMERACT 9 the workshop concentrates on 3 of these: sleep disturbance in inflammatory arthritis; measuring the effectiveness of health consumers; and outcomes that are appropriate measures of the benefits of educational and psychological interventions. From the broad collection of topics identified at the OMERACT workshops has emerged the notion that there might be a "patient core set," to complement the traditional clinical core set of outcomes (particularly in relation to RA, where most of the OMERACT-related work has been undertaken). At OMERACT 9 we explore what might be encompassed by such a concept, and how we might measure its usefulness. The remainder of this article sets out the position in each of these areas.

SLEEP

Patient-reported outcomes (PRO) provide an assessment of a patient's health, well-being, and treatment from the patient's perspective. In particular, during the plenary voting at OMERACT 6 a research agenda was identified that incorporated the need for adequate measuring tools for sleep disturbance. The focus here is on the PRO of sleep quality in patients with RA. Individuals with a variety of common medical illnesses including arthritis frequently experience sleep

From the University of Bristol Academic Rheumatology Unit, Bristol Royal Infirmary, Bristol; University of London, Centre for Behavioural and Social Sciences in Medicine, London, UK; and the Institute of Population Health and the Department of Epidemiology and Community Medicine, University of Ottawa, Ottawa, Canada.

J.R. Kirwan, MD, University of Bristol Academic Rheumatology Unit, Bristol Royal Infirmary; S. Newman, PhD, University of London, Centre for Behavioural and Social Sciences in Medicine; P.S. Tugwell, MD, MSc, Institute of Population Health, University of Ottawa; G.A. Wells, PhD, Department of Epidemiology and Community Medicine, University of Ottawa.

Address correspondence to Dr. J.R. Kirwan, University of Bristol Academic Rheumatology Unit, Bristol Royal Infirmary, Bristol BS2 8HW, UK. E-mail: John.Kirwan@Bristol.ac.uk

disturbances. It is recognized that medical illnesses can adversely affect sleep quality, and that pain, infection, and inflammation can induce symptoms of excessive daytime sleepiness and fatigue. In particular, this is true for patients with RA. For properly assessing sleep for patients with RA, the availability, applicability, and responsiveness of measures of sleep quality in RA patients need to be evaluated.

The questions addressed in this section of the Patient Perspective Workshop at OMERACT 9 concern placing priorities on the different domains of sleep, determining sleep instruments that would be appropriate for RA patients using their psychometric properties, and choosing between a single item versus a summated scale for assessing sleep. These issues need to be considered in relation to the views of patients, clinicians, and researchers in the area, where differing opinions may exist related to these priorities, properties, and choices. This workshop will consider whether the patient's perspective might differ and how we might best understand the priorities that patients put on the domains and methods of assessment.

The first step in this process was undertaken in preparation for the OMERACT meeting, and was to conduct a systematic review of instruments assessing sleep quality following the methodology of the Cochrane Collaboration, and to identify domains related to sleep that are applicable to RA patients. Work completed to date includes a systematic review and an initial listing of sleep domains. In conducting the systematic review, the following steps were undertaken: a comprehensive literature search was conducted; citations and articles were selected using predefined criteria by 2 independent reviewers; information on the instruments was extracted from the articles using 2 independent reviewers; characteristics of the instruments including format, instructions, and psychometric properties were summarized; and a data synthesis and analysis was done, including subgroup analysis. In particular the various domains related to sleep that were assessed in the sleep instruments were identified and summarized, and their applicability to chronic diseases, specifically RA, was evaluated.

The literature review included Medline (1966 to Jan 2007), that yielded 3751 citations, and PsychINFO (1806 to Jan 2007) that yielded 174 citations for articles limited to tests and measures. In addition, Web-based databases (MAPPI Research Institute and ETS Test Collection), as well as sleep assessment textbook chapters, bibliographies of sleep research, and review articles were searched. We selected self-report instruments designed to assess sleep and sleep disorders in adults. With the exception of pain conditions we did not include scales that had been developed for specific disease populations.

The literature search yielded 57 instruments that assessed a variety of domains related to sleep, including: sleep initiation, sleep maintenance, sleep adequacy, somnolence, sleep-related behaviors, sleep-related cognitions and beliefs, "arousability" traits, physical environmental, sleep-related quality of life, daytime functioning, and health status. Various characteristics of the instruments were assessed and summarized, including the psychometric properties of the OMERACT filter of truth, discrimination, and feasibility, and measurement model properties such as the number of items, response format (most of the instruments had a Likert or visual analog scale), scoring system, timeline, and complexity varied from a single domain to multifactorial.

The next step in this process, which is a goal of this work at OMERACT 9, is a best matching of the priority domains with the sleep instruments deemed of high quality and acceptability from a psychometric perspective. That is, a number of domains related to sleep have been identified, and several sleep instruments have been reviewed that are applicable to RA. The Patient Perspective Workshop will lead to an understanding of sleep quality from the patient perspective and for the proper establishment of the acceptability and applicability of the domains and specific sleep instruments.

EFFECTIVE CONSUMER

Patients are increasingly being encouraged to participate in their own care[8-10] and to do so they will need to participate directly in the workings of their healthcare system. For example, a system of direct access to hospital specialist care in the UK requires patients to decide for themselves when they should attend specialist clinics[11]. Care has become more patient-centered, where the patients' needs and wants are addressed and considered. With this shift also has come a movement to empower and equip patients to manage their disease and to use healthcare resources effectively[12]. Consequently, programs and initiatives have been created to empower, educate, and provide information to consumers, sometimes known generically as "self-management interventions"[13].

Evaluating and comparing programs to create effective consumers capable of managing their care has been a challenge. There is an urgent need for scales to assess the ability of the patient to find and use health information, make and implement health decisions, communicate with others, and negotiate roles to participate in and manage health. No current validated outcome measure seems right to evaluate such programs.

An OMERACT Effective Consumer Scale to assess ability of patients to participate in and manage personal healthcare was developed through an iterative process of patient interviews, focus groups, and patient surveys undertaken in several countries[14]. The scale was analyzed using Item Response Theory methods. The current version of the Effective Consumer Scale asks 17 questions relevant to the skills outlined above relating to the ability to participate in and manage healthcare. Participants rate statements about knowledge, attitudes, and behaviors, noting how often the statements are true for them, i.e., "never" to "always." A total score is calculated

by adding the scores and converting to a percentage scale: the higher the score the more effective the consumer. Detailed information about the performance of the scale when used in the evaluation of an arthritis self-management program[15] will be made available at the workshop, and participants will consider the validity and areas of usefulness for the scale.

EDUCATIONAL AND PSYCHOLOGICAL INTERVENTIONS

Individuals with arthritis are faced with a range of different possible interventions, the mainstay of which are provided by the doctor or nurse and involve provision of medication. However, the different medications affect different aspects of the condition. Some are designed to directly treat the condition and have the potential to modify the disease, while others are designed to treat the symptoms. In addition, patients are offered potential treatments from other healthcare professionals such as physiotherapists, who may be involved in increasing mobilizing of joints or processes around joint protection, and occupational therapists, who are frequently concerned with issues of activities of daily living and use of various aids to assist in these activities to increase independence.

More recent developments in arthritis are psychoeducational interventions. These vary in objective and approach, but all build on psychological variables that have emerged as factors related to symptoms, functional status, and psychological well-being in arthritis[16].

However, educational interventions have not shown a substantial or sustained effect on clinical measures used to assess arthritis treated with medications[17]. Therefore, the question in this section is whether there are key outcomes that should be addressed in psychoeducational interventions in arthritis that may differ from those used in pharmacological studies. This issue needs to be considered in relation to the views of clinicians and researchers in the area, where differing opinions as to the value of these interventions seem to be related to which outcomes they are able to influence. Some feel that psychoeducational interventions only have a value if they have an effect on the same key outcomes that are influenced by pharmacological treatment. (However, many seem also to expect that psychoeducational treatments should continue working after the intervention is finished. This is a requirement seldom asked of a medicine, which, in general, is only expected to work while it is being administered.)

At the other extreme others are content if these interventions are able to influence psychological concepts such as self-efficacy. (In this scenario, it may be that there are no improvements in the primary symptoms of arthritis: pain, stiffness, and disability.) This workshop will consider whether the patient's perspective might differ from these extremes. It will also consider how we might best understand the priorities that patients put on the benefits of attending psychoeducational interventions and how those benefits might be measured.

POTENTIAL AND MEANING OF A "PATIENT CORE SET"

Patients and clinicians have different perspectives on outcomes in arthritis[3,4,6,18]. However, it is not clear whether these represent fundamentally different appreciations of the nature of the disease, or simply a different (but equivalent) way of assessing the surface outcomes derived from the underlying pathology of the disease. Clinicians have defined a "core set" of outcomes in several rheumatic diseases, including RA[1]. These are required in all studies of interventions in RA, but the increasing recognition of the differing perspective of patients and clinicians has raised the possibility of defining a "patient core set" with a similar imperative. However, it is not clear how such a concept would relate to the clinician's core set, nor how the area might be clarified. Three possible relationships are postulated, as follows; there may be more.

Possibility 1. Both the patient and the clinician core sets capture the essence of RA activity well. They are, in effect, 2 different ways of describing the same thing. They are 2 sides of the same coin. However, even in these circumstances it is likely that both the patient and the clinician core sets capture the essence of RA activity in only a fuzzy way. Some aspects of RA are not included (e.g., the Disease Activity Score[19] does not include fatigue), while some measures may capture other disease processes (e.g., pain may not be caused by inflammation). So there is a fuzzy overlap of the 2 sides of the coin, but the 2 core sets are roughly equivalent.

Possibility 2. The patient core set might be much less closely related to the underlying nature of RA. Patients might not see the measures in the same way as clinicians. Patients might want to include measures that capture a lot of non-RA symptoms, and hence their core set might be considered to represent some wider aspect of health.

Possibility 3. Patients are not really interested in describing RA as the consequence of a disease process. What they want is information about, for instance, the effects of RA on their life or, perhaps, they want to see outcomes such as better sleep without the anxiety and depression they might be suffering. Alternatively, perhaps, simply reducing unpredictable variations in disease would be a worthwhile outcome from a patient's perspective, even if the total extent of disease over time was not changed.

If we recognize and accept these possibilities, then those trying to develop a patient core set will need to distinguish between them. One part of the workshop will ask participants how we can distinguish these possibilities. Perhaps the use of qualitative studies with patients would clarify the situation. What statistical analyses of derived core sets would help to separate them? Could different core sets be used for different purposes? In which case, it must be questioned if they are really "core"? Perhaps the notion of a patient core set really rep-

resents a different way of expressing our existing outcomes (from the clinician core set measurements) in a way that is more useful to patients? We anticipate that consideration of these issues at the Workshop will drive forward the research agenda in these areas.

REFERENCES

1. Felson DT, Anderson JJ, Boers M, et al. The American College of Rheumatology preliminary core set of disease activity measures for rheumatoid arthritis clinical trials. Arthritis Rheum 1993;36:729-40.
2. Wells G, Anderson J, Beaton D, et al. Minimal clinically important difference module: summary, recommendations, and research agenda. J Rheumatol 2001;28:542-5.
3. Kirwan J, Heiberg T, Hewlett S, et al. Outcomes from the patient perspective workshop at OMERACT 6. J Rheumatol 2003;30:868-72.
4. Kirwan JR, Ahlmen M, de Wit M, et al. Progress since OMERACT 6 on including the patient perspective in rheumatoid arthritis outcome assessment. J Rheumatol 2005;32:2246-9.
5. Kirwan JR, Hewlett S, Heiberg T, et al. Incorporating the patient perspective into outcome assessment in rheumatoid arthritis — progress at OMERACT 7. J Rheumatol 2005;32:2250-6.
6. Kirwan J, Hewlett S. Patient perspective workshop: Reasons and methods for measuring fatigue in rheumatoid arthritis. J Rheumatol 2007;34:1171-3.
7. Kirwan J, Minnock P, Adebajo A, et al. Patient perspective workshop: Fatigue as a recommended patient-centred outcome measure in rheumatoid arthritis. J Rheumatol 2007;34:1174-7.
8. Department of Health. The expert patient: A new approach to chronic disease management for the 21st century. London: HM Stationery Office; 2001.
9. Holm S. Justifying patient self-management — evidence based medicine or the primacy of the first person perspective. Med Health Care Phil 2005;8:159-64.
10. Lorig KR, Sobel DS, Stewart AL, et al. Evidence suggesting that a chronic disease self-management program can improve health status while reducing hospitalization: a randomized trial. Med Care 1999;37:5-14.
11. Hewlett S, Kirwan J, Pollock J, et al. Patient-initiated outpatient follow-up in rheumatoid arthritis: six year randomised controlled trial. BMJ 2005;330:171-6.
12. Canadian Institutes of Health Research, Institute of Musculoskeletal Health and Arthritis, Annual Report 2003-2004. [Cited 2008 Jan. 15] Available from: www.cihr-irsc.gc.ca/f/ documents/imha_ annual_report_2003-2004.pdf.
13. Tugwell P, Santesso N, O'Connor A, Wilson AJ; Effective Consumer Investigative Group. Knowledge translation for effective consumers. Phys Ther 2007;87:1728-38.
14. Kristjansson E, Tugwell PS, Wilson AJ, et al. Development of the effective musculoskeletal consumer scale. J Rheumatol 2007;34:1392-400.
15. Santesso N, Rader T, Wells GA, et al. Responsiveness of the Effective Consumer Scale (EC-17). J Rheumatol 2009;36:2087-91.
16. Newman S, Steed L, Mulligan K. Self-management interventions for chronic illness. Lancet 2004;364:1523-37.
17. Riemsma RP, Taal E, Kirwan JR, Rasker JJ. Systematic review of rheumatoid arthritis patient education. Arthritis Care Res 2004;51:1045-59.
18. Hewlett S. Patients and clinicians have different perspectives on outcomes in arthritis. J Rheumatol 2003;30:87-9.
19. Prevoo ML, van't Hof MA, Kuper HH, van Leeuwen MA, van de Putte LB, van Riel PL. Modified disease activity scores that include twenty-eight-joint counts. Development and validation in a prospective longitudinal study of patients with rheumatoid arthritis. Arthritis Rheum 1995;38:44-8.