

Rheumatology Outcomes: The Patient's Perspective

ALISON CARR, SARAH HEWLETT, ROD HUGHES, HELENE MITCHELL, SARAH RYAN, MAGGIE CARR,
and JOHN KIRWAN

ABSTRACT. Our aim was to explore the patient's perspective of outcomes in rheumatoid arthritis (RA) to identify which outcomes are important to patients and how patients calibrate what constitutes a meaningful change in those outcomes. A qualitative study was performed using focus groups in 5 clinical centers in different geographical locations in the UK. Each group contained 6 to 9 patients with RA who were purposively sampled to include men and women with a range of age, disease duration, functional disability, work disability, and current disease activity. Each focus group lasted around 1 h and addressed 3 questions: What outcomes from treatment are important to RA patients? What makes patients satisfied or dissatisfied with a treatment? How do patients decide that a treatment is working? Patients identified as important not only physical outcomes such as pain and disability, but also fatigue and a general feeling of wellness. The relative importance of these outcomes depended on the stage of disease and on specific situations, such as a disease flare. Satisfaction was influenced by communication, access to treatment, and treatment efficacy. Treatment efficacy was related to symptom reduction, with the magnitude of reduction necessary for efficacy dependent on the stage of disease. For example, large changes were deemed necessary with disease of long duration, while in early disease, even small changes could be important. Our data support existing knowledge of the importance of pain and mobility as treatment outcomes, but raise new and important issues: Some outcomes of importance to patients are not currently measured and there are no measures available to capture them. Existing measures need to be calibrated to take account of the differing importance of outcomes at different stages of disease and variations in the magnitude of change within the same outcome that indicate treatment efficacy. (*J Rheumatol* 2003;30:880-3)

Key Indexing Terms:

RHEUMATOID ARTHRITIS
RANDOMIZED CONTROLLED TRIAL

OUTCOME AND PROCESS ASSESSMENT
PATIENT PARTICIPATION

Introduction

Patient-reported outcomes (of function^{1,2}, general health status^{3,4}, and quality of life^{5,6}) are increasingly used to supplement objective (clinical or biological) measures of disease in assessments of the quality of services, health care need, treatment effectiveness, and cost utility. This reflects a growing appreciation of the importance of how patients feel and how satisfied they are with treatment and with disease outcomes.

In this respect, patient measures are seen as ways of capturing patients' perspectives of their disease and treat-

ment, their perceived need for health care, and their preferences for treatment and disease outcomes⁷. They are hailed as being "patient-centered." But, although they are increasingly based on patients' definitions of outcome, they are used to determine the effectiveness of treatment in clinical trials without any idea as to which of the outcomes are the most important to patients, and without any patient-based calibration of what constitutes a meaningful change in these outcomes⁸⁻¹². For example, is pain or function the most important outcome of treatment with a disease modifying antirheumatic drug (DMARD)? What degree of pain is acceptable when there is a significant improvement in function and vice versa? How do patients titrate the experience of side effects against improvements in outcome when making judgments about treatment efficacy? Are there outcomes of great importance to patients that are not currently being measured at all?

Our study represents a first step towards answering some of those questions in a population of UK patients with rheumatoid arthritis (RA). The aim of this multicenter pilot study was to explore the patient's perspective of outcomes in RA by asking them to address 3 questions: What outcomes are important to RA patients? What makes patients satisfied or dissatisfied with a treatment? How do patients decide that a treatment is working?

From the University of Nottingham, Nottingham; University of Bristol, Bristol; Ashford and St Peter's Hospital Trust, Chertsey; Guy's, King's and St Thomas' School of Medicine, London; and Haywood Hospital, Stoke-on-Trent, UK.

A. Carr, PhD, Special Lecturer in Epidemiology, University of Nottingham, UK; S. Hewlett, PhD, ARC Senior Lecturer in Rheumatology (Health Professionals), University of Bristol; R. Hughes, MD, Consultant Rheumatologist, Ashford and St Peter's Hospital Trust; H. Mitchell, BSc, Research Assistant, Guy's, King's and St Thomas' School of Medicine; S. Ryan, PhD, Consultant Nurse Rheumatology, Haywood Hospital; M. Carr, MSc, Rheumatology Nurse Practitioner, Ashford and St Peter's Hospital Trust; J. Kirwan, MD, Reader in Rheumatology, University of Bristol.

Address reprint requests to Dr. A. Carr, Academic Rheumatology, University of Nottingham, Clinical Sciences Building, City Hospital, Nottingham, NG5 1PB, UK.

Methods

A qualitative study was performed using focus groups in 5 clinical centers in different UK locations: Bristol, London, Chertsey, Nottingham, and Stoke-on-Trent. Each group contained between 6 and 9 patients with RA who were purposively sampled from local populations of RA patients to include men and women and a range of age, disease duration, functional disability, work disability, and current disease activity. Characteristics of each of the groups are given in Table 1. The groups were facilitated by the authors in their local centers. Each group lasted around 1 hour and the discussions in each group were tape recorded and transcribed verbatim. The groups were asked to address 3 questions: (1) What outcomes from your treatments are important to you? (2) What makes you satisfied or dissatisfied with a treatment? (3) How do you decide that a treatment is working?

Interpretative phenomenological analysis (IPA) was used to analyze the data. IPA is a qualitative methodology that recognizes that examining the participants' experience (phenomenology) must be done through both the context in which it is related (the effect of the focus group) and the analysis of the researcher (i.e., their interpretation). Analysis followed the recommended 4 steps: (1) The transcripts were read and re-read to gain a general sense and potential themes arising from the data noted, informed by the researchers' knowledge of the focus group. (2) Upon re-reading, themes were systematically identified and organized into groups. (3) Themes were defined in more detail, and inter-relationships established. (4) Themes were organized into a report, giving the meaning of the patient's experience that is grounded in their own words.

Scientific rigor was established by inviting an independent qualitative researcher to examine the reports from each center, to see whether the themes identified were justified by the data. Finally, the researchers examined all 5 sets of analysis together for themes, providing a single, final report covering all centers.

Results

Patients. Characteristics of participating patients are given in Table 1.

Universal themes. In general there was strong concordance

of central themes across all 5 groups. The central themes for each of the 3 questions are given in Table 2.

Important outcomes. Important themes and issues arose from the focus group discussions that highlighted the complexity of measuring outcomes that are important to patients. These included the idea that different outcomes assume primary importance at different stages of the disease and in specific situations. For example, patients described how the relative importance of pain and mobility changed over time (pain most important in early disease and mobility/independence more important in later disease) and during acute flares of their arthritis (pain most important).

Patient 1: "In the early days it was pain [that was important] and it was getting rid of the pain."

Patient 2: "In acute attacks it is definitely reduction in pain that I am most interested in."

Patient 3: "We all start at the beginning wanting the pain to be eased a bit but then you get used to it ... your mobility goes and you want to get on and improve that."

Fatigue was consistently mentioned as an important outcome, and patients distinguished between tiredness and a complete, systemic fatigue that was related to their arthritis. This outcome was seen as important because it affected other outcomes (functional activities, work, social activities) and contributed to an overall sense of well-being.

Patient 4: "That's what you want the drugs to be able to, make it so that you're, so that they sort of compensate the tiredness."

Patient 5: "...you're not tired from work, you're just tired from the arthritis. I mean, you can sleep all night and you get up on a Saturday morning and just 'I don't want to do this' and you're just so tired. Not many people understand."

The concept of a general, overall feeling of wellness or well-being was often described as an important outcome, although what exactly it consisted of was not clear. Some patients described it in the context of a balance between symptom reduction or minimization and a lack of side effects, but it was not solely limited to this.

Patient 6: "What I wanted out of my treatment was this sense of well-being."

Patient 7: "...you know, I just want to feel more well in myself than I do and I just don't."

Another important outcome was a return to normality

Table 1. Participating patients in each focus group.

Center	N	Gender, M:F	Mean Age	Age, yrs (min-max)	Mean Disease Duration, yrs	Disease Duration, yrs (min-max)
Bristol	6	2:4	64	52-70	12	3-24
Chertsey	9	3:6	58	41-79	13	3-26
London	9	4:5	60	33-81	—	—
Nottingham	9	4:5	64	48-79	14	4-24
Stoke	6	3:3	58	51-64	9	2-20

Table 2. Universal themes for the 3 questions.

Question	Central Themes
What outcomes are important?	Physical (pain, disability, deformity) General well being (fatigue, feeling well) Independence Return to normality Emotional impact Fear of the future The relative importance of outcomes changes over time and depending on circumstances
What makes you satisfied or dissatisfied with treatment?	Treatment efficacy Side effects Patient-health professional communication Access to care
How do you decide that treatment is working?	Symptom reduction "Forgetting you have RA" Change in priorities for outcome over time Magnitude of improvement/change varies with disease duration

described either by reference to a peer group without arthritis, or to the patient's own level of pre-arthritis activity or lifestyle.

The issue of side effects was raised in relation to outcome, with the idea that side effects were to some extent titrated against outcome, i.e., side effects above a certain threshold would negate the positive effects of treatment on other outcomes, such as a reduction in pain or increase in mobility. One report suggested that side effects were evidence that a treatment was working.

Patient 8: "What you find with a lot of the drugs and the treatment, you lose your personality, you lose yourself as you used to be. Alright, the physical self's not good, but I think I would prefer to be a little immobile, take as much of the drug as I can, and to go on to try and get more success when I reach a certain point, rather than make me feel ill."

Satisfaction and dissatisfaction with treatment. Patients reported issues around communication, access to treatment, and treatment efficacy as major influences on satisfaction with treatment. These results are interesting but not central to the debate on outcome measurement and will be explored further elsewhere. One interesting finding was the reported frustration with the inability of doctors to find the one treatment that would be effective for an individual patient, without having to try all the alternatives.

Patient 9: "There seemed to be an awful lot of guesswork with the medication and somebody who likes to adopt a clinical approach to most things, I was quite surprised that after all the time that rheumatoid arthritis has been around they didn't have a narrow field to work in. It was, that one didn't work so try that one, try that one, try that one; and the side effects and early treatment of the arthritis were very dissatisfying for me."

Decisions about treatment efficacy. As expected, many of

the decisions about treatment efficacy were based on symptom modification (reduction in pain, increase in function and mobility, return to activities). However, some patients also raised the issue of how much of a change in each outcome would be necessary for them to consider that the treatment was working. These patients indicated that the magnitude of the change necessary for efficacy would differ depending on the stage of the disease and would be complicated by the natural variability of the disease. For example, patients felt that with longer disease duration, larger changes were necessary to assume importance, whereas in early disease, even small changes were important.

Patient 10: "It's very hard trying to gauge how severe the inflammation is because it's so erratic from day to day, so when you do go on a new treatment, unless you do get immediate relief or relief within a day, it's very hard to gauge whether that treatment's working because you have good days and bad days so it could be that you're experiencing a good day."

Patient 11: "I notice small changes, you know I can notice if like one finger hurts more or doesn't hurt at all ...I do actually think, though, that it is important for me to notice the small changes, because if one or two slip by, a year down the line you've lost the use of your knee, or you, you know, you don't walk how you used to. So I think it is important."

Patient 4: "I think maybe as the arthritis progresses, and it sadly gets worse with a lot of people, it may be like D was saying, you don't recognize the small changes so much and you don't think they are as important, I mean, just because you've got used to putting up with aches and pains generally, you know."

Summary

The data from these 5 focus groups support some existing

knowledge on patient-based outcomes (that pain and mobility are very important) but raise some new and important issues:

- Some outcomes of importance to patients may not currently be measured in assessments of treatment efficacy
- For some of these outcomes, there may be validated outcome measures available, but for others our understanding of the meaning of the outcome and how to quantify it are either absent or are very rudimentary (for example, assessment of wellness or well-being, or return to normality)
- The challenge for outcome assessment is how to calibrate new and existing measures to take account of (a) variations in the weighting of importance of different outcomes at different stages of disease and in different circumstances (e.g., when pain is more important than mobility and vice versa) and (b) variations in the magnitude of change within the same outcome that is considered evidence of treatment efficacy at different stages of disease (e.g., small vs large changes in pain, depending on whether it is early or late disease).

REFERENCES

1. Fries JF, Spitz P, Kraines RG, Holman HR. Measurement of patient outcome in arthritis. *Arthritis Rheum* 1980;23:137-45.
2. Meenan RF, Gertman PM, Mason JM. Measuring health status in arthritis: the Arthritis Impact Measurement Scale. *Arthritis Rheum* 1980;23:146-52.
3. Euroqol Group. Euroqol EQ-5D User Guide. Rotterdam: Rotterdam Centre for Health Policy and Law, Erasmus University; 1996.
4. Ware JE, Sherbourne CD. The MOS 36-item Short-form Health Survey (SF-36) I. Conceptual framework and item selection. *Med Care* 1992;30:473-83.
5. O'Boyle CA, McGee H, Hickey A, O'Malley K, Joyce CRB. Individual quality of life in patients undergoing hip replacement. *Lancet* 1992;339:1088-91.
6. De Jong Z, van der Heijde D, McKenna SP, Whalley D. The reliability and construct validity of the RAQoL: a rheumatoid arthritis-specific quality of life instrument. *Br J Rheumatol* 1997;36:878-83.
7. Carr AJ, Higginson I. Measuring quality of life: Are quality of life measures patient-centred? *BMJ* 2001;322:1357-60.
8. Kwoh K, Feinstein A, Gifford R, et al. Physician-patient agreement on patients' priorities for important treatment outcomes [abstract]. *Arthritis Rheum* 1987;30 Suppl:S101.
9. Daltroy L, Eaton H, Hashimoto H, Liang MH. Rheumatoid arthritis patients' expectations for treatment outcomes [abstract]. *Arthritis Rheum* 1995;38 Suppl:S310.
10. Woolhead GM, Carr AJ, Wilkinson M, Bannister GC, Ackroyd CE. Expectations of treatment and outcome in patients awaiting joint replacement surgery and general orthopaedic outpatients [abstract]. *Arthritis Rheum* 1996;39 Suppl:S174.
11. Mitchell H, Stowers K, Hughes RA, Carr AJ. Patients' self-assessment of disease activity and severity in rheumatoid arthritis [abstract]. *Arthritis Rheum* 1999;42 Suppl:S274.
12. Carr AJ, Hughes RA, Stowers K. What influences placebo response to a complementary therapy, glucosamine sulphate? [abstract]. *Rheumatology* 2000;36:S238.