Patient centred outcomes in osteoarthritis

J W J Bijlsma

The introduction of minimal clinically important improvement and patient acceptable symptom state is a major advance in outcome research

Osteoarthritis (OA) is the most common joint disorder in the world; it occurs in the majority of people over the age of 65 and is an important cause of (work) disability. The public health impact of OA is immense and will further increase with the growing elderly population. Therefore, the search for, and evaluation of, treatments for OA are relevant research topics, not only for the medical community, including individual patients and doctors, but also for pharmaceutical companies and health economists. Assessment of present and future treatment modalities in OA is important and requires a balanced approach.

OUTCOME ASSESSMENT
Outcome assessment is the measurement process whereby the consequences of disease and health management interventions are evaluated. Outcome measures may be divided into two broad categories: observer dependent (or assessor rated) and observer independent (or self rated). In general, observer independent clinical measures are based on self administered questionnaires, whereas observer dependent clinical measures include questionnaires scored by the interviewer, physical findings, and tests of performance rated on technical instruments, such as walking time.

Many physicians tend to value assessor rated outcome measures higher than self rated outcome measures. However, this interpretation is obsolete and does not allow consideration of the patients’ perspectives. In line with the OMERACT recommendations, most studies in OA use clinical outcome measures of pain, function, and patient global assessment (all self rated) and, only for studies over 1 year, imaging (assessor rated). Clinical trials in OA assess short term changes in symptoms and long term changes in structural improvement. As yet, no definite proof of structural improvement has been documented in OA research.

PATIENTS’ PERSPECTIVES
The sequential OMERACT conferences have boosted the science of clinical measurement to generate new knowledge and to develop consensus on the use of methodology in rheumatology, first focusing on rheumatoid arthritis (RA), but more recently also, on other rheumatic diseases, including OA. As a new development at the 6th conference, in Australia in 2002, the incorporation of patient perspectives in arthritis measurement research was introduced. This major step was based on the recognition that outcome assessment, and in turn, treatment decisions, vary greatly among healthcare providers.

"Outcome assessment and treatment decisions vary greatly among healthcare providers"

"Good" was the "gold standard". For instance, patients with knee OA consider themselves clinically improved if the decrease in pain exceeds 19.9 mm on the 0–100 mm visual analogue scale (VAS). It is based on the patient’s opinion as an external anchor and contrasts changes within patients at the individual level (proportion of improved patients) instead of at the group level (mean change in a variable).

To calculate this score patients own assessment of their response on a five point Likert scale at the final visit. The timeframe has been arbitrarily set at 4 weeks; the MCI probably will be different at 2 or 6 weeks and the timeframe will have implications for the use of the MCI. In the French study 399 rheumatologists included 1362 patients, fewer than four patients for each doctor, probably biasing the sample towards a population with more severe OA. This is mirrored in the high scores on the VAS for pain, patient global assessment, and WOMAC function score. Therefore, we cannot from this particular study conclude that 20 mm improvement in VAS pain in a patient with knee OA is always clinically relevant; we need to validate these results in other patient populations.

In addition, probably not only the described actual decrease but also the decrease below a certain level will be relevant. Improvement of pain of 20 mm on a 0–100 mm VAS, will be differently experienced by a patient who started with a pain score of 60 mm at baseline in comparison with a patient...
with a baseline VAS score of 30 mm. Therefore, a combination of change in disease activity (pain, function) as well as the level of current disease activity is useful, as rheumatologists now are using in RA. To be classified as a responder, patients with RA should have a significant change in Disease Activity Score as well as low current disease activity. When we try to target our treatment aims in OA: improvement of 20 mm on a 0–100 mm VAS as well an actual level below 20 mm on the VAS should be reached before we might define a response as “good”.

“Treatments targets should combine change in disease activity with a maximum level of disease activity”

In the study on MCII in OA different values are given for knee and hip OA, suggesting that smaller improvements in pain, patient global assessment, and WOMAC function are meaningful in hip OA than in knee OA. This is a relevant point to note, but difficult to interpret; a perhaps too easy interpretation is that hip OA is more bothersome then knee OA. The authors also show in their report that absolute and relative changes in MCII vary, based on baseline scores; the consequence of this observation could be an adaptation of the treatment target to a combination of change in disease activity (for example, pain) with a maximum level of disease activity, as described above.

Patient acceptable symptom state

The study on the patient acceptable symptom state (PASS) takes the issue one step further and really puts the patient in the centre. The PASS is defined based on the wellbeing of the patient: “feeling good”. The PASS is more relevant to the patient: when a patient has a relevant MCII but does not feel good, the target of the treatment has not been reached. The “gold standard” in the PASS evaluation is the answer a patient gives at the end of the treatment period to the following question: “Taking into account all activities you have during your daily life, your level of pain, and also your functional impairment, do you consider that your current state is satisfactory?” Fifty eight per cent of patients with knee OA and 50% with hip OA answered “yes” to this question. Based on this answer, scores on VAS pain, patient global assessment, and on WOMAC functional score could be evaluated to calculate the PASS; the PASS for hip OA was slightly higher then for knee OA (all levels of pain, patient global assessment, and function score around 33).

The PASS is by definition an absolute value and reflects the situation at one particular moment, it does not reflect a change, as does the MCII. The PASS was shown to depend on the starting values of pain, patient global assessment and, especially, WOMAC function score. Therefore, the combination of change and actual level should be evaluated in the interpretation of PASS as well.

Other factors that might influence the PASS, such as age, sex, and disease duration, have been evaluated, but did not modify the PASS estimates. However, because the gold standard “feeling good” might be influenced by psychological factors as well, an evaluation of the possible influences of depression, coping styles, and so on, will be relevant.

SUMMARY

The introduction of the minimal clinically important improvement, and of the patient acceptable symptom state is a major step forward in outcome research in OA. Before we can use this new set of measurements in daily practice, it is evident that these instruments need further validation and perhaps modification. The knowledge thus obtained will certainly help us to better understand and appreciate findings from OA research, but in the end, for the patient it all comes down to the most relevant questions: Do you feel good? Do you feel improved? Is this treatment worthwhile?

ACKNOWLEDGEMENT

I thank my colleagues J W Jacobs and S M Verstappen for their comments on this editorial.

REFERENCES

Patient centred outcomes in osteoarthritis

J W J Bijlsma

doi: 10.1136/ard.2004.025072

Updated information and services can be found at:
http://ard.bmj.com/content/64/1/1

These include:

References
This article cites 9 articles, 5 of which you can access for free at:
http://ard.bmj.com/content/64/1/1#BIBL

Email alerting service
Receive free email alerts when new articles cite this article. Sign up in the box at the top right corner of the online article.

Topic Collections
Articles on similar topics can be found in the following collections

Degenerative joint disease (4641)
Musculoskeletal syndromes (4951)
Osteoarthritis (931)
Connective tissue disease (4253)
Rheumatoid arthritis (3258)
Epidemiology (1367)
Immunology (including allergy) (5144)
Pain (neurology) (883)

Notes

To request permissions go to:
http://group.bmj.com/group/rights-licensing/permissions

To order reprints go to:
http://journals.bmj.com/cgi/reprintform

To subscribe to BMJ go to:
http://group.bmj.com/subscribe/