

Patient-Reported Outcomes in Rheumatoid Arthritis: Why are They Important and How Should They Be Assessed?

Romatoid Artrit'te Hasta İlişkili Sonuç Parametreleri: Neden Önemli ve Nasıl Değerlendirmeli?

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Abstract

Patient reported outcomes have become increasingly important in the evaluation of rheumatoid arthritis (RA) over recent years. Besides pain and functional capacity, fatigue, sleep and well-being are also important for RA patients. We will discuss here the importance of patient-reported outcomes, different domains of health, and how to evaluate them, with a focus on questionnaires available in Turkey.

(*Turk J Rheumatol 2010; 25: 99-104*)

Key words: Rheumatoid arthritis, outcome measure, response criteria, fatigue, pain

Received: 20.06.2010

Accepted: 05.07.2010

Özet

Son yıllarda romatoid artrit (RA) değerlendirilmesinde hasta ilişkili sonuç parametreleri giderek daha fazla önem kazanmıştır. Ağrı ve fonksiyonel kapasite haricinde yorgunluk, uyku ve iyilik de RA'lı hastalar için önemlidir. Bu derlemede hasta ilişkili sonuç parametreleri ve sağlığın farklı alanlarının önemini, nasıl değerlendirileceğini Türkiye'de kullanılan anketlere odaklanarak tartışacağız. (*Turk J Rheumatol 2010; 25: 99-104*)

Anahtar sözcükler: Romatoid artrit, sonuç parametresi, cevap kriterleri, yorgunluk, ağrı

Alındığı Tarih: 20.06.2010

Kabul Tarihi: 05.07.2010

Introduction

Rheumatoid arthritis (RA) is traditionally assessed by physical examination by a physician, by laboratory tests and radiographs, in keeping with a "biomedical model," the dominant paradigm of 20th-century medicine. However, since the start of the new millennium, there has been growing interest in assessment of RA from the patient's perspective (1).

In this article, we will discuss the importance of patient reported outcomes (PROs), the different domains

or dimensions of health important for patients with RA, and the questionnaires available to assess PROs.

1. Why should we assess PROs ?

There are several arguments in favour of assessing PROs.

A. Assessing the patients' perspective

For the purpose of preventing joint destruction in RA, it is important to be able to detect inflammation, i.e. synovitis and acute phase reactants since these elements

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doi: 10.5152/tjr.2010.11

seem closely correlated to further bone erosions (2). However, it is not sufficient to monitor these objective elements reflecting inflammation. Indeed, RA is also a disease which leads to a considerable burden of disease for patients, i.e. to symptoms such as pain and functional disability. Since the final objective of treatment is in fact better health-related quality of life (3), monitoring patients' symptoms is necessary in RA, as it is in other chronic diseases which impact quality of life, if we want to be able to assess the efficacy of our treatments. This is particularly important since the patient's perspective on outcomes is different from the physician's perspective, in RA (Figure 1).

B. PROs bring interesting and valuable data

They have no cost and are non invasive. They have good psychometric properties: some PROs have been found to be as informative as joint counts, radiographic and laboratory data for the assessment of baseline status, change during interventions, and are predictive of long-term outcomes (4-6). This is particularly true for the Health Assessment Questionnaire, HAQ.

C. In clinical trials

Pros give us the patients' assessment of efficacy of treatment, which is important when taking therapeutic decisions. Assessment of PROs is also mandatory for the obtention of drug licensing (for the Food and Drug Administration). Finally, assessment of quality of life (using generic instruments) allows comparisons across diseases, necessary for economic analyses.

D. In clinical practice

Of course, assessing PROs during an outpatient clinic takes time. However, we still believe it is important to assess PROs. Assessing patient global allows the calculation of the disease activity score (DAS); we suggest that a visual analog scale (VAS) for pain and fatigue should also be assessed, as well as morning stiffness. By such a standardised assessment, we obtain the patients' assessment of efficacy of treatment, and we can positively influence the patient-physician relationship since the patient feels listened to. We do not suggest that fatigue will influence disease-modifying decisions, but other therapeutic modalities (physical therapy, anti-depressant drugs ...) can be prescribed if needed.

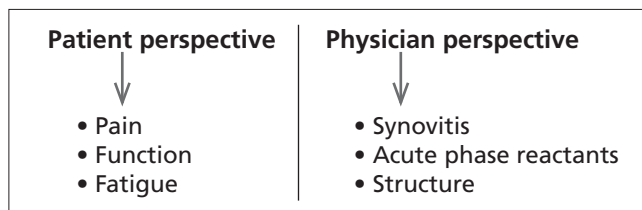


Figure 1. Illustration of the different perspectives in RA

2. Different PROs in RA

A. Some PROs are frequently assessed

Since 1993, assessment of the activity of RA has become standardised, after the elaboration of a 'Core Set' of domains or dimensions which have become mandatory to report in trials. The 'Core Set' is used both in Europe and in the United States since it is recognised both by the American College of Rheumatology (ACR) (7, 8) and the European League Against Rheumatism (EULAR) (9), (Table 1). In the 'Core Set', 3 elements pertain to the patients' perspective; these elements are pain, functional capacity and patient global assessment. These elements are usually reported in trials, as shown by Dr Kalyoncu from Ankara (10): more than half of the studies recently published in RA, reported these outcomes.

B. Other PROs are rarely assessed

Several publications issued from patient group discussions (11, 12) or patient focus groups (13) indicate that some domains or areas of health which are important for patients are unrecognised and underestimated in RA. These domains include, among others, fatigue (11, 13, 14), well-being (11, 13, 14), sleep patterns (11), work incapacity (13) or return to normal

Table 1. The 'Core Set' in RA

Painful joint count
Synovial joint count
Acute phase reactants
Global assessment (doctor)
Pain (assessed by patient)
Global assessment (patient)
Functional capacity (patient)

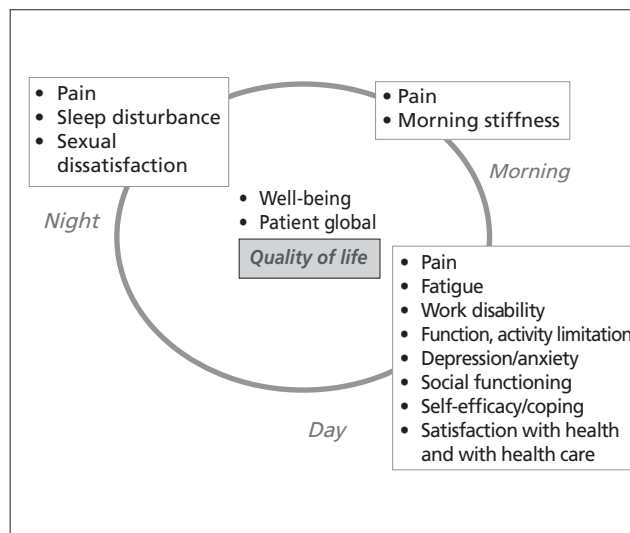


Figure 2. Domains of health which are important in RA

life (13, 14) and independence (i.e. being able to manage daily activities such as personal hygiene) (13). PROs in RA are synthesized Figure 2. Most of the domains important for patients are rarely reported in publications (10).

3. Description of some of the domains of health in PROs (15)

A. Pain, functional capacity and patient global assessment

Pain and functional disability are part of the RA Core Set (7) and are regularly cited by people with RA as important (11-16).

Pain VAS is considered as the gold standard to assess pain (17). Because of its simplicity, VAS is a useful method of assessment for PROs. However, there are some limits to the use of VAS. Elderly persons, low-literacy populations, and some cultural groups have difficulties conceptualizing a VAS. In these cases, numerical rating scales may be a useful alternative. Giving the patient the opportunity to rate himself in comparison to a previous rating may also be helpful. An important element is that a VAS or numerical rating scale is very quick and easy to apply and can also be used as part of daily clinical practice.

The HAQ and the shorter, modified-HAQ are easy to administer self-questionnaires which comprise 8 categories of functioning: dressing, rising, eating, walking, hygiene, reach, grip, and usual activities (18,19).

Global assessment is usually assessed with a VAS but the wording varies widely (10).

B. Fatigue

Fatigue was poorly recognised before 2003 (11). Since that date, several qualitative studies (13,14) have pointed out the importance of fatigue for patients with RA; fatigue is intrusive and overwhelming in RA according to patients, and has consequences on all aspects of quality of life.

Fatigue VAS is a single-item scale. It measures the severity of fatigue over the past week with a specific question (20). Fatigue VAS is simple and reproducible, and the validated wording is included in Table 2.

C. Other domains

Other domains reported in the literature as important include well-being, sleep disturbance, coping, social life, professional status (ability to work) and satisfaction with health care (11-14, 16).

Morning stiffness is often assessed, reflecting the inflammatory component of pain, but is not highly sensitive to change.

3. Multi-domain assessments

A. Quality of life

Health-related quality of life assesses in fact several aspects of RA. It is frequently assessed by the SF36 (21). Composite scores which are patient reported include the patient activity scale (PAS) (22), the Routine Assessment of Patient Index Data 3 (RAPID3) (23) et the recently published European League Against Rheumatism tool, the Rheumatoid Arthritis Impact of Disease (RAID) (1). The first 2 criteria integrate the 3 domains usually assessed in RA, namely, pain, function and patient global.

B. The RAID

Under the aegis of EULAR, the European League Against Rheumatism, an international task force elaborated a new composite response score for clinical trials in RA, based on the patients' perception of the impact of the disease on domains of health: the patient-derived preliminary RA Impact of Disease (RAID) score (1). The score includes 7 domains prioritised by patients. The domains of highest importance are pain, functional disability, and fatigue; the 4 other domains are emotional and physical well-being, sleep disturbance and coping (Table 2). The RAID has been elaborated and validated with the participation of Turkey (Dr. Gogus and Dr. Gunendi) therefore this score can be used in Turkish (Table 3). The RAID is viewed as an additional instrument for the assessment of RA, giving supplementary information on patient-relevant domains.

Conclusion

In conclusion, assessment of PROs is increasingly important in RA. PROs capture information which is relevant for the patients although their value for treatment-modifying decisions remains to be established. Fatigue should be taken into account in RA and treatments which are efficacious for RA-related fatigue should be assessed.

However, further work is needed in PROs: which ones are of greatest interest according to the underlying cultural background, how to find the validated questionnaires, prognostic value of PROs, cutoffs defining patient acceptable symptom states and clinically relevant improvement, PROs in other diseases. The European League Against Rheumatism is planning to finance future studies in PROs in the next years, so we may obtain some of these answers soon!

Conflict of Interest

No conflict of interest is declared by author.

Table 2. The RAID score, a composite score to capture impact of RA (1)

RAID questionnaire

1. Pain

Circle the number that best describes the pain you felt due to your rheumatoid arthritis during the last week:

None	0	1	2	3	4	5	6	7	8	9	10	Extreme
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2. Functional disability assessment

Circle the number that best describes the difficulty you had in doing daily physical activities due to your rheumatoid arthritis during the last week.

No difficulty	0	1	2	3	4	5	6	7	8	9	10	Extreme difficulty
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3. Fatigue

Circle the number that best describes how much fatigue you felt due to your rheumatoid arthritis during the last week.

No fatigue	0	1	2	3	4	5	6	7	8	9	10	Totally exhausted
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4. Sleep

Circle the number that best describes the sleep difficulties (i.e., resting at night) you felt due to your rheumatoid arthritis during the last week.

No difficulty	0	1	2	3	4	5	6	7	8	9	10	Extreme difficulty
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5. Physical well-being

Considering your arthritis overall, how would you rate your level of physical well being during the past week? Circle the number that best describes your level of physical well-being.

Very good	0	1	2	3	4	5	6	7	8	9	10	Very bad
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6. Emotional well-being

Considering your arthritis overall, how would you rate your level of emotional well being during the past week? Circle the number that best describes your level of emotional well-being.

Very good	0	1	2	3	4	5	6	7	8	9	10	Very bad
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7. Coping

Considering your arthritis overall, how well did you cope (manage, deal, make do) with your disease during the last week?

Very well	0	1	2	3	4	5	6	7	8	9	10	Very poorly
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RAID SCORING AND CALCULATION RULES

The RAID is calculated based on 7 Numerical rating scales (NRS) questions. Each NRS is assessed as a number between 0 and 10. The 7 NRS correspond to pain, function, fatigue, sleep, emotional well-being, physical well-being, and coping/self-efficacy.

1. Calculation

RAID final value =

$$(\text{pain NRS value (range 0-10)} \times 0.21) + (\text{function NRS value (range 0-10)} \times 0.16) + (\text{fatigue NRS value (range 0-10)} \times 0.15) + (\text{phys well being NRS value (range 0-10)} \times 0.12) + (\text{sleep NRS value (range 0-10)} \times 0.12) + (\text{emotional well being NRS value (range 0-10)} \times 0.12) + (\text{coping NRS value (range 0-10)} \times 0.12).$$

Thus, the range of the final RAID value is 0-10 where higher figures indicate worse status

2. Missing data imputation

If one of the 7 NRS values composing the RAID is missing, the imputation is as follows:

- a. calculate the mean value of the 6 other (non-missing) NRS (range, 0-10)
- b. impute this value for the missing NRS
- c. Then, calculate the RAID as explained above

If 2 or more of the NRS are missing, the RAID is considered as missing value (no imputation)

Table 3. The RAID is validated in Turkish (1)**1. Ağrı**

Geçen hafta romatoid artritimize bağlı hissettiğiniz ağrıyı en iyi tarif eden rakamı yuvarlak içine alınız.

Yok	0	1	2	3	4	5	6	7	8	9	10	Aşırı
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2. İşlevsel özürlülük değerlendirimi

Geçen hafta romatoid artritimize bağlı günlük fiziksel aktivitelerinizi yapmakta çektiğiniz güçlüğü en iyi tarif eden rakamı yuvarlak içine alınız.

Zorluk yok	0	1	2	3	4	5	6	7	8	9	10	Aşırı zorluk
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3. Yorgunluk

Geçen hafta romatoid artritimize bağlı ne kadar yorgunluk hissettiğinizi en iyi tarif eden rakamı yuvarlak içine alınız.

Yorgunluk yok	0	1	2	3	4	5	6	7	8	9	10	Tamamen bitap düşmüş
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4. Uyku

Geçen hafta romatoid artritimize bağlı hissettiğiniz uyku güçlüklerini (ör. gece dinlenirken) en iyi tarif eden rakamı yuvarlak içine alınız.

Zorluk yok	0	1	2	3	4	5	6	7	8	9	10	Aşırı zorluk
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5. Fiziksel iyilik

Genel olarak hastalığınızı göz önüne aldığınızda, geçen haftaki fiziksel iyilik halinizi nasıl değerlendirirsiniz? Fiziksel iyilik halinizi en iyi tarif eden rakamı yuvarlak içine alınız.

Cok iyi	0	1	2	3	4	5	6	7	8	9	10	Cok kötü
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6. Duygusal iyilik

Genel olarak hastalığınızı göz önüne aldığınızda geçen haftaki duygusal iyilik halinizi nasıl değerlendirirsiniz? Duygusal iyilik halinizi en iyi tarif eden rakamı yuvarlak içine alınız.

Cok iyi	0	1	2	3	4	5	6	7	8	9	10	Cok kötü
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7. Başa çıkabilme

Genel olarak hastalığınızı göz önüne aldığınızda geçen hafta hastalığınızla nasıl başa çıkabildiniz? (idare etmek, dayanmak)

Cok kötü	0	1	2	3	4	5	6	7	8	9	10	Cok kötü
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