Review

Introducing new tools for assessment of parent- and child-reported outcomes in paediatric rheumatology practice: a work in progress

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ABSTRACT

It is increasingly recognised that regular assessment of parent- and child-reported outcomes (PCROs) in routine paediatric rheumatology practice may help to increase the quality of care of children with rheumatic diseases. However, most of the instruments available for assessment of PCROs have remained essentially research tools and are not routinely administered in most centres. Recently, new multidimensional questionnaires for paediatric rheumatic diseases have been devised. These tools have been specifically designed for regular administration in a busy clinical setting and have the advantage over other clinical measures of incorporating all main PCROs in a single instrument. This review describes briefly the multidimensional questionnaires developed for the assessment of PCROs in children with juvenile idiopathic arthritis, juvenile dermatomyositis, and juvenile systemic lupus erythematosus and discusses the rationale underlying their creation. Furthermore, it illustrates the methodology and benefits related to the use of multidimensional questionnaires in the collection of standardised quantitative data.

Introduction

In recent years, there has been a growing interest in the assessment of parent- and child-reported outcomes (PCROs) in paediatric rheumatic diseases (PRDs) (1-4). Integration of these measures in the clinical evaluation is considered important as they reflect the parent's and child's perception of the disease course and effectiveness of therapeutic interventions. Because parents and children (when mature enough to understand the clinical and therapeutic issues related to their disease) are asked with increasing frequency to actively participate in shared decision-making, integration of their perspective in clinical assessment may facilitate concordance with physician's choices and improve adherence to treatment (5-7). In addition, the use of PCROs may help the physician to identify with greater accuracy the salient issues for each patient and to focus the attention on the relevant matters. Thus, information obtained from the parent or the child may contribute to the success of patient care. It is now agreed that the inclusion of PCROs in clinical practice may lead to improve the quality of care (8).

A number of tools for the assessment of PCROs in PRDs are available, including visual analogue scales (VAS) for rating of child's overall well-being and intensity of pain, and questionnaires for the evaluation of functional ability and health-related quality of life (HRQOL) (1, 2, 4, 9). These clinical measures have been included in a multitude of observational studies, clinical trials, and long-term outcome surveys. Some of them are part of standardised core sets of outcome measures, disease activity state definitions, or composite disease activity scores for juvenile idiopathic arthritis (JIA) (10-13), juvenile dermatomyositis (JDM) (14, 15), and juvenile systemic lupus erythematosus (JSLE) (16).

However, in spite of their popularity and widespread use, most of the instruments used to assess PCROs have remained essentially research tools and are not routinely administered in most paediatric rheumatology centres. One of the reasons that may explain why these evaluations are uncommonly performed in daily clinical care is the length and complexity of some questionnaires. There is the concern that

Table I. Example of patient flow sheet monitoring obtained using the JAMAR. This patient had a good initial response to intra-articular corticosteroid therapy and methotrexate, but experienced a severe disease flare in May 2010. After the start of etanercept, disease remission was achieved in March 2011.

	04/08/2009	14/09/2009	01/02/2010	07/06/2010	05/07/2010	04/11/2010	10/03/2011	29/09/2011
Parent proxy-reported questionnaire data								
Overall well-being (0-10)	8	0.5	5	9	1.5	3	0	0
Pain (0-10)	8	0.5	5	8	1	0.5	0	0
Disease activity (0-10)	8.5	0.5	6.5	8.5	2	1	0	0
Physical function (0-45)	4	0	2	3	1	1	0	0
Health-related quality of life (0-30)	12	3	7	13	6	6	2	1
Physical health (0-15)	11	3	3	10	2	2	0	0
Pyschosocial health (0-15)	1	0	4	3	4	4	2	1
Morning stiffness	30min - 1hr	No	30min-1hr	30min-1hr	No	No	No	No
Disease course	–	Much improved	Unchanged	Unchanged	Much improved	Much improved	Slightly improved	Unchanged
Disease status	–	Remission	Persistent activity	Persistent activity	Remission	Remission	Remission	Remission
Satisfaction with illness outcome	No	Yes	No	No	Yes	Yes	Yes	Yes
Physician-reported outcomes								
Physician's global assessment (0-10)	7.5	3.5	9	9.5	6	0.5	0	0
Active joint count (0-73)	6	2	10	14	4	1	0	0
Restricted joint count (0-67)	4	1	10	13	0	1	0	0
Laboratory data								
Erythrocyte sedimentation rate (mm/h)	44	24	32	39	9	20	8	11
C-reactive protein (mg/dl)	1.00	<0.46	1.53	2.89	1.20	<0.46	<0.46	<0.46
Composite scores and therapeutic response								
JADAS10 (0-40)	23.9	6.4	25.2	34.4	11.5	4.5	0	0
JAPAI4 (0-40)	32	4	19	33	9.5	10.5	2	1
ACR Pediatric response	–	70%	Non responder	Non responder	70%	70%	100%	100%
Drug therapy								
Methotrexate	Yes	Yes	Yes	Yes	Yes	Yes	Yes	–
Etanercept	–	–	–	Yes	Yes	Yes	Yes	Yes
Prednisone	–	–	Yes	Yes	–	–	–	–
Intra-articular corticosteroid injection	Yes (5 joints)	–	–	–	–	–	–	–

JAMAR: Juvenile Arthritis Multidimensional Assessment Report; JADAS: Juvenile Arthritis Disease Activity Score; JAPAI: Juvenile Arthritis Parent Assessment Index; ACR: American College of Rheumatology.

their regular administration may interfere with office routine and time management, with consequent increased costs and time.

On the other hand, the heterogeneous and multidimensional nature of PRDs implies that numerous disease domains should be evaluated simultaneously to appraise the full extent of the illness. In this respect, there are several PCROs not addressed by conventional instruments, such as evaluation of morning stiffness and overall level of disease activity, rating of disease status and course, proxy- or self-assessment of joint involvement and extra-articular symptoms, description of side effects of medications, and assessment of therapeutic compliance and satisfaction with the outcome of the illness, which may provide valuable insights into the influence of the disease and its treatment on child's health.

These considerations have provided the rationale for the development of new multidimensional questionnaires for the assessment of patients with PRDs in standard clinical care that incorporate all main PCROs. The purpose of this review is to describe briefly these tools and to highlight their potential utility in day-to-day practice.

Juvenile Arthritis Multidimensional Assessment Report (JAMAR)

The multidimensional questionnaire for JIA has been the first to be developed (17). It is a 4-page tool that incorporates 15 PCROs. Of these items, 10 have been previously validated and 5 are descriptive in nature. The JAMAR includes quantitative measures of physical function, pain, disease activity, overall well-being, and HRQL. In addition, questionnaire completers are asked to assess articular and extra-articular symptoms, morning stiffness, disease status and course, medication side effects, compliance with prescribed therapy, problems at school, and satisfaction with illness outcome. Overall, the JAMAR addresses all domains included in the WHO International Classification of Functioning and Health (ICF) (7). Data obtained from the JAMAR enables the calculation of 2 novel composite scores entirely based on PRCOs: the Juvenile Arthritis Parent Assessment Index (JAPAI) and the Juvenile Arthritis Child Assessment Index (JACAI) (18).

The JAMAR is available in a parent proxy-reported version for ages 2–18 years and in a child self-report version

for ages 7–18. The questionnaire format proved to be user-friendly, easy to understand, and readily answered by parents and children. Completion of the questionnaire takes <15 min and scoring takes <5 min. The JAMAR was tested in 618 Italian children with JIA and was found to perform well in characterising differences in level of disease activity and severity (17).

The JAMAR has been selected for the assessment of PCROs in a multinational study aimed to investigate the EPidemiology, treatment and Outcome of Childhood Arthritis throughout the world (EPOCA Study) (19). For the purposes of this study, the JAMAR has been or is currently being translated and cross-culturally adapted and validated in 38 national languages. One of the main objectives of the EPOCA Study is to promote regular use of quantitative clinical measures and incorporation of PCROs in routine paediatric rheumatology practice.

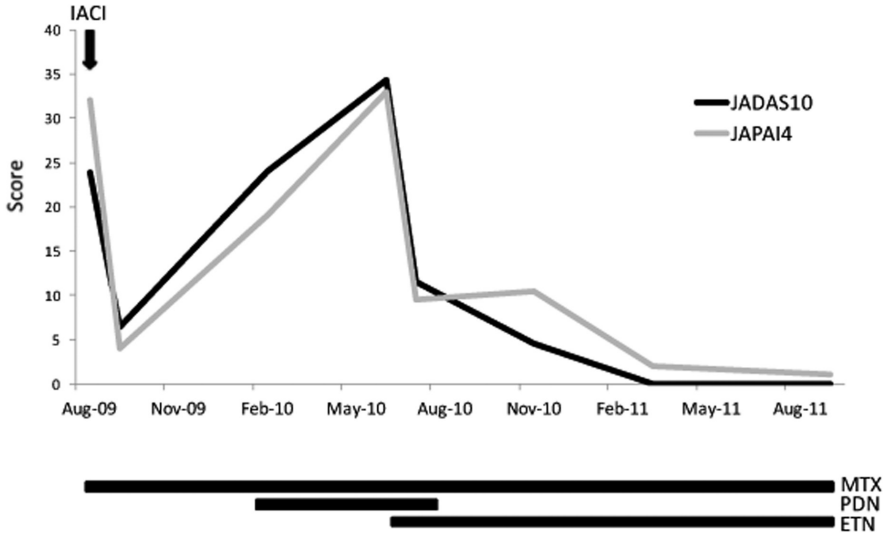

Fig. 1. Time course of composite scores, along with therapeutic interventions, derived from the flow sheet reported in Table I. JADAS: Juvenile Arthritis Disease Activity Score; JAPAI: Juvenile Arthritis Parent Assessment Index; IACI: intraarticular corticosteroid injection; MTX: methotrexate; PDN: prednisone; ETN: etanercept.

Juvenile Dermatomyositis Multi-dimensional Assessment Report (JDMAR) and Juvenile Systemic Lupus Erythematosus Multi-dimensional Assessment Report (JSLEMAR)

The multidimensional questionnaires for JDM and JSLE have been modelled on the JAMAR. Their format is, therefore, identical, and many items are equal. However, the JDMAR includes a different physical function tool, which is specific for JDM and is in progress of separate validation (20). In addition, it contains, beside the 3 visual analogue scales (VAS) for rating of pain, disease activity and overall well-being, a fourth VAS for assessment of fatigue. The evaluation of morning stiffness is omitted and the section devoted to articular and extra-articular manifestations is replaced with a checklist of the most common symptoms of JDM. Likewise, assessment of treatment toxicity is focused on the medications most frequently administered to children with JDM. The JDMAR is currently in progress of validation in the context of a multinational collaborative effort.

The JSLEMAR is very similar to the JDMAR. However, the sections devoted to disease symptoms and drug toxicity list the characteristic clinical manifestations of JSLE and the most typical side effects of lupus medications, respectively. Furthermore, physical function assessment is not included. The JSLEMAR is still work in progress, but its validation is planned to be started soon.

Multidimensional questionnaires for other paediatric illnesses

The underlying principles of multidimensional questionnaires have recently drawn the attention of paediatric rheumatologists with interest in other systemic disorders. This has generated ongoing initiatives aimed to devise versions devoted to systemic vasculitides and autoinflammatory diseases (21, 22). In perspective, the same model might also prove attractive to paediatricians dealing with non-rheumatologic chronic illnesses or conditions, such as diabetes, asthma, inflammatory bowel diseases, chronic renal failure, muscular dystrophies, haemophilia, cystic fibrosis, obesity, and others.

Conclusions

The development of the multidimensional questionnaires has introduced a new approach to clinical care of children with PRDs. Through administration of these questionnaires, information related to patient history may be collected as standardised quantitative data. This data, combined with physician-reported measures and laboratory tests, may be used to guide therapeutic choices and to monitor patient status over time. Use of questionnaires is important because they address the primary concerns of children and their parents.

The multidimensional questionnaires have been specifically designed for regular administration in a busy clinical setting, with particular attention to feasibility and acceptability in daily care. They are completed in the waiting area before the patient is called into an examining room. Almost of the work is done by the parent or the patient, not the physician or the staff, and the physician should spend only a few seconds reviewing and scoring the data. The best strategy to assure completion of questionnaires at each visit is for the receptionist or a nurse to distribute a questionnaire at the time of patient registration for the visit. Importantly, by involving the nurses in the help activity, they can be motivated to gain a more important role within the team.

Completion of the questionnaires helps the parent and the patient to focus on information needed for care and enhances their capacity to describe concerns in the limited time allotted for a clinical encounter. Availability of the data to the physician at the time of the visit is

helpful, particularly in view of the importance of the information provided by parents and patients in clinical decisions. The use of questionnaires requires a change in clinic procedure, which may be seen as added complexity. However, their administration not only supports management decisions and improves documentations, but may also save time (after a brief "learning curve," as required with any new activity) (23).

Regular use of the multidimensional questionnaires enables keeping a flow sheet of patient's course over time. A flow sheet may facilitate the recognition of possible changes in clinical symptoms, functional capacity, pain, overall well-being, fatigue, and psychological status from previous visits. This method of handling clinical data appears very useful in the management of chronic diseases such as PRDs, as it allows the clinician to record serial parent/patient data, together with physician-reported outcomes, laboratory tests, medication regimen, and other information (Table I) (24). Composite scores computed with flow sheet data can be plotted in a graph to provide an overview at a glance of the patient's course over time, which is a cost-effective procedure (Fig. 1).

Nowadays, computer-based utilities, such as office-based touchscreen computers, telephone-based interactive voice response systems, handheld computers, and mobile phones, are emerging to address workflow challenges in obtaining, aggregating, calculating, and displaying data in real time, as well as minimising response errors. A computer-based touchscreen questionnaire process offers advantages over paper-based process in facilitating the collection of PCROs, ensuring reliability and validity (*e.g.* error checking) of the data capture, and simplifying the effective use of the data (25). By means of this technology, the questionnaires are automatically scored and results are stored in a database. Information can be presented in real time electronically with summary scores from previous encounter. Development of electronic versions of questionnaires on touchscreen handheld devices is in progress at the senior author's unit.

As is the case with new assessment tools, there are some caveats with the multidimensional questionnaires. These instruments may not provide sufficient detail regarding PCROs of sleep disturbance, coping, and family life. Furthermore, it has been argued that their current format may not fully elicit some subjective elements of patient history, particularly those determined by patient's own values (7). Further development of the questionnaires requires continuing research, with introduction of possible modifications based on clinical experience and emerging concepts. We acknowledge that our work owes a great deal to previous work of Dr Ted Pincus on development and validation of multidimensional questionnaires for adult patients with rheumatoid arthritis (26).

In conclusion, the multidimensional questionnaires provide a promising approach to quantitative measurement in paediatric rheumatology care. These tools allow for greater focus on issues important to parents or patients, and may be used effectively to guide management, document change in health status, and assess outcomes. Regular administration of the new questionnaires is helpful to implement treat-to-target strategies in standard clinical settings (27, 28). Thus, availability of these instruments may foster regular assessment of PCROs in routine practice and contribute to improve the quality of care of children with PRDs.

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References

1. DUFFY CM: Measurement of health status, functional status, and quality of life in children with juvenile idiopathic arthritis: clinical science for the pediatrician. *Pediatr Clin North Am* 2005; 52: 359-72.
2. BRUNNER HI, GIANNINI EH: Health-related quality of life in children with rheumatic diseases. *Curr Opin Rheumatol* 2003; 15: 602-12.
3. FELDMAN BM, GRUNDLAND B, MCCULLOUGH L, WRIGHT V: Distinction of quality of life, health related quality of life, and health status in children referred for rheumatologic care. *J Rheumatol* 2000; 27: 226-33.
4. BRUNNER HI, RAVELLI A: Developing outcome measures for paediatric rheumatic diseases. *Best Pract Res Clin Rheumatol* 2009; 23: 609-24.
5. GARCIA-MUNITIS P, BANDEIRA M, PISTORIO A *et al*.: Level of agreement between children, parents, and physicians in rating pain intensity in juvenile idiopathic arthritis. *Arthritis Rheum* 2006; 55: 177-83.
6. CONSOLARO A, VITALE R, PISTORIO A *et al*.: Physicians' and parents' ratings of inactive disease are frequently discordant in juvenile idiopathic arthritis. *J Rheumatol* 2007; 34: 1773-6.
7. LUCA N, FELDMAN BM: Pediatric rheumatology: Improving the assessment of children with JIA. *Nat Rev Rheumatol* 2011; 7: 442-4.
8. BERARD R, LAXER RM: Improving the quality of care in children with juvenile idiopathic arthritis: a step in the right direction. *J Rheumatol* 2011; 38: 789-90.
9. FILOCAMO G, CONSOLARO A, SOLARI N *et al*.: Recent advances in quantitative assessment of juvenile idiopathic arthritis. *Ann Paediatr Rheum* 2012; 1: 84-96.
10. GIANNINI EH, RUPERTO N, RAVELLI A, LOVELL DJ, FELSON DT, MARTINI A: Preliminary definition of improvement in juvenile arthritis. *Arthritis Rheum* 1997; 40: 1202-9.
11. WALLACE CA, GIANNINI EH, HUANG B, ITERT L, RUPERTO N: American College of Rheumatology provisional criteria for defining clinical inactive disease in select categories of juvenile idiopathic arthritis. *Arthritis Care Res* (Hoboken) 2011; 63: 929-36.
12. MAGNI-MANZONI S, RUPERTO N, PISTORIO A *et al*.: Development and validation of a preliminary definition of minimal disease activity in patients with juvenile idiopathic arthritis. *Arthritis Rheum* 2008; 59: 1120-7.
13. CONSOLARO A, RUPERTO N, BAZSO A *et al*.: Development and validation of a composite disease activity score for juvenile idiopathic arthritis. *Arthritis Rheum* 2009; 61: 658-66.
14. MILLER FW, RIDER LG, CHUNG YL *et al*.: Proposed preliminary core set measures for disease outcome assessment in adult and juvenile idiopathic inflammatory myopathies. *Rheumatology* (Oxford) 2001; 40: 1262-73.
15. RUPERTO N, RAVELLI A, PISTORIO A *et al*.: The provisional Paediatric Rheumatology International Trials Organisation/American College of Rheumatology/European League Against Rheumatism Disease activity core set for the evaluation of response to therapy in juvenile dermatomyositis: a prospective validation study. *Arthritis Rheum* 2008; 59: 4-13.
16. RUPERTO N, RAVELLI A, CUTTICA R *et al*.: The Pediatric Rheumatology International Trials Organization criteria for the evaluation of response to therapy in juvenile systemic lupus erythematosus: prospective validation of the disease activity core set. *Arthritis Rheum* 2005; 52: 2854-64.
17. FILOCAMO G, CONSOLARO A, SCHIAPPAPIETRA B *et al*.: A new approach to clinical care of juvenile idiopathic arthritis: the Juvenile Arthritis Multidimensional Assessment Report. *J Rheumatol* 2011; 38: 938-53.
18. CONSOLARO A, RUPERTO N, PISTORIO A *et al*.: Development and initial validation of composite parent- and child-centered disease

assessment indices for juvenile idiopathic arthritis. *Arthritis Care Res* (Hoboken) 2011; 63: 1262-70.
19. CONSOLARO A, RUPERTO N, FILOCAMO G *et al*.: Seeking insights into the EPidemiology, treatment and Outcome of Childhood Arthritis through a multinational collaborative effort: Introduction of the EPOCA study. *Pediatr Rheumatol Online J* 2012; 10: 39.
20. FERRARI C, CONSOLARO A, VARNIER GC *et al*.: Comparison of construct validity of two functional ability measures in children with juvenile dermatomyositis. *Ann Rheum Dis* 2012; 71(Suppl. 3): 427.
21. FIDANCI BE, ACIKEL C, FIDANCI K, YILDIZ D, KARAMAN D, DEMIRKAYA E: Validity and reliability: to use in pediatrics. *Ann Paediatr Rheum* 2012; 1: 147-55.
22. FIDANCI BE, DEMIRKAYA E, ACIKEL C *et al*.: The invisible part of the iceberg: qualitative aspects of childhood vasculitis. *Clin Exp Rheumatol* 2013 (in press).
23. PINCUS T, SOKKA T: Complexities in the quantitative assessment of patients with rheumatic diseases in clinical trials and clinical care. *Clin Exp Rheumatol* 2005; 23 (Suppl. 39): S1-S9.
24. PINCUS T, MANDELIN AM, SWEARINGEN CJ: Flowsheets that include MDHAQ physical function, pain, global, and RAPID3 scores, laboratory tests, and medications to monitor patients with all rheumatic diseases: an electronic database for an electronic medical record. *Rheum Dis Clin North Am* 2009; 35: 829-xi.
25. NEWMAN ED, LERCH V, JONES JB, STEWART W: Touchscreen questionnaire patient data collection in rheumatology practice: development of a highly successful system using process redesign. *Arthritis Care Res* (Hoboken) 2012; 64: 589-96.
26. PINCUS T, ASKANASE AD, SWEARINGEN CJ: A multi-dimensional health assessment questionnaire (MDHAQ) and routine assessment of patient index data (RAPID3) scores are informative in patients with all rheumatic diseases. *Rheum Dis Clin North Am* 2009; 35: 819-27.
27. CONSOLARO A, NEGRO G, LANNI S, SOLARI N, MARTINI A, RAVELLI A: Toward a treat-to-target approach in the management of juvenile idiopathic arthritis. *Clin Exp Rheumatol* 2013; 30 (Suppl. 73): S157-S172.
28. ANINK J, DOLMAN KM, MERLIJN VAN DER BERG J, VAN VEENENDAAL M, KUIJPERS TW, VAN ROSSUM MAJ: Two-year outcome of juvenile idiopathic arthritis in current daily practice: what can we tell our patients? *Clin Exp Rheumatol* 2012; 30: 972-8.