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Patients' Experience of Myositis and Further Validation of a Myositis-specific Patient Reported Outcome Measure — Establishing Core Domains and Expanding Patient Input on Clinical Assessment in Myositis. Report from OMERACT 12

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ABSTRACT. ***Objective.*** The Outcome Measures in Rheumatology (OMERACT) myositis working group was established to examine patient-reported outcomes (PRO) as well as to validate patient-reported outcome measures (PROM) in myositis.

Methods. Qualitative studies using focus group interviews and cognitive debriefing of the myositis-specific Myositis Activities Profile (MAP) were used to explore the experience of adults living with polymyositis (PM) and dermatomyositis (DM).

Results. Preliminary results underscore the importance of patient input in the development of PROM to ensure content validity. Results from multicenter focus groups indicate the range of symptoms experienced including pain, fatigue, and impaired cognitive function, which are not currently assessed in myositis. Preliminary cognitive debriefing of the MAP indicated that while content was deemed relevant and important, several activities were not included; and that questionnaire construction and wording may benefit from revision. A research agenda was developed to continue work toward optimizing PRO assessment in myositis with 2 work streams. The first would continue to conduct and analyze focus groups until saturation in the thematic analysis was achieved to develop a framework that encompassed the patient-relevant aspects of myositis. The second would continue cognitive debriefing of the MAP to identify potential areas for revision. There was agreement that further work would be needed for inclusion body myositis and juvenile dermatomyositis, and that the inclusion of additional contributors such as caregivers and individuals from the pharmaceutical/regulatory spheres would be desirable.

Conclusions. The currently used PROM do not assess symptoms or the effects of disease that are most important to patients; this emphasizes the necessity of patient involvement. Our work provides concrete examples for PRO identification. (First Release May 1 2015; J Rheumatol 2015;42:2492–5; doi:10.3899/jrheum.141243)

Key Indexing Terms:
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The idiopathic inflammatory myopathies (IIM) are a group of rare heterogeneous systemic inflammatory conditions clinically characterized by muscle weakness and reduced muscle endurance, limiting activities of daily living and lowering the health-related quality of life (HRQOL)[1,2,3,4,5].

Patient-reported outcome measures (PROM) are critical tools to use in clinical practice and research settings to evaluate treatment effects in the domains of most importance to the patient. However, many clinical assessment tools used in the rheumatic diseases were developed with limited or no patient input, and existing PROM have not been thoroughly evaluated through the lens of patients. There has been minimal research done to establish those aspects of myositis most important to patients. The Outcome Measures in Rheumatology (OMERACT) Myositis Working Group was established to address these gaps.

The International Myositis Assessment and Clinical Studies Group (IMACS) has proposed a consensus definition of a clinically important improvement of disease activity[6], including clinician-reported and patient-reported outcomes. The OMERACT myositis working group has brought together the expertise and knowledge of multidisciplinary healthcare providers, qualitative methodologists, and of critical importance, 2 myositis patient research partners (PRP; USA and Sweden) to apply OMERACT methodology in developing and/or validating PROM for myositis.

At OMERACT 11 (May 2012), the Myositis Working Group reviewed PROM used in IIM, demonstrating that most instruments were generic measures to evaluate physical function and HRQOL[7] across diseases. Among only a few disease-specific instruments, the Myositis Activities Profile (MAP) is the only specific measure for adult polymyositis (PM) and dermatomyositis (DM)[7].

Items in the MAP were selected from the International Classification of Impairments, Disability and Handicap to reflect domains of health[8] selected by the myositis researchers. A first draft was presented to 10 Swedish patients with DM/PM, who rated importance and difficulty in performing the different activities[8]. A final version of the MAP contained 31 questions divided into 4 subscales and 4 single items (scored separately), asking patients to consider both difficulty and importance in their assessment[8]. At OMERACT 11 we evaluated the MAP through the OMERACT filter 1.0 of Truth, Discrimination, and Feasibility, concluding that the final questionnaire required additional content validation to ensure it accurately reflected the patient experience of disease.

At the same meeting, preliminary results were presented from a US focus group to investigate which domains patients with PM and DM consider important to evaluate[7].

A research agenda was established to conduct additional qualitative studies in myositis patients representing at a minimum Europe, North America, and Asia, as well as taking the MAP back to patients to further evaluate its content validity. The current report describes data presented at OMERACT 12 (May 2014) and our future research agenda in accordance with the OMERACT Filter 2.0 in the core area of Life Impact, domain Patients' Perception of Health. The specific aims were to (1) investigate patients' experience of living with myositis to define core domains to be assessed in IIM, and (2) conduct further content validation of the MAP through cognitive debriefing to understand patients' thoughts and beliefs of questionnaire items and structure.

MATERIALS AND METHODS

Patients' experience of living with myositis. Focus groups were conducted using a semistructured interview guide that was developed based on discussions of working group members at OMERACT 11 and the American College of Rheumatology 2013 meetings. Two general questions were asked: "In what way has myositis changed your life?" and "Can you describe a typical day in your life?" as well as asking at the end about domains that were not reflected in the overall discussion.

Face and content validity of the MAP. The one-on-one "think aloud" cognitive debriefing interview method was used, where patients are asked to voice their reasoning as they completed each item in the MAP questionnaire[9].

Focus groups and interviews were audiotaped, transcribed, and analyzed using systematic text condensation (STC), identifying categories and subcategories[10].

RESULTS

The OMERACT 12 Myositis Working Group meeting. To provide important context of the patient's perspective, 2 PRP shared their experiences of living with myositis as well as their experiences of medical care, participation in research, and the importance of support from other myositis patients. Both PRP have had myositis for several years with flares and periods with more stable disease activity. Side effects of medications such as type 2 diabetes and cardiovascular disease also affected quality of life. Exercise was described as an important part of treatment. Following this, preliminary results from our ongoing qualitative studies were presented.

Patients' experience of living with myositis. To date, 6 focus groups have been conducted (USA = 2, Sweden = 2, and South Korea = 2). Altogether 36 (Sweden, n = 11; USA, n = 12; South Korea, n = 13) patients with adult PM or DM were included. Transcripts from Sweden were fully analyzed according to STC, while US and South Korean transcripts were evaluated only using the first steps of the STC. Five categories emerged from these preliminary analyses: Symptoms; Activity Participation; Strategies and Recovery; Knowledge of Disease and Self-management; and Emotional Factors.

The informants described the category Symptoms related to their muscles, lungs and skin, emphasizing their experience of pain, stiffness and discomfort, fatigue, insomnia, symptom variations, and cognitive dysfunction, as well as limitations in daily activities and participation in society. The category Strategies included examples akin to the category self-manage-

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ment that has emerged in OMERACT qualitative activities in other diseases[11]. Finally, a category of Emotional Factors was identified describing grief, sadness, depressive moods, and anxiety, but also resilience, coping, and acceptance.

During the following group discussion our PRP confirmed these categories as important aspects of myositis. To reach thematic saturation, it was agreed by attendees that additional focus groups should include patients from various age groups and both sexes, and patients from different practice settings. Methodological discussions focused on the process of forward and backward translation, and consistency of qualitative analysis.

Face and content validity of the MAP. Preliminary results were presented based on interviews with 7 informants with PM and DM from Sweden. Five different categories emerged concerning the questionnaire itself: Questionnaire Structure and Grading; Relevance of Content; Vagueness of Items; Two Questions in One; and Missing Concepts and Activities. Informants described difficulties in providing a grade incorporating both difficulty with and the importance of an individual activity. While content was relevant to most informants, a number of activities were identified as not included, and it was difficult to answer about activities that they did not normally perform. There was also a lack of precision in many items.

During the discussion the following points were made: The OMERACT 12 Myositis Working Group agreed that the "think aloud" methodology for questionnaire evaluation was an appropriate approach. The working group attendees encouraged us to perform additional interviews with patients from different age groups and areas of residence, and both women and men. The English version of the MAP should be cognitively debriefed in a similar manner to confirm the Swedish findings.

Finally, the OMERACT 12 Myositis Working Group endorsed our approach to date and voted affirmatively that we are on target regarding our efforts.

DISCUSSION

OMERACT Filter 2.0 and identifying domains. Prior efforts to identify relevant domains in myositis have focused on physical dimensions such as muscle weakness. Our qualitative studies are in line with the recommendations in the OMERACT filter 2.0 to address patients' perception of health. The results highlight the importance to patients of other symptoms including pain and fatigue as well as the effect on mental health. Finally, these studies bring out the effect of myositis on relationships. These additional symptoms and effects have not been comprehensively evaluated in myositis patients, highlighting the need for further research in the area. Further, the continuing emphasis on pain as an important symptom indicates the striking disconnect between the physician's understanding of IIM as "painless muscle weakness" and the patient's experience of the condition.

The ongoing focus groups are the first step in the OMERACT Filter 2.0 process to establish domains that are of importance to adult patients with PM and DM. Additional focus groups are planned. Educational level is an important aspect that needs to be addressed when identifying focus group informants. Individual interviews may be needed to ensure thematic saturation. Additional studies are needed including healthcare providers, caregivers, and other stakeholders such as pharmaceutical/regulatory representatives to identify domains they consider important to measure. To reconcile domains important to patients and others involved, further Delphi activities are planned to identify preliminary core domains and then to construct reflective PROM in adult PM and DM. Our PRP will be instrumental throughout this process. However challenging, it will be very important to consider all aspects of systemic involvement in myositis. In fact, efforts could be facilitated by learning from results of other OMERACT workshops. For example, interstitial lung disease (ILD) is a common feature in myositis, and core domains proposed by the OMERACT connective tissue disease–ILD group[12] could be used also for myositis in ILD-specific clinical trials. We aim to develop/validate PROM covering myositis-relevant World Health Organization International Classification of Functioning, Disability and Health components Activity and Participation[13], according to the OMERACT filter 2.0 recommendation.

The diversity of symptoms experienced by patients with myositis calls for disease-specific measures. There is still a need to use generic PROM in studies aiming at comparing aspects of health over several health conditions and also in myositis trials until disease-specific patient-driven validated PROM are available.

Bringing the MAP back to patients. The MAP was developed to evaluate limitations in activities related to myositis. Our objective was to reevaluate the content validity of the current measure to inform the development of a revised version of the MAP. Our debriefing has thus far established that, while many relevant activities are covered, there are several limitations, highlighting the importance of cognitive debriefing and revision to ensure that the MAP appropriately captures the concept of measurement in ways that are understandable and relevant to patients. The new version of MAP will be the first PROM that has been developed based on patients' perceptions of activity and participation but will need further validation to fully meet the OMERACT filters 1.0 and 2.0. The think-aloud methodology has been used to capture the concept of thoughts and understanding of items to develop or adapt PROM, for example, in rheumatoid arthritis and juvenile idiopathic arthritis[14,15], but to our knowledge, our study is the first to use this method in evaluating a myositis PROM.

Our PRP will have a very important role in the development and wording of the new patient-driven MAP by continuously giving input at all steps of the validation process

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along with coauthorship. The new MAP will be tested using Rasch methodology to optimize subscales and scoring, and to eliminate item redundancy.

Leading up to OMERACT 13, we also plan to continue with expansion of focus groups with PM or DM and caregivers. Further we plan to expand focus groups to include patients with inclusion body myositis and juvenile DM, as well as their caregivers, in this qualitative multicenter study.

The results presented at OMERACT 12 by the myositis working group emphasize the necessity of patient involvement in identifying what is important to measure as a starting point for developing PROM. Through qualitative exploration, patient language provides the experiential framework from which relevant and prioritized aspects of a disease can be identified. Patients can also provide input and guidance to determine whether a questionnaire can evaluate what is actually experienced by someone living with a chronic health condition. Our work provides concrete examples for other groups who are interested in PROM development.

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