Patient involvement in developing core outcome sets. An OMERACT case study.

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Amsterdam, November 16, 2018


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taking pictures allowed

Overview

- Introducing OMERACT and PPIE in Core outcome set development according to the OMERACT filter 2.1
- Patient involvement throughout the process of selecting Psoriatic Arthritis core domains - Step 1 (WHAT)
- Patient involvement throughout the process of selecting Psoriatic Arthritis core instruments - Step 2 (HOW)
- Conclusions

COMET Initiative

OMERACT Outcome Measures in Rheumatology

OMERACT (Outcome Measures in Rheumatology)

Concerted effort for validation and consensus

- *Started 1990, outcome measures in rheumatoid arthritis for clinical trials*
- *Extended to other rheumatic diseases*
- Develop core outcome set, through literature search, validation, consensus
- International conference every 2 years
  - (vote, endorse, measures to carry forward)
- Participants:

  Rheumatologists, methodologist, administrators (including FDA), industrial sponsors,

  Patient representatives (since 2002)

Filter 1 –

Truth, Discrimination and Feasibility

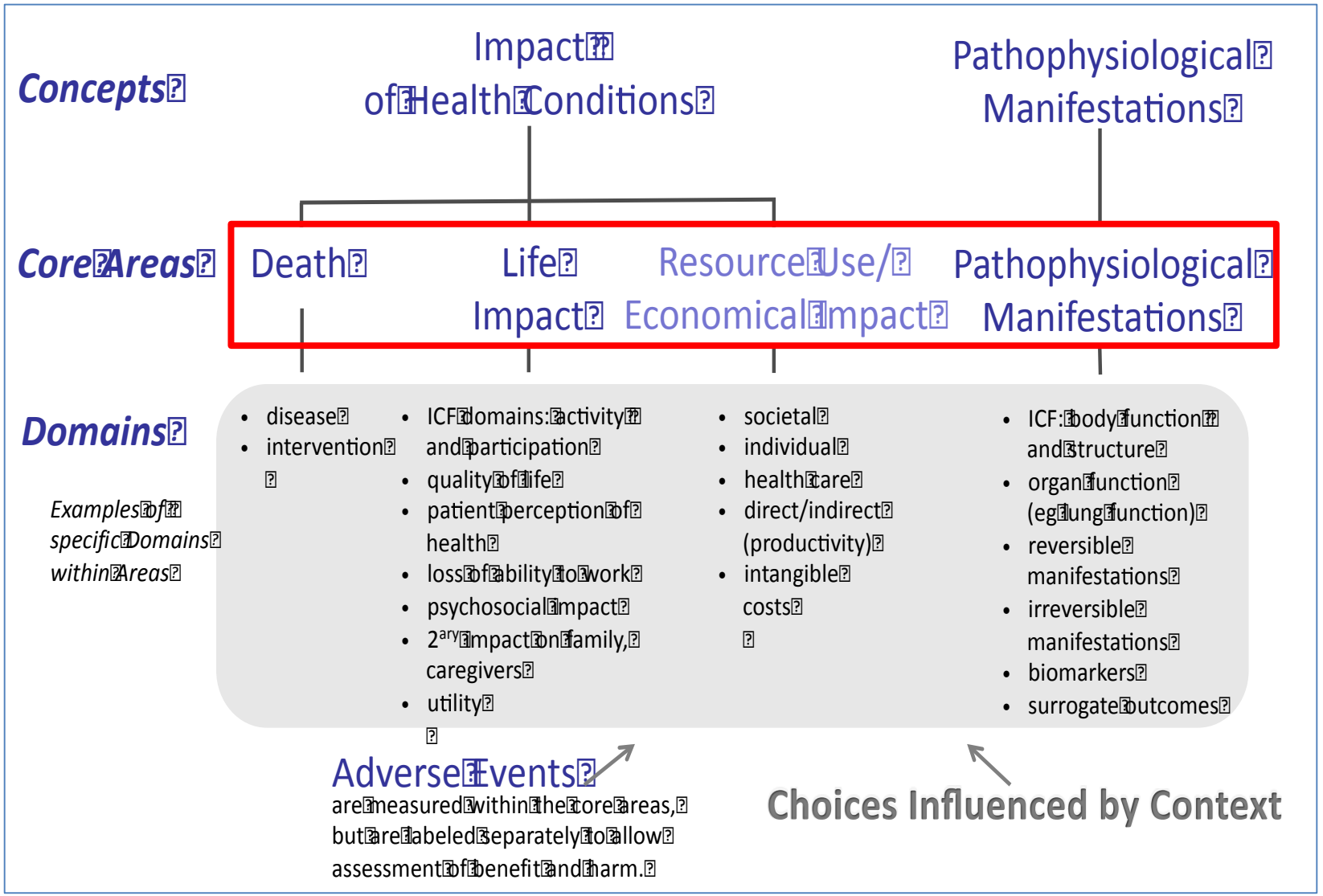
Filter 2.0→2.1 – framework for measurement (2012→2017)

Boers M et al. J Rheumatol 1998;25:198-9.

Boers, at al. Journal of Clinical Epidemiology 2014;67:745-53.

OMERACT
Outcome Measures in Rheumatology

OMERACT disease specific core domain sets



Boers M, Kirwan JR, Wells G, Beaton D, Gossec L, d'Agostino MA, et al. Developing core outcome measurement sets for clinical trials: OMERACT filter 2.0. J Clin Epidemiol 2014;67:745-53.

OMERACT Outcome Measures in Rheumatology

STEP 1: Developing a Core Domain Set

Core Areas

Death

Life Impact

Resource Use

Pathophysiological Manifestations

Setting/Contextual factors
Adverse events

Literature review
List of Domains
& Instruments

Stakeholder input

All important stakeholders are included from the start: patients and their proxies, caregivers, researchers, etc.

Match Domains to Core Areas

Draft Core Domain Set

consensus

update cycle

Core Domain Set

agreement on **what** to measure
at least one Domain from each Core Area

Source:
OMERACT Handbook
http://www.omeract.org/pdf/OMERACT_Handbook.pdf
Boers M, et al. J Clin Epidemiol. 2014; 67(7):745-53

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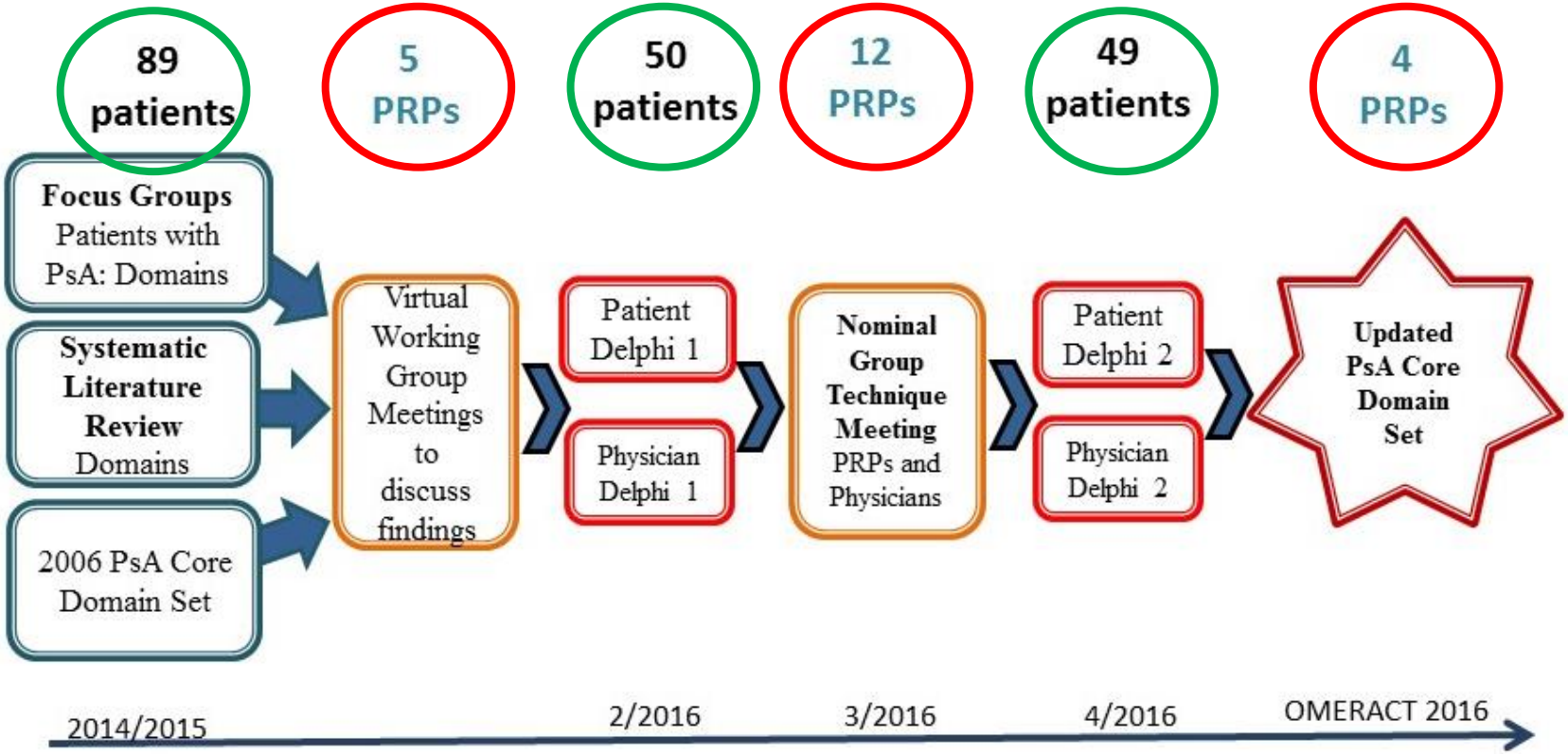
Trying to ensure representativeness



Work streams Update core outcome set for Psoriatic Arthritis

OMERACT Outcome Measures in Rheumatology

GRAPPA Group for Research and Assessment of Psoriasis and Psoriatic Arthritis

Methods of involvement

Patient participation – following OMERACT recommendations*

The patient voice was sought through

1. Active partnership of five patient research partners (PRPs) in the working group and one PRP in the Steering Group
2. International focus group study representing five continents and including seven countries
3. Delphi study
4. Consensus meeting

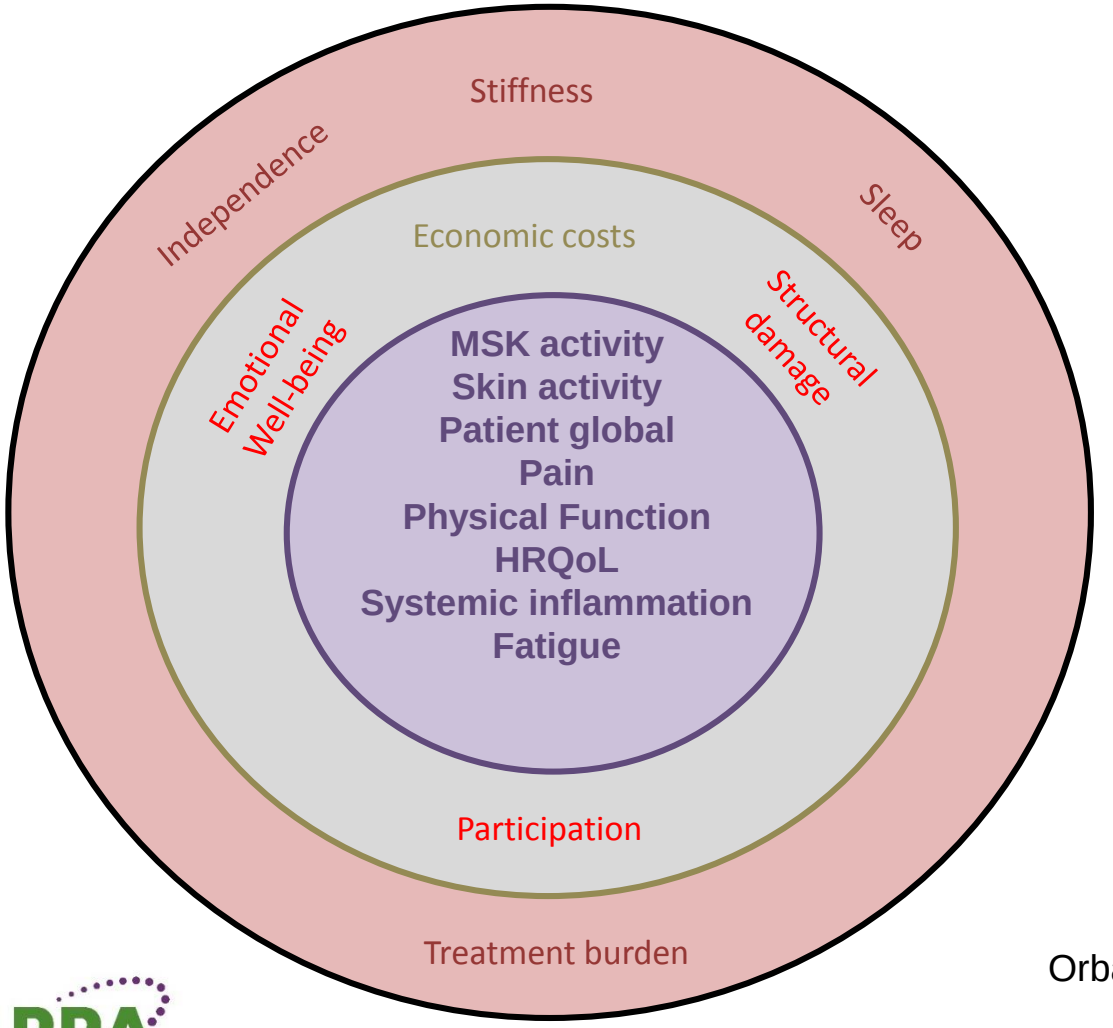
Cheung PP ea Recommendations for the Involvement of Patient Research Partners (PRP) in OMERACT Working Groups. *The Journal of rheumatology* 2016;43(1):187-93)
De Wit M ea, Successful Stepwise Development of Patient Research Partnership: 14 Years' Experience of Actions and Consequences in OMERACT, *The Patient* 2016,

GRAPPA
Group for Research and Assessment of Psoriasis and Psoriatic Arthritis

What has been the impact of patient and PRP involvement (COMET 2016)?

- PRP involvement in coding focus group transcripts ensured domains important to patients were captured.
- PRP involvement in developing the domain Delphi list ensured that domain descriptions were phrased in a manner understandable to patients.
- Integration of the patient perspective in a meaningful and representative manner provided face validity to the COS
- PRP involvement in the consensus process resulted in new domains on the research agenda and in the middle core. No patient relevant domains were added to the inner core.

2016 PsA GRAPPA-OMERACT Core Domain Set



Orbai, A. et al. *Annals of Rheum Diseases* 2016

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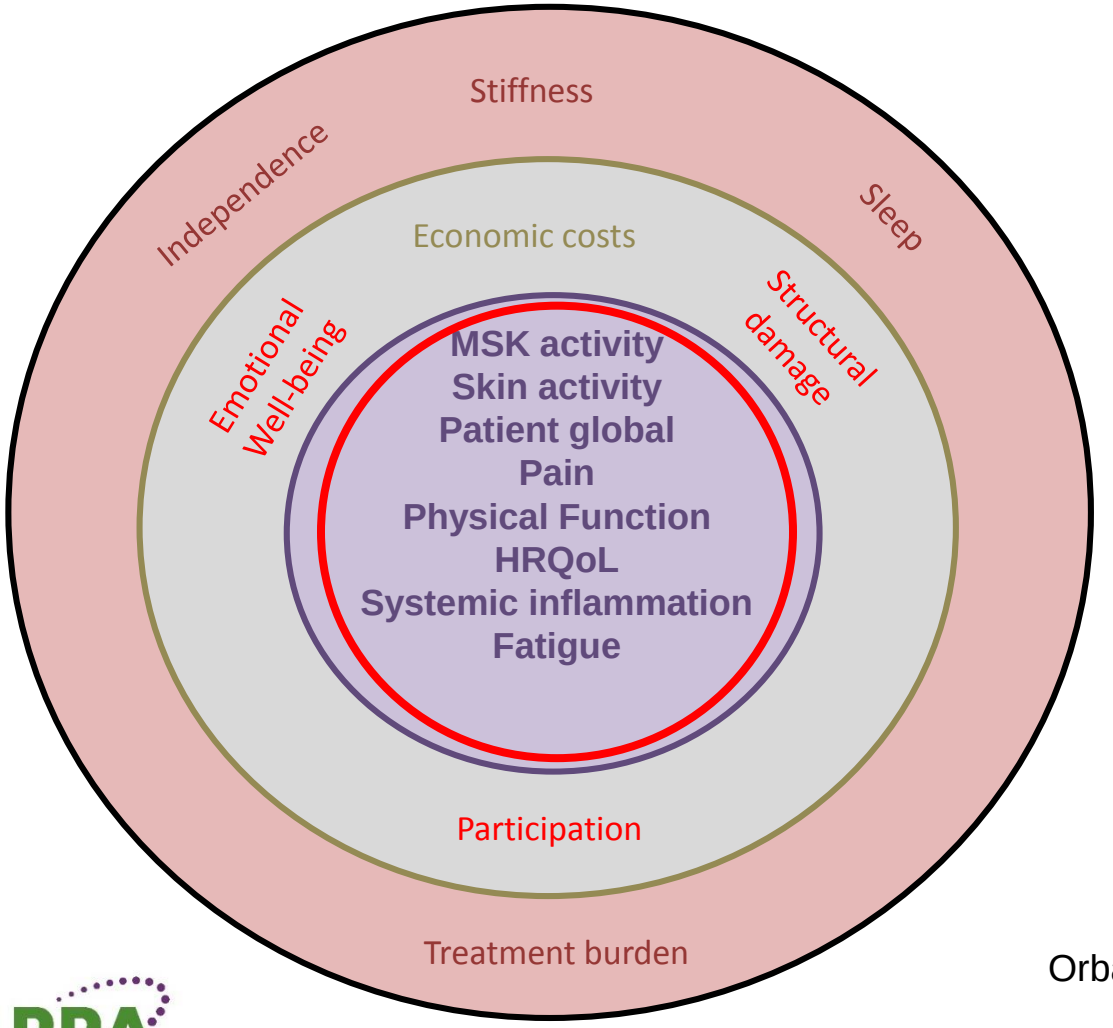

Identifying and selecting the tools

Orbai, A. et al. *Annals of Rheum Diseases* 2016

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Step 2. COMPACT Protocol for selecting PsA core instruments

PsA Core Domain Set

OMERACT 2016

What's already known?
Systematic Literature Reviews Of PsA Instruments

What can we learn from existing trials?
Analysis of RCT datasets

What do patients and clinicians think?

Delphi and Consensus Building

8 domains, 24 candidate tools, selecting 8 tools

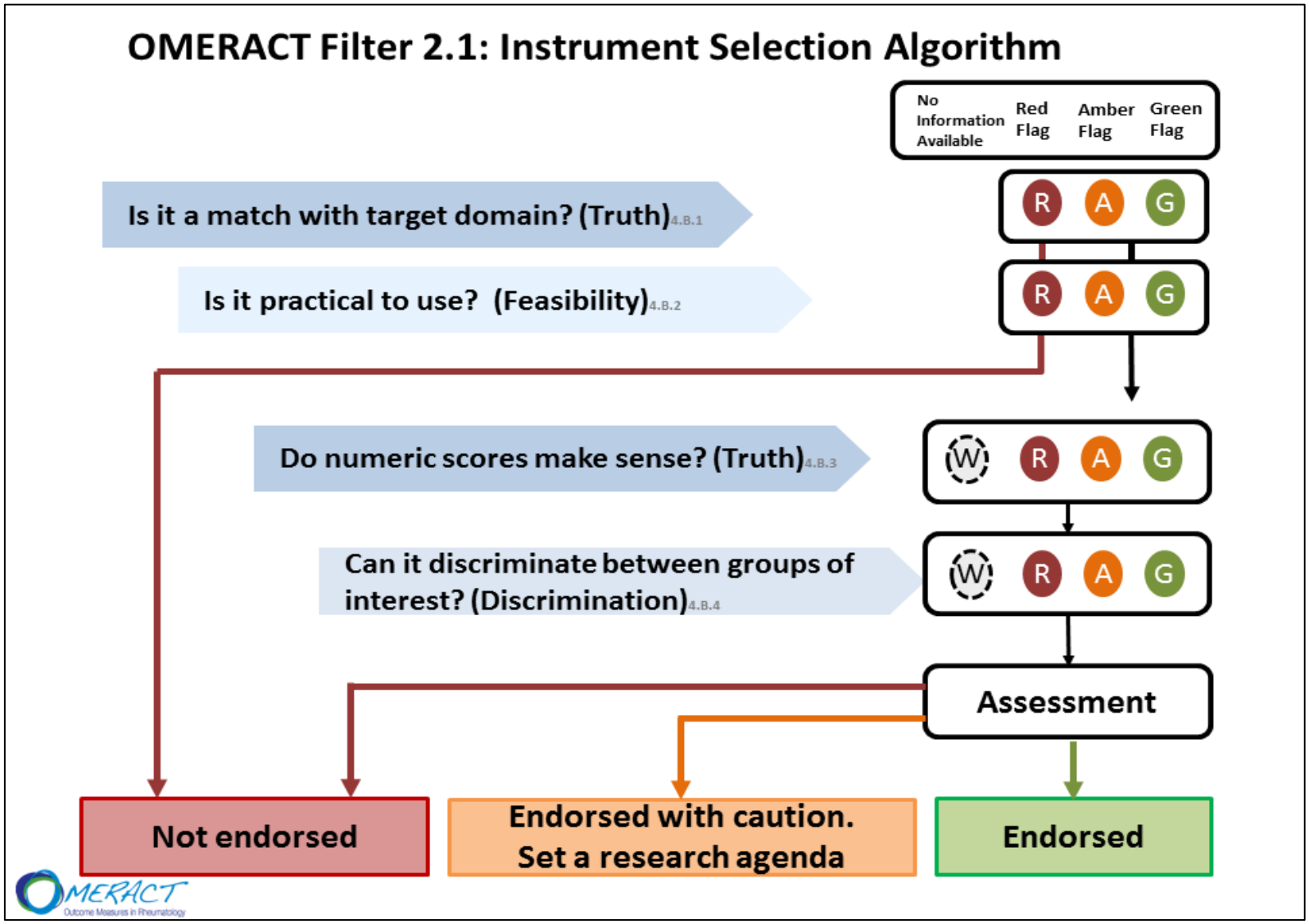
PsA Core Outcome Measure Set

OMERACT 2018

Patient meeting at GRAPPA, Focus Groups, GRAPPA small groups

GRAPPA
Group for Research and Assessment of Psoriasis and Psoriatic Arthritis

OMERACT Instrument Selection Process



Assessment of Instrument Properties

1) Truth 1: Domain Match

Assessment of Content Validity: does the instrument measure what it's supposed to measure?

2) Feasibility

Is the instrument practical for use?

3) Truth 2: "Numerical Sense"

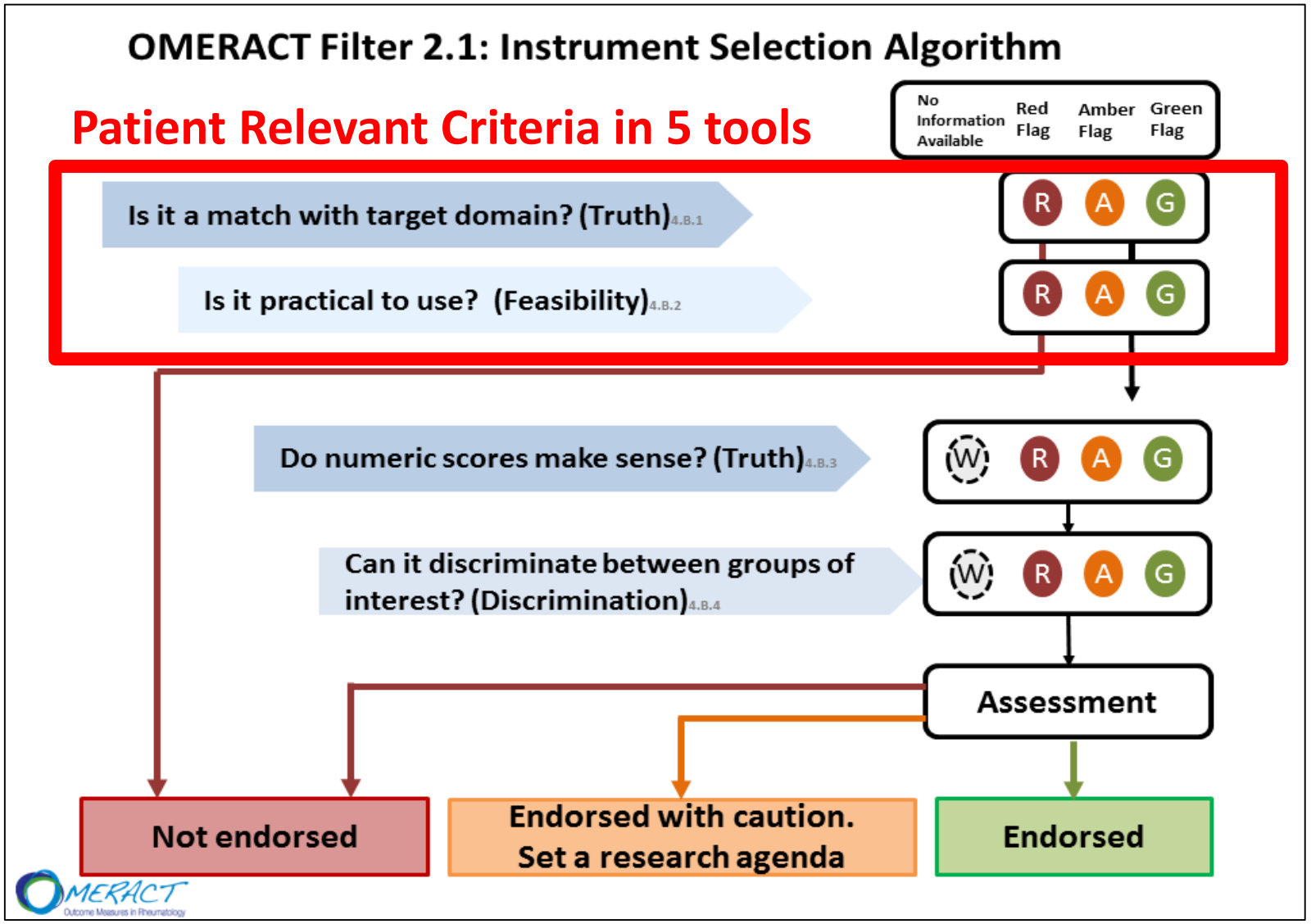
Assessment of Construct validity
"the degree to which the scores on the instrument relate to other measures (patient-report or clinical indicators) in a manner that is consistent with theoretically derived, a priori hypotheses concerning the domains that are being measured."

4) Discrimination

Assessed in the following ways:

1. Stability in situation of no change (test-retest reliability)
2. Detecting change in situations of change
3. Sensitivity to change in an RCT
4. Thresholds of meaning defined

PsA Instrument Selection Process (2017)



Content and Face Validity and Feasibility of 5 Candidate Instruments for Psoriatic Arthritis Randomized Controlled Trials: The PsA OMERACT Core Set Workshop at the GRAPPA 2017 Annual Meeting

Richard Holland, William Tillett, Alexis Ogdie, Ying Y. Leung, Dafna D. Gladman, Kristina Callis Duffin, Laura C. Coates, Philip J. Mease, Lihi Eder, Vibeke Strand, Musaab Elmamoun, Pil Højgaard, Jeffrey Chau, Maarten de Wit, Niti Goel, Chris A. Lindsay, Oliver FitzGerald, Bev Shea, Dorcas Beaton, and Ana-Maria Orbai

The Journal of Rheumatology

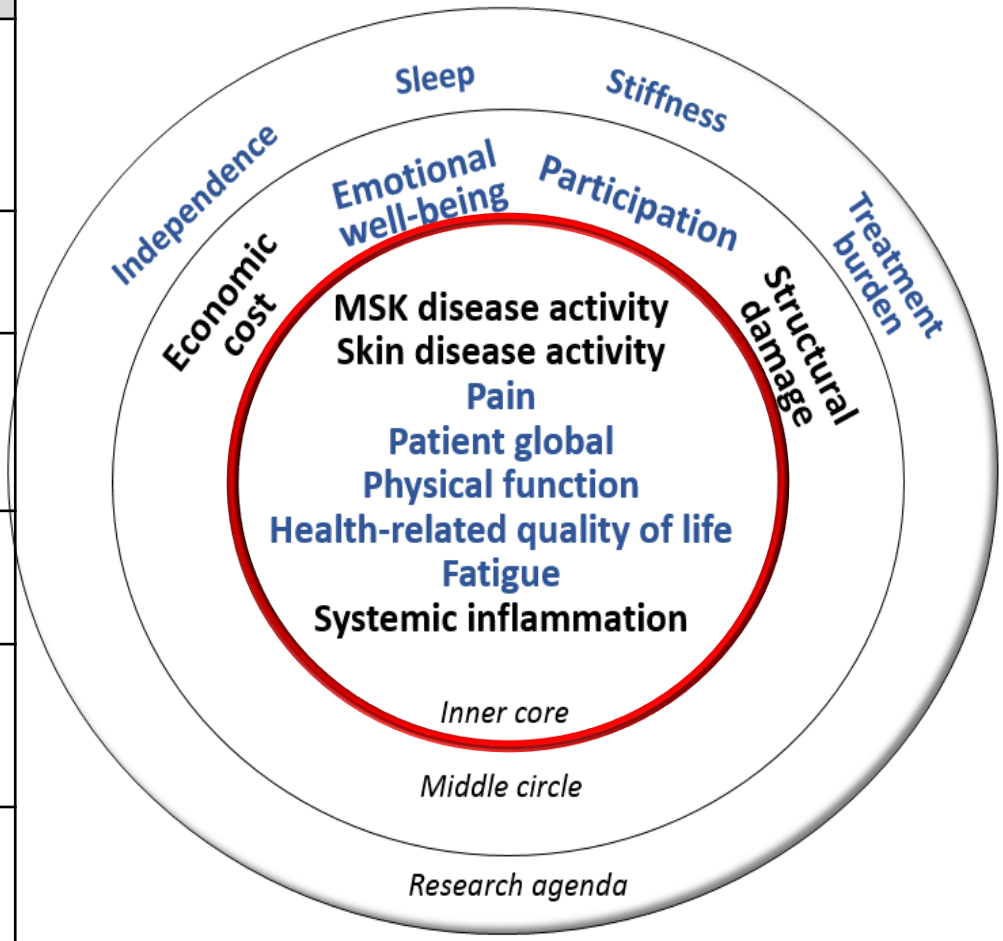
J Rheumatol 2018;94;17-25

Task for Today GRAPPA 2017

Truth 1: Content validity | **2. Feasibility**

Breakout gps	Core domain	Instruments
GROUP A **2 PRPs:**	• **MSK DA: arthritis** • **MSK DA: dactylitis** • **MSK DA: spine**	• Joint counts: 66/68, Dactylitis count (0-20), Leeds Dactylitis Index (LDI), LDI basic
GROUP B **2 PRPs:**	• **MSK DA: enthesitis** • **Skin DA: skin**	• SPARCC, LEI. • PSI, PASI, BSA, PGA,
GROUP C **2 PRPs:**	• **Patient global**	• Patient global VAS (1-4), Patient global NRS (1-3)
GROUP D **2 PRPs:**	• **Physical function**	• HAQ-DI, mHAQ, SF-36 PF10, PROMIS-PF
GROUP E **2 PRPs:**	• **HRQoL/DI**	• SF-36 (MCS, PCS, domain scores), PsAID12, PsAID9,
GROUP F **2 PRPs:**	• **Fatigue** • **Systemic inflammation**	• NRS Fatigue, FACIT Fatigue, PROMIS Fatigue • ESR, CRP

Population: PEOPLE with PsA
Setting: PsA randomized controlled trial

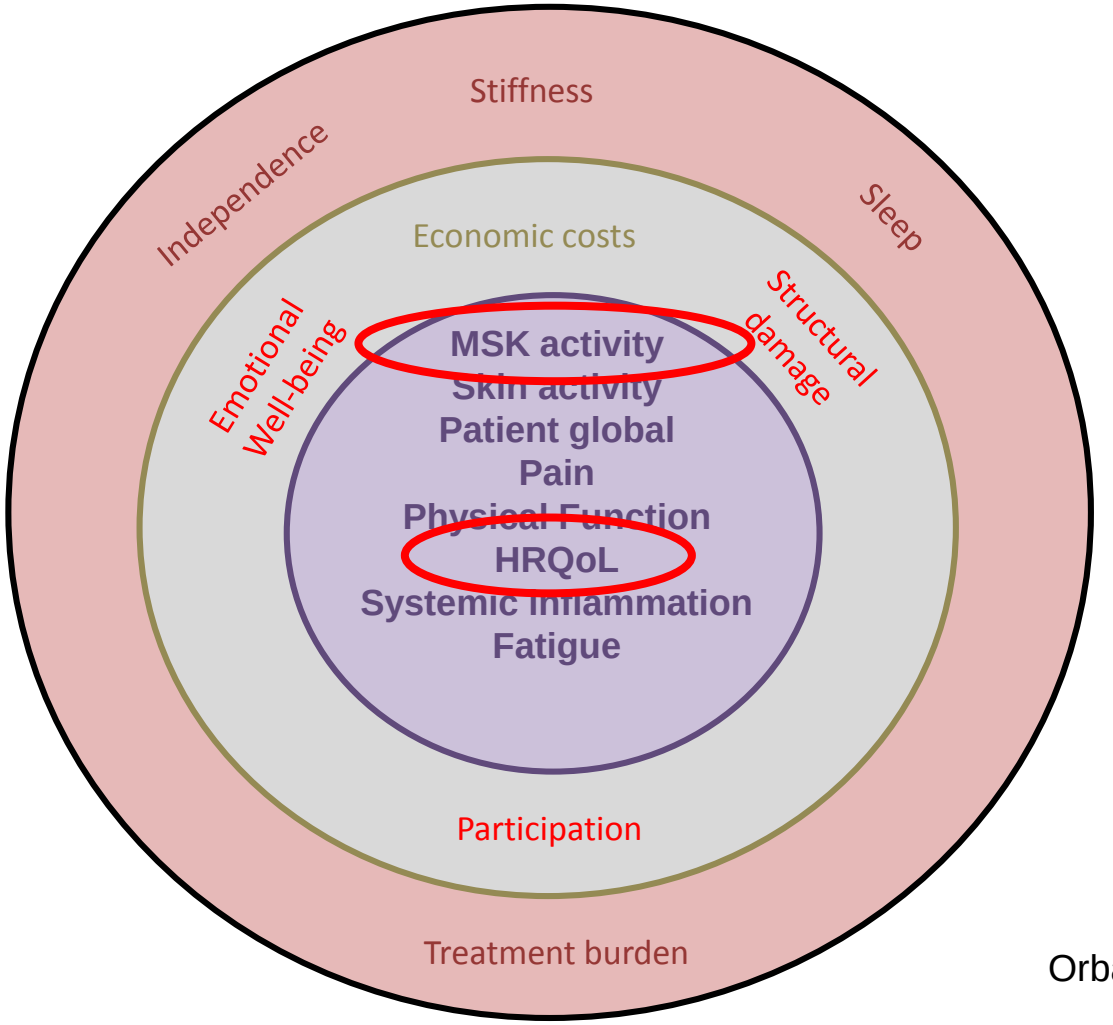


PsA core Domain set
Orbai, et al. ARD 2016; 76 (4), 673-680

Workshop conclusion

- Patients like all other stakeholders need to be introduced to and supported in the tools selection process.
- A patient booklet, with plain language summaries of the history and key features of the instruments (incl. the full tekst of the instrument), is very helpful.
- There is a limited number of instruments that can be discussed and agreed on at a face-2-face meeting due to time constraints.
- Patients can add valuable contributions to assessing domain match and feasibility issues.

2016 PsA GRAPPA-OMERACT Core Domain Set



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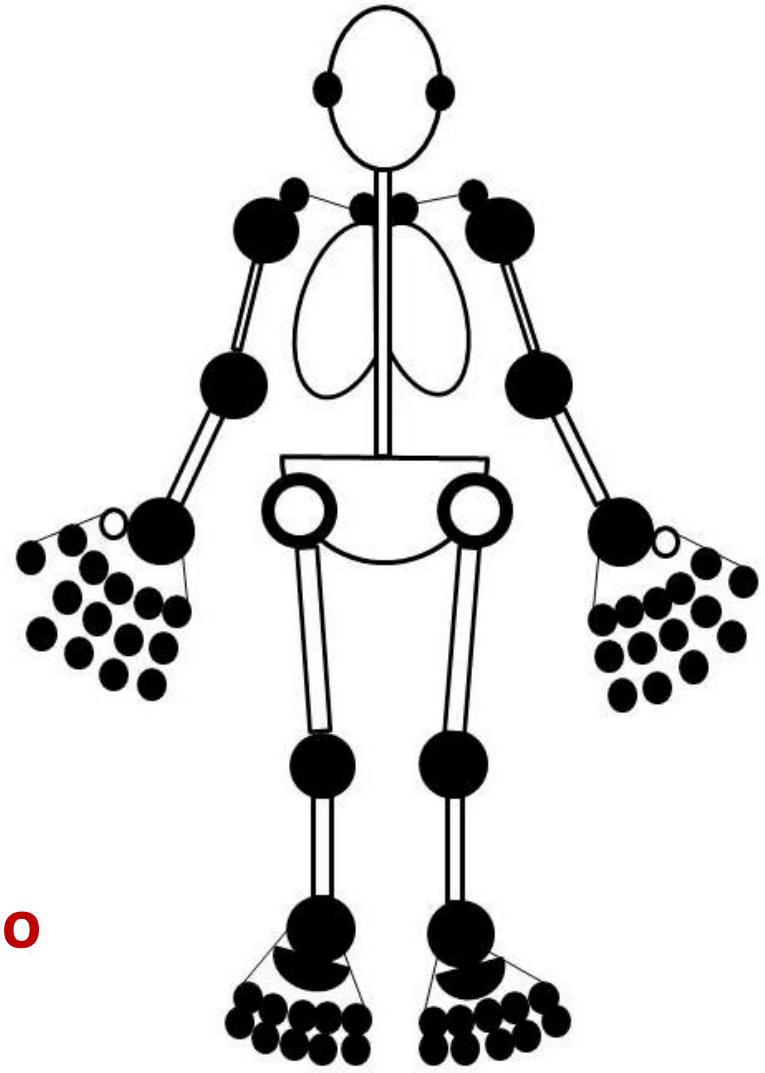
Example 1

Peripheral Joint Activity

As part of the core domain
Musculoskeletal Disease Activity

66/68 Tender and Swollen Joint Count



1. Two minute instruction video

2. Questionnaire

3. One hour Webinar at 2 time slots

1. Does the 66/68 joint count have content validity?

- Domain: MSK disease activity – peripheral arthritis
- Patient Checklist for Domain Match
  - Is this instrument measuring what YOU want to measure?
  - Is the instrument free of redundant, unnecessary or potentially inappropriate or sensitive items?
  - Have all important elements been included?
  - Are elements in the assessment clearly outlined and described?
  - Are the responses suitable and complete for each item considering your intended application?
  - Is the method of scoring appropriate (e.g., weighting)?
  - Do you think there should be any additional items?
  - Do you think that any items should be removed?
  - Overall did it seem to ask about important parts of your experience in this domain?

1. Does the 66/68 joint count have content validity?

- Domain: MSK disease activity – peripheral arthritis
- Checklist for Domain Match
  - Is this instrument measuring what YOU want to measure? **YES**
  - Is the instrument free of redundant, unnecessary or potentially inappropriate or sensitive items? **YES**
  - Have all important elements been included? **Maybe DIPs of toes?**
  - Are elements in the assessment clearly outlined and described? **YES**
  - Are the responses suitable and complete for each item considering your intended application? **YES**
  - Is the method of scoring appropriate (e.g., weighting)? **YES – no weighting**
  - Do you think there should be any additional items? **NO**
  - Do you think that any items should be removed? **NO**
  - Overall did it seem to ask about important parts of your experience in this domain? **YES**

66/68 Joint Count matches the Domain MSK Disease Activity

2. Is the 66/68 joint count feasible?

- Checklist for Feasibility (short version)
  - Is it easy enough to complete?
  - Does it take a reasonable amount of time to complete it?
  - Is the amount of equipment and training that would be needed before you can do this reasonable?

2. Is the 66/68 joint count feasible?

- Checklist for Feasibility (short version)
  - Is it easy enough to complete? **YES**
  - Does it take a reasonable amount of time to complete it? **YES**
  - Is the amount of equipment and training that would be needed before you can do this reasonable? **YES**

Example 2

Health Related Quality of Life

PsAID 12
Psoriatic Arthritis
Impact of Diease

1. One hour Webinar at 2 time slots
2. Face-2-face meeting (12 PRPs) before filling in the domain match & feasibility questionnaire
3. PRP's in working group

The EULAR Psoriatic Arthritis Impact of Disease: PsAID9 for clinical trials

We want you to indicate how much your psoriatic arthritis impacts your health. Please tell us how you have been feeling this last week.

1. Pain

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

2. Fatigue

Circle the number that best describes the overall level of fatigue due to your psoriatic arthritis you have experienced during the last week:

3. Circle the number that best ... you f...

Circle t... ful...

Ci...

...nd annoyance ... ...g the last

7. Slee...

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

8. Coping

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

9. Anxiety, fear and uncertainty

Circle the number that best describes the level of anxiety, fear and uncertainty (for example about the future, treatments, fear of loneliness) due to your psoriatic arthritis you have experienced during the last week:

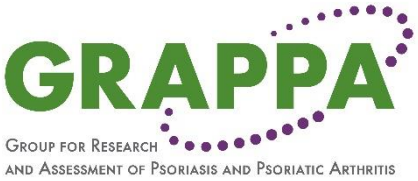
THANK YOU FOR ANSWERING THIS QUESTIONNAIRE

Conclusion

- Patient involvement is feasible in all stages of COS development.
- Task and role in Core Domain Set (What) development varies from the patient role in Core Instrument Set (How) development. In the latter patient involvement should be focused on domain match and feasibility.
- Consider both the involvement of patients AND Patient Research Partners when developing a COS.
- Depending on the instrument, different formats for collecting data about domain match and feasibility of instruments should be applied. There is no ‘one size fits all’ method for patient involvement.

Acknowledgment

The GRAPPA-OMERACT working group



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