Dr. Maarten
de Wit

Amsterdam
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Feasibility and added value of patient involvement in the development of core outcome set development
A case study from the field of rheumatology

Collaborators

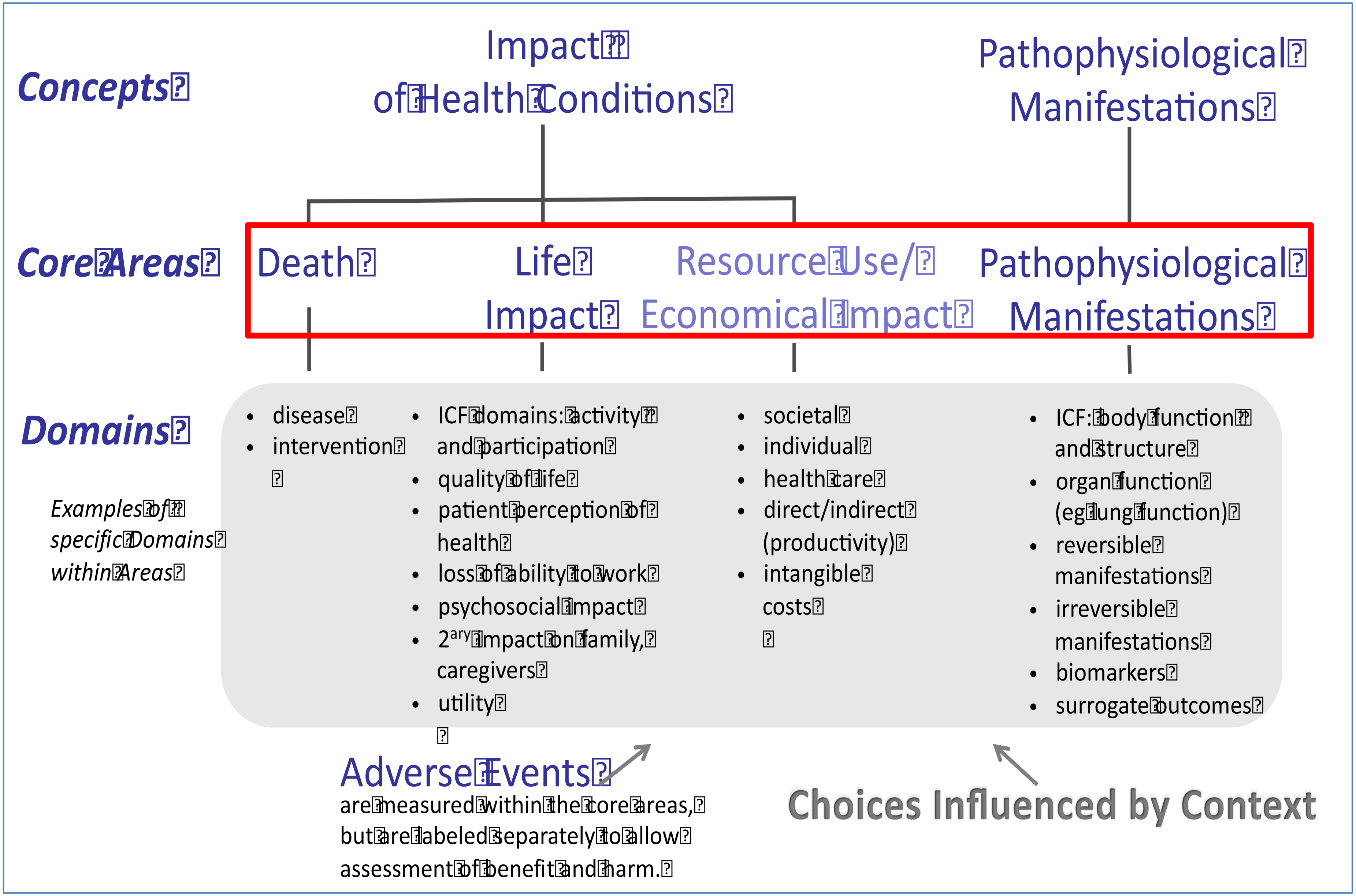
M. de Wit
A. Ogdie
W. Campbell
P. Mease, P.
N. Goel
L. Gossec,

Y.Y. Leung
C. Lindsay
P. Palominos
I. Steinkoenig
S. Grieb
A-M Orbai

Overview

- Core domain set development according to the OMERACT filter 2.0
- Patient involvement througout the process of updating the Psoriatic Arthritis core domain set
- Challenges of patient involvement in core set development
- Conclusions

OMERACT disease specific core domain sets



Concepts

Impact of Health Conditions

Pathophysiological Manifestations

Core Areas

Death

Life Impact

Resource Use/ Economical Impact

Pathophysiological Manifestations

Domains

Examples of specific Domains within Areas

- disease
- intervention

- ICF domains: activity and participation
- quality of life
- patient perception of health
- loss of ability to work
- psychosocial impact
- 2ary impact on family, caregivers
- utility

- societal
- individual
- health care
- direct/indirect (productivity)
- intangible costs

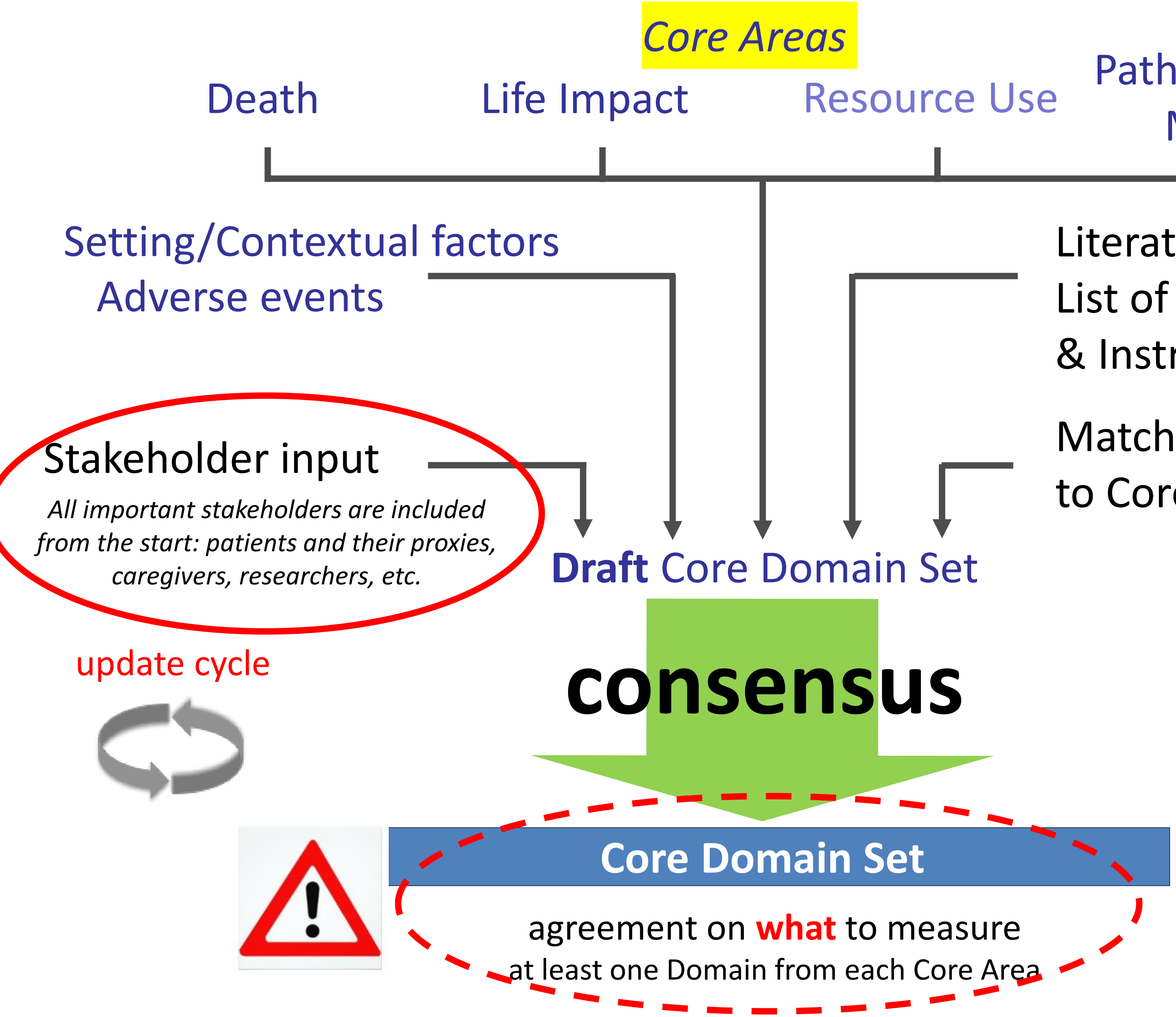
- ICF: body function and structure
- organ function (eg lung function)
- reversible manifestations
- irreversible manifestations
- biomarkers
- surrogate outcomes

Adverse Events

are measured within the core areas, but are labeled separately to allow assessment of benefit and harm.

Choices Influenced by Context

OMERACT Filter 2.0: 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

Match Domains to Core Areas

Stakeholder input

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

update cycle

Draft Core Domain Set

consensus

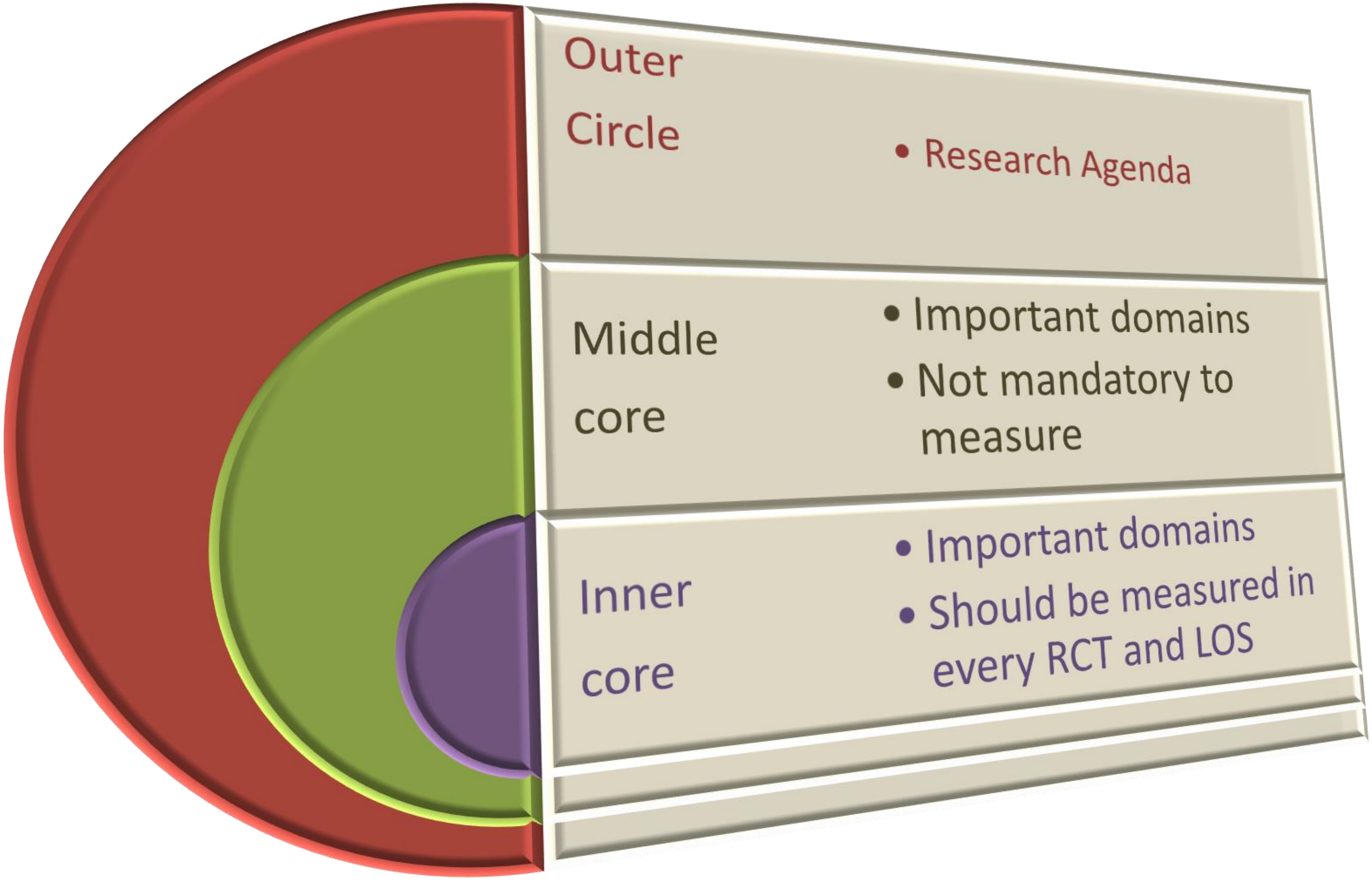
Core Domain Set

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

Template of an OMERACT core domain set



Outer Circle

- Research Agenda

Middle core

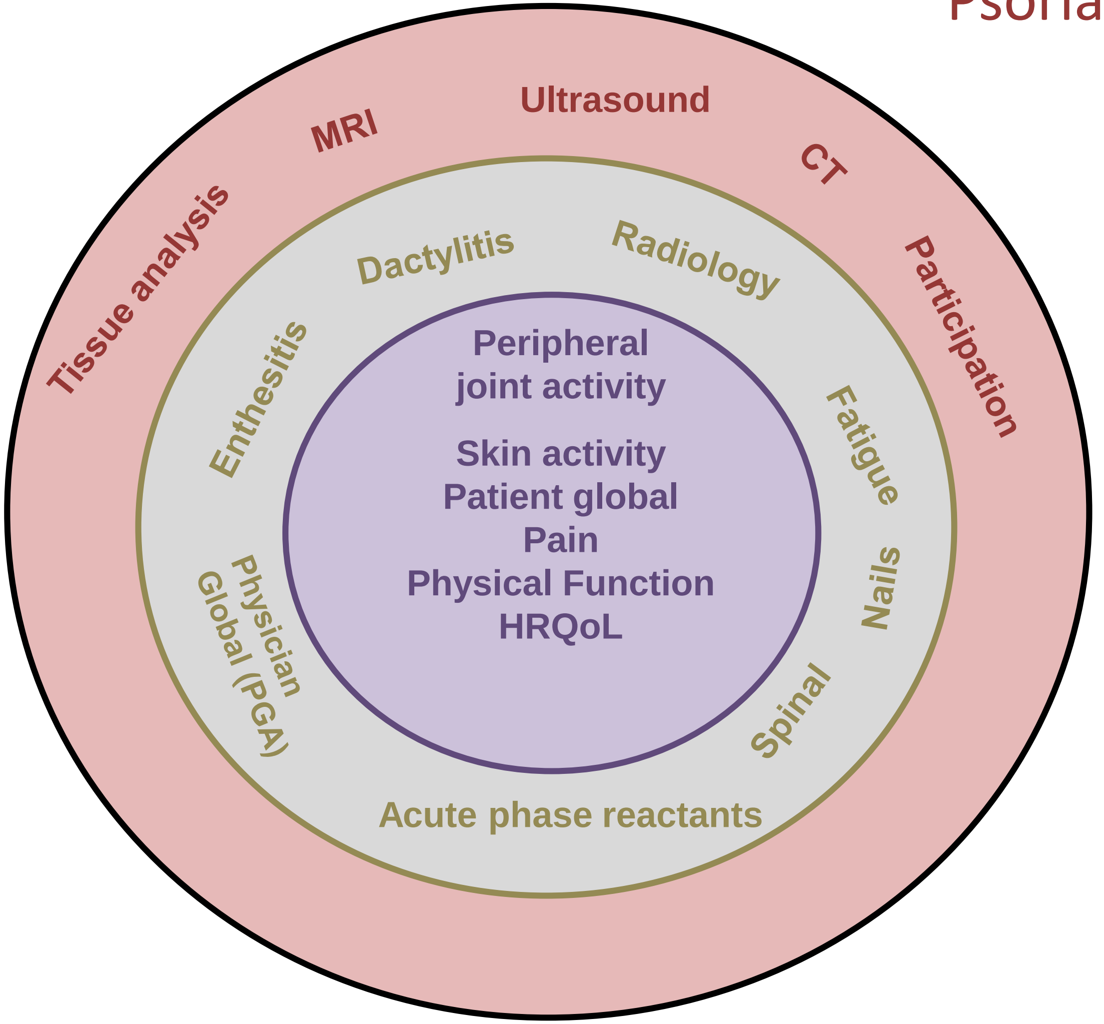
- Important domains
- Not mandatory to measure

Inner core

- Important domains
- Should be measured in every RCT and LOS

OMERACT-GRAPPA Psoriatic Arthritis core set

2006



Ultrasound

MRI

CT

Tissue analysis

Participation

Dactylitis

Radiology

Enthesitis

Fatigue

Nails

Physician Global (PGA)

Spinal

Acute phase reactants

Peripheral joint activity

Skin activity

Patient global

Pain

Physical Function

HRQoL

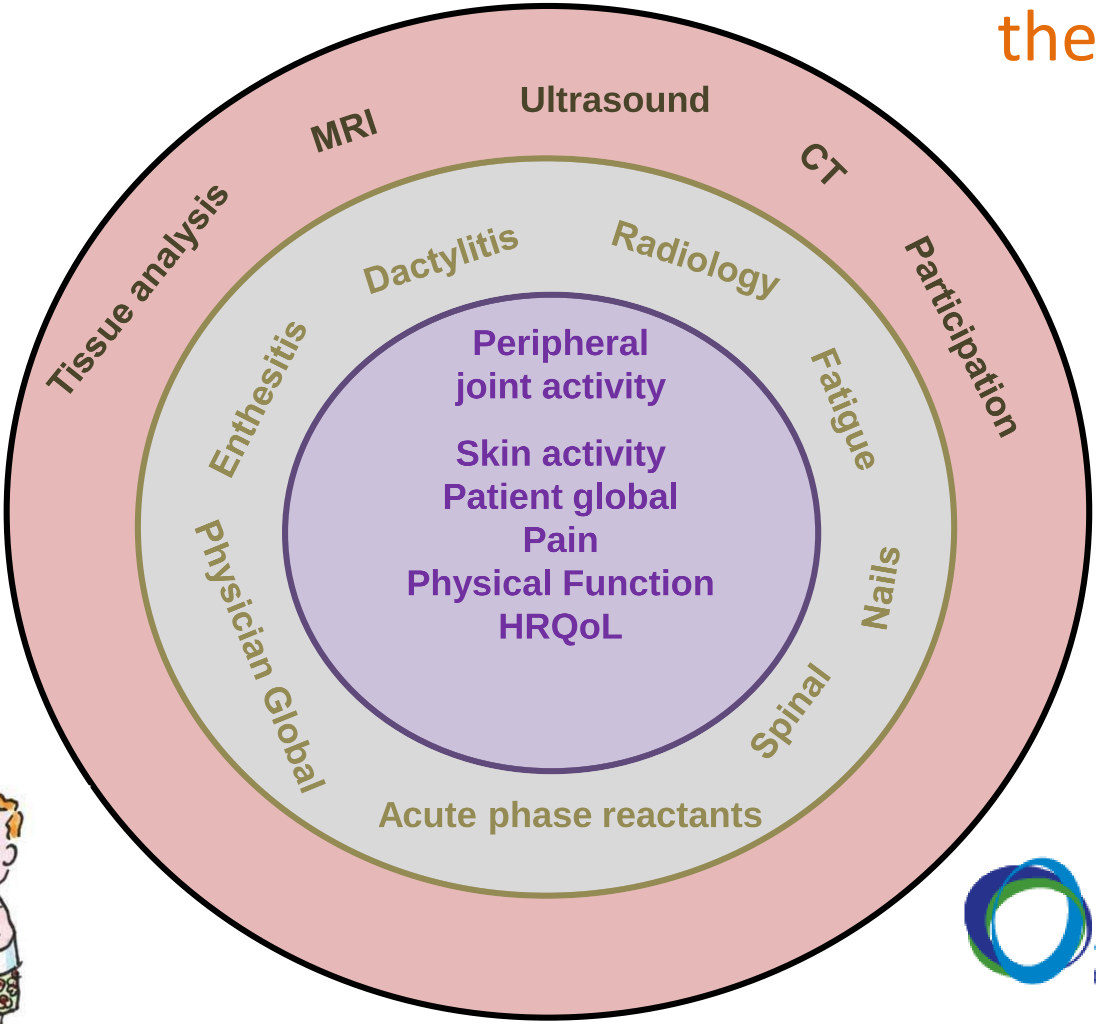
Gladman D, et al. J Rheum 2006

Why updating the PsA COS?

2006

2012

2013



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Physical Function

HRQoL

NEW

OMERACT
Outcome Measures in Rheumatology

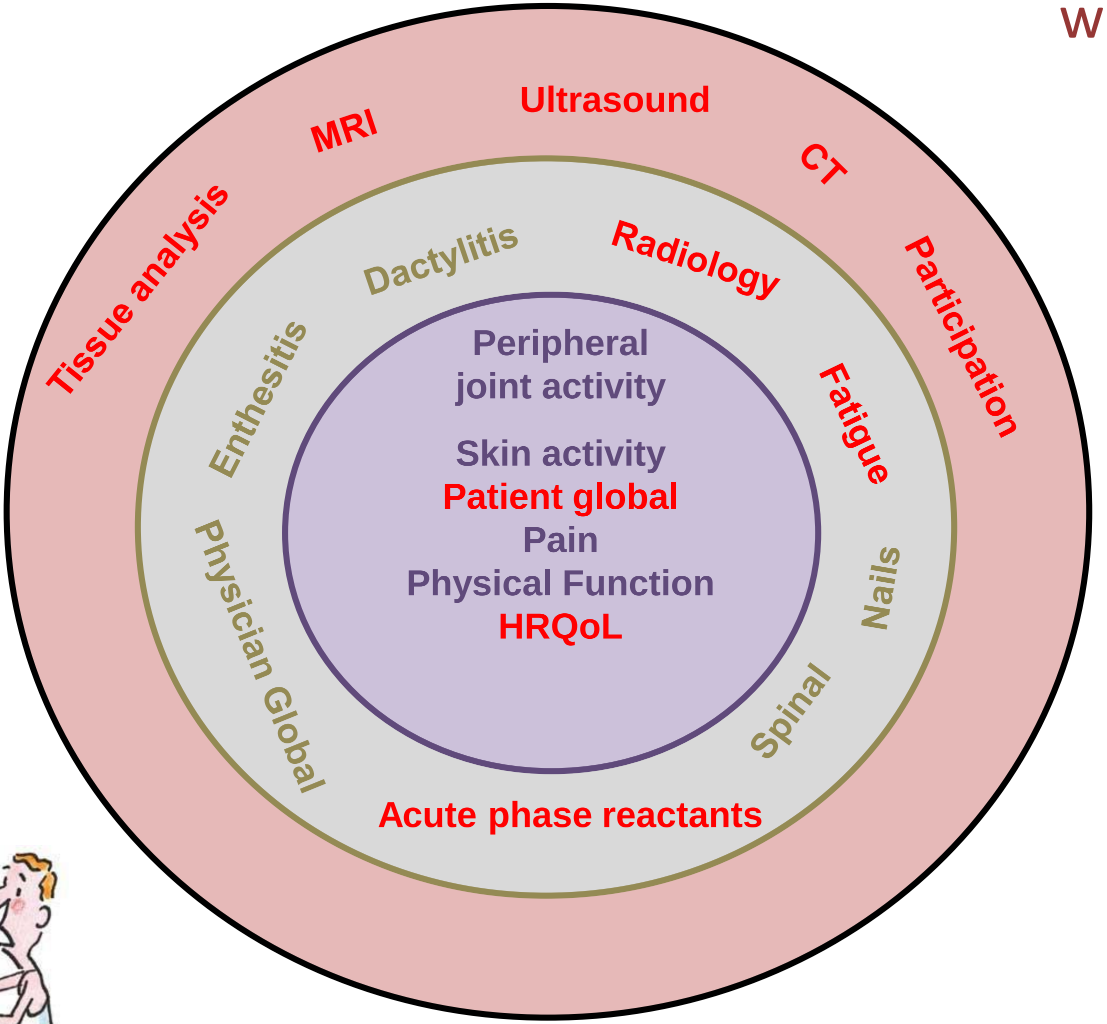
Tillett W, Adebajo A, Brooke M, et al. Patient involvement in outcome measures for psoriatic arthritis. *Curr Rheumatol Rep* 2014;16(5):418.

Core set discussed with patients

2006

2012

2013



Peripheral joint activity

Skin activity

Patient global

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2006

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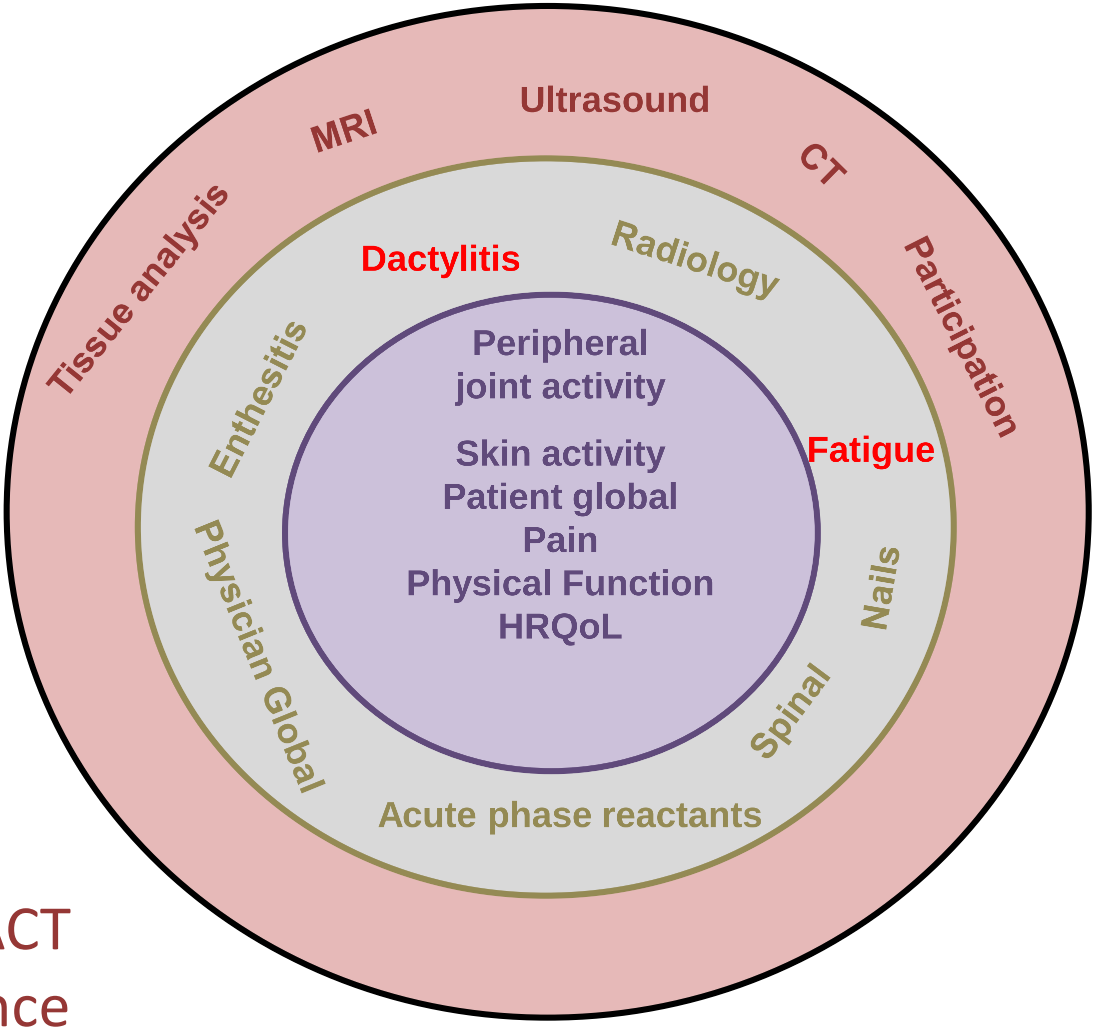

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Peripheral
joint activity

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Patient global
Pain
Physical Function
HRQoL

Need to
update the
PsA core set
with active
patient
involvement

OMERACT
conference
2014

Tillett W, Eder L. Enhanced patient involvement and the need to revise the core set- report from the PsA workshop at OMERACT 2014. *Jrn of Rheum*, 2015

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Methods of involvement

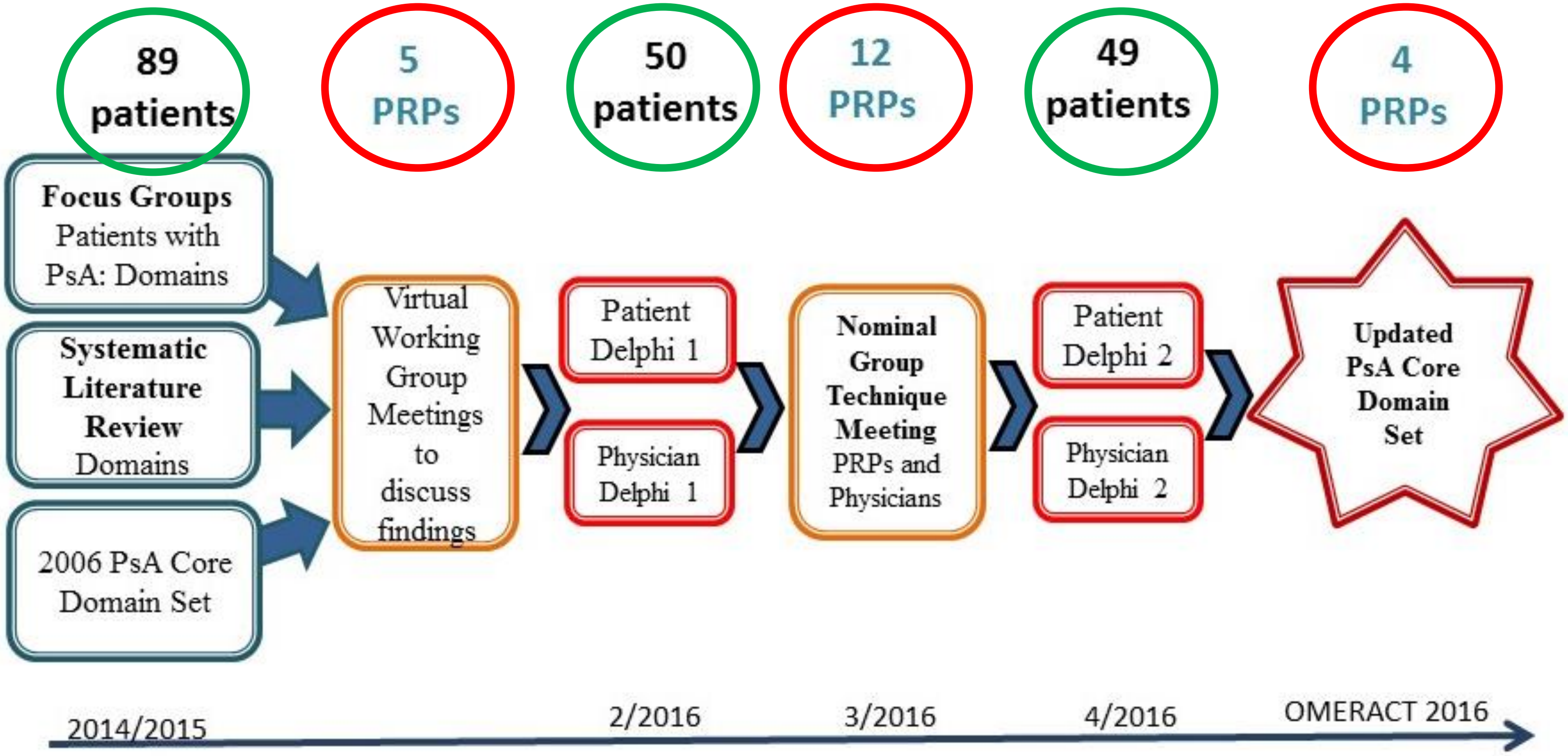
Patient participation – following OMERACT recommendations*

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,

Trying to ensure representativeness



Work streams Update core outcome set for Psoriatic Arthritis

What about PRPs Tasks?

	Steering group	Working group	Study design	SLR	FG protocol	FG moderation analysis	Delphi Design	NGT meeting
PRP 1		X	X	X	X	X	X	X
PRP 2		X	X		X		X	X
PRP 3		X	X	X	X		X	X
PRP 4	X	X	X	X	X	X	X	X
PRP 5					X			
PRP 6					X			
PRP 7					X	X	X	X
PRP 8								
PRP 9								
PRP 10								
PRP 11		X	X	X	X			
PRP 12								
PRP 13						X		
PRP 14					X			
TOTAL	1	5	5	4	9	4	5	5

What about PRPs' recognition?

Co-authorship

	Steering group	Working group	Study design	SLR 1	FG protocol	FG moderation analysis	Delphi Design	NGT meeting
PRP 1		X	X	X	X	X	X	X
PRP 2		X	X		X		X	X
PRP 3		X	X	X	X		X	X
PRP 4	X	X	X	X	X	X	X	X
PRP 5					X			
PRP 6					X			
PRP 7					X	X	X	X
PRP 8								
PRP 9								
PRP 10								
PRP 11		X	X	X	X			
PRP 12								
PRP 13						X		
PRP 14					X			
TOTAL	1	5	5	4	9	4	5	5

Extended PRP involvement

	Steering group	Working group	Study design	SLR 1	FG protocol	FG moderation analysis	Delphi	NGT meeting
PRP 1		X	X	X	X	X	X	X
PRP 2		X	X		X		X	X
PRP 3		X	X	X	X		X	X
PRP 4	X	X	X	X	X	X	X	X
PRP 5					X		X	X
PRP 6					X		X	X
PRP 7					X	X	X	X
PRP 8							X	X
PRP 9							X	X
PRP 10							X	X
PRP 11		X	X	X	X		X	X
PRP 12							X	X
PRP 13						X		
PRP 14					X		X	
TOTAL	1	5	5	4	4	4	13	12

New domains identified through SLR and the qualitative studies

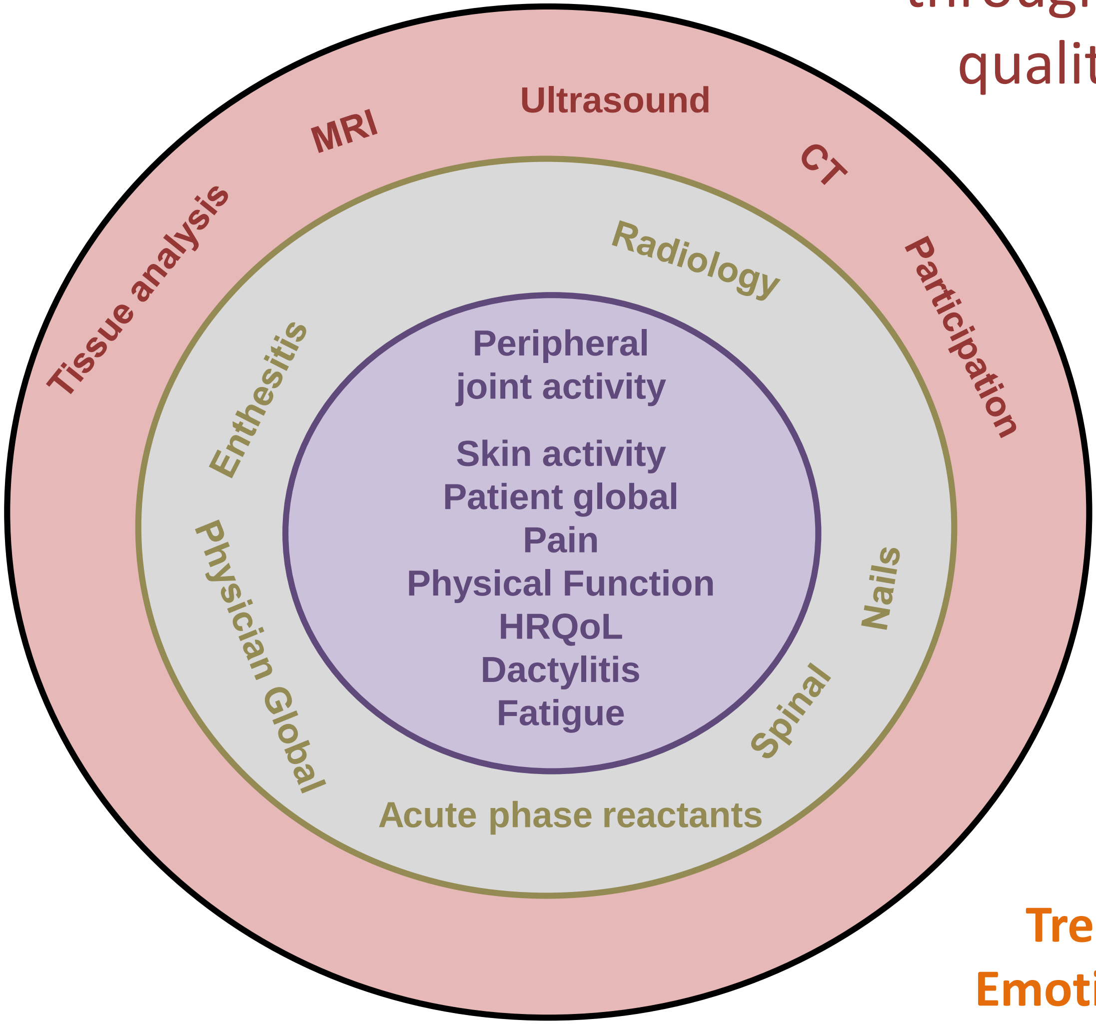
2006

2012

2013

2014

2015



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Tissue analysis
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Enthesitis
Physician Global
Acute phase reactants
Spinal
Nails

Peripheral joint activity
Skin activity
Patient global
Pain
Physical Function
HRQoL
Dactylitis
Fatigue

Systemic inflammation

Independence
Stiffness
Sleep
Treatment burden
Emotional wellbeing

Economic costs

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

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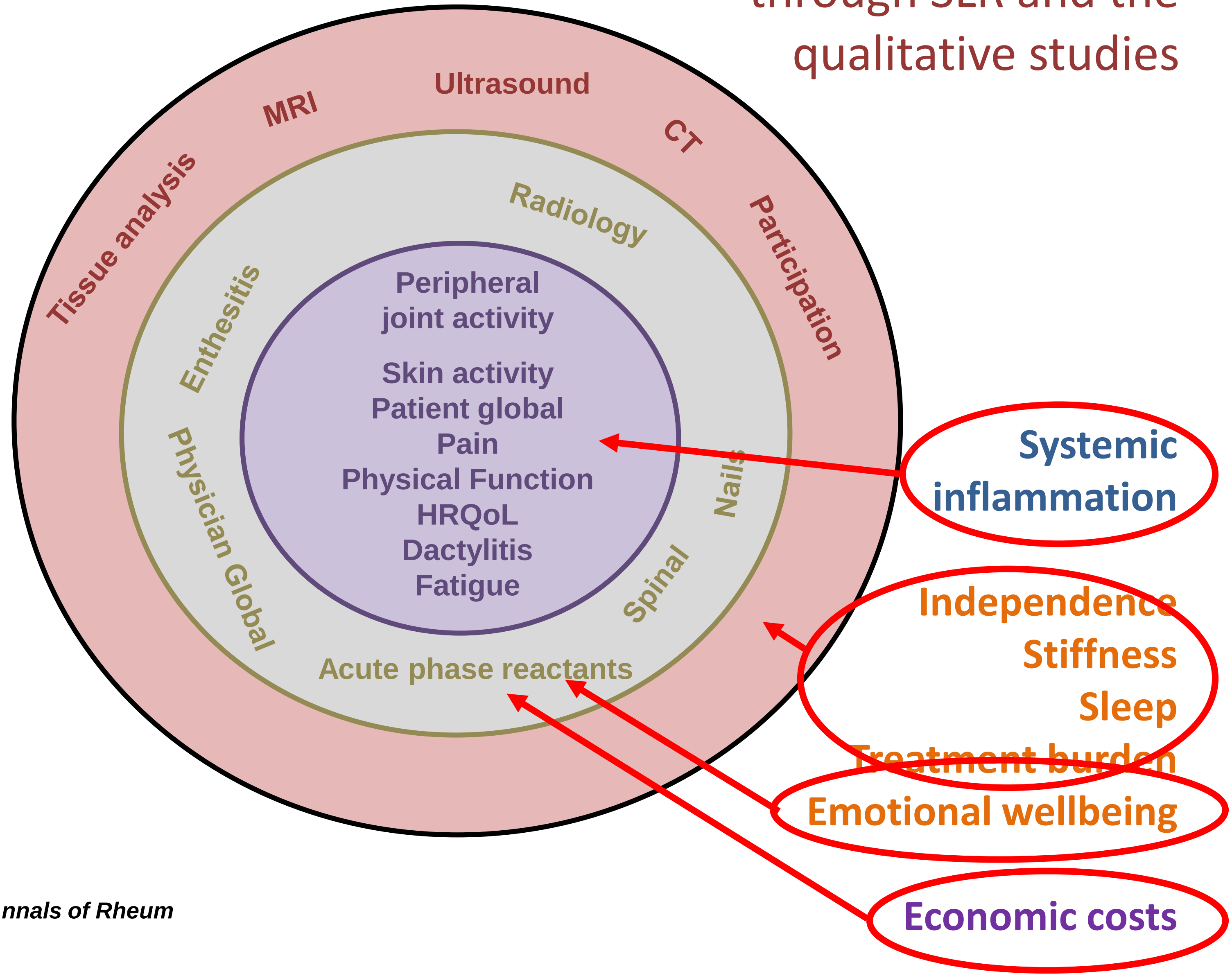

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What were challenges of involving patients in the COS update process?

1. Unanticipated Work Load for PRPs and researchers
2. Communication and equal collaboration

   How to enhance new approaches and attitudes

3. Ensuring broad representativeness of patients' perspectives

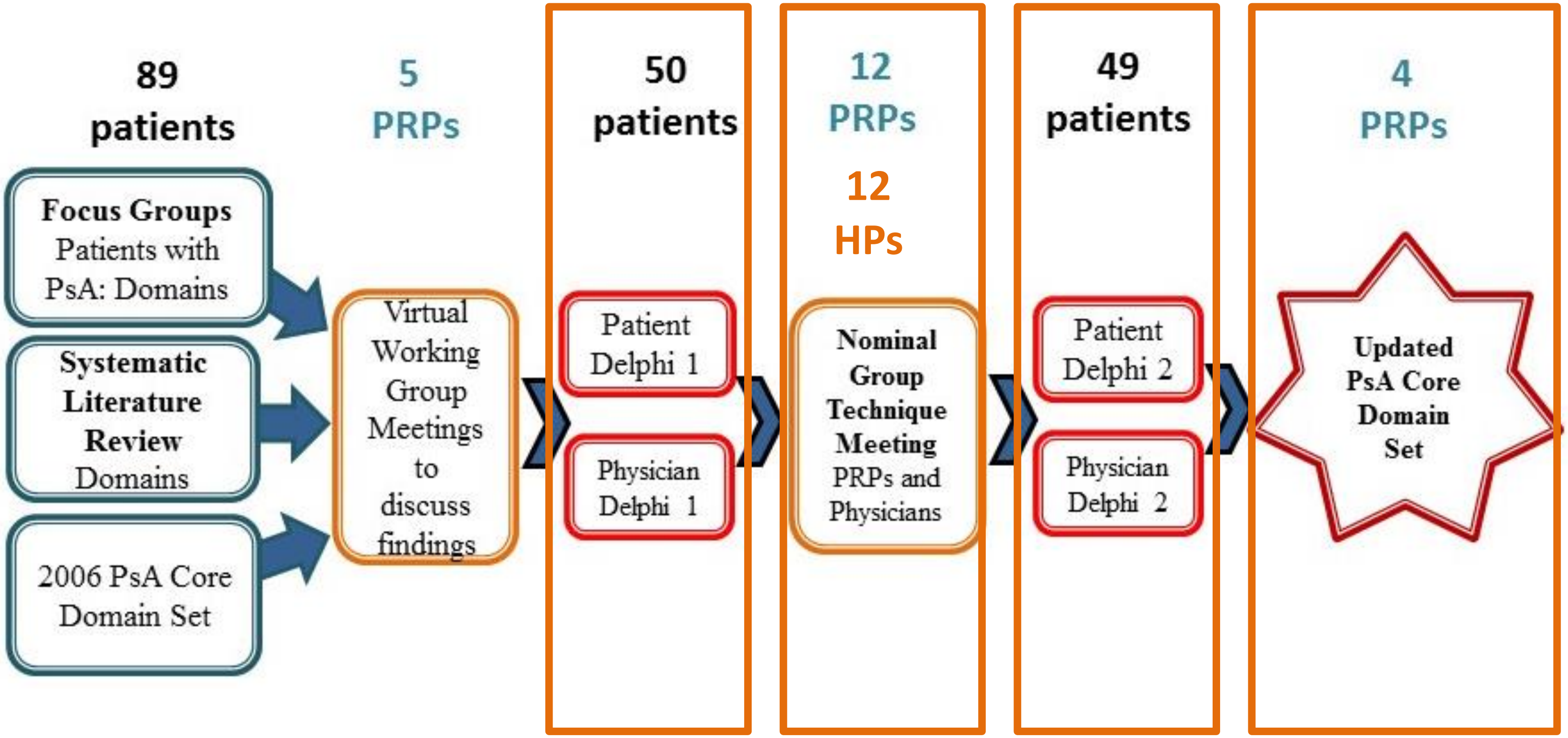
   In demography, geography, disease severity and in numbers

4. Keeping PRPs and researchers motivated to collaborate
5. Preserving the patient perspective throughout the research process

Preserving the patients’ perspectives

- Core domain sets should be short and feasible to measure, and based on stakeholder input and consensus.
- But how can we guarantee that consensus is obtained without losing important domains for patients and hence content validity of the core set?

PRP participation as integral part of the consensus building



89 patients

Focus Groups
Patients with PsA: Domains

Systematic Literature Review
Domains

2006 PsA Core Domain Set

5 PRPs

Virtual Working Group Meetings to discuss findings

50 patients

Patient Delphi 1

Physician Delphi 1

12 PRPs

12 HPs

Nominal Group Technique Meeting
PRPs and Physicians

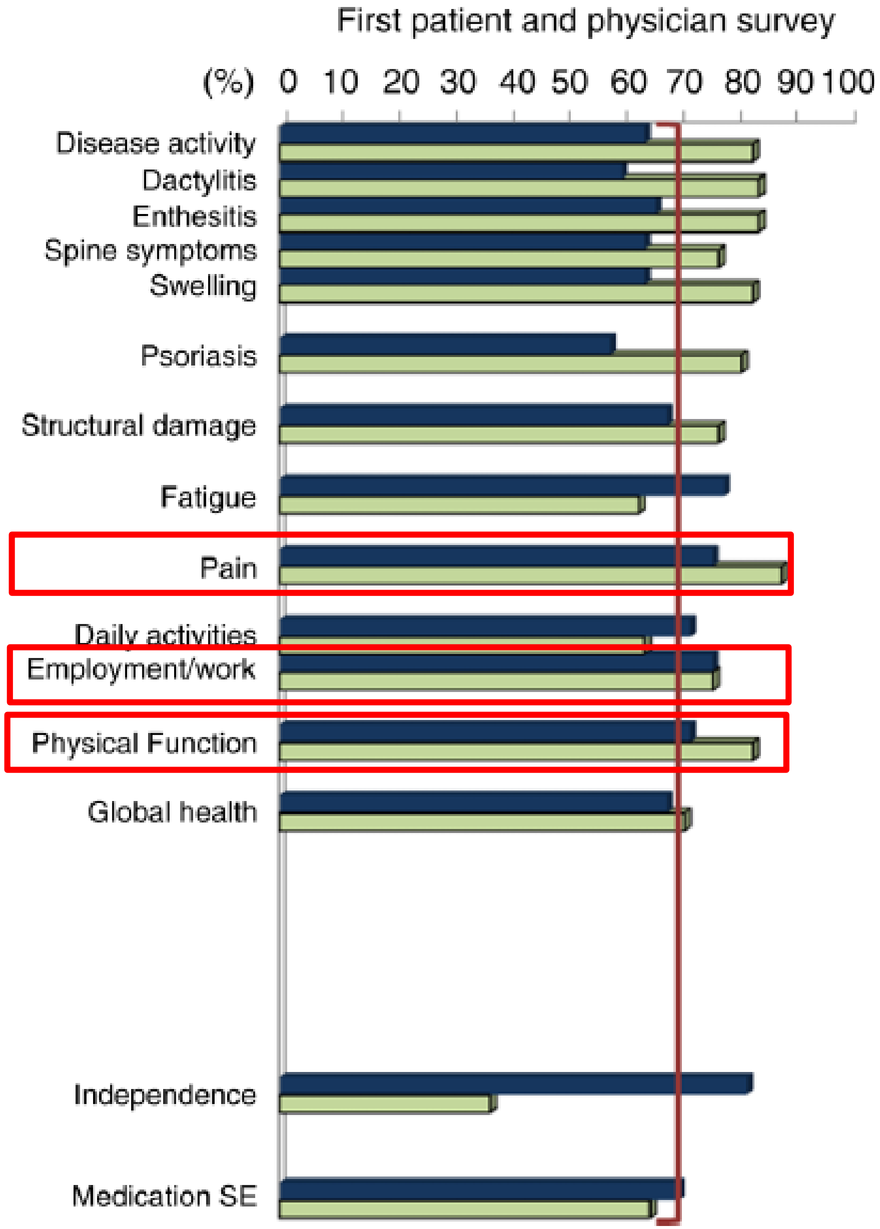
49 patients

Patient Delphi 2

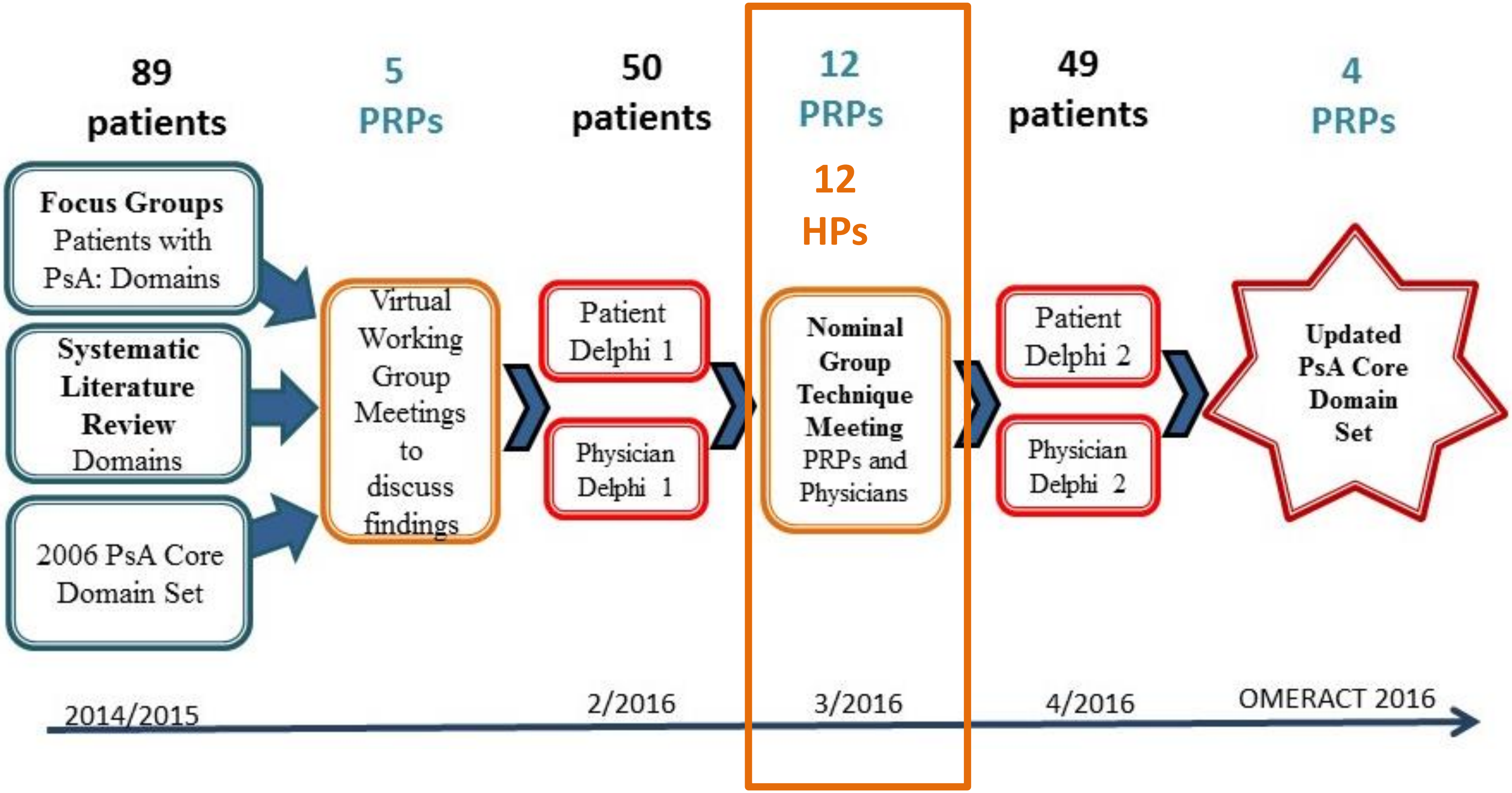
Physician Delphi 2

4 PRPs

Updated PsA Core Domain Set



Orbai AM ea, International patient and physician consensus on a psoriatic arthritis core outcome set for clinical trials. *Annals of the rheumatic diseases* 2016.



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Updated PsA Core Domain Set

2014/2015

2/2016

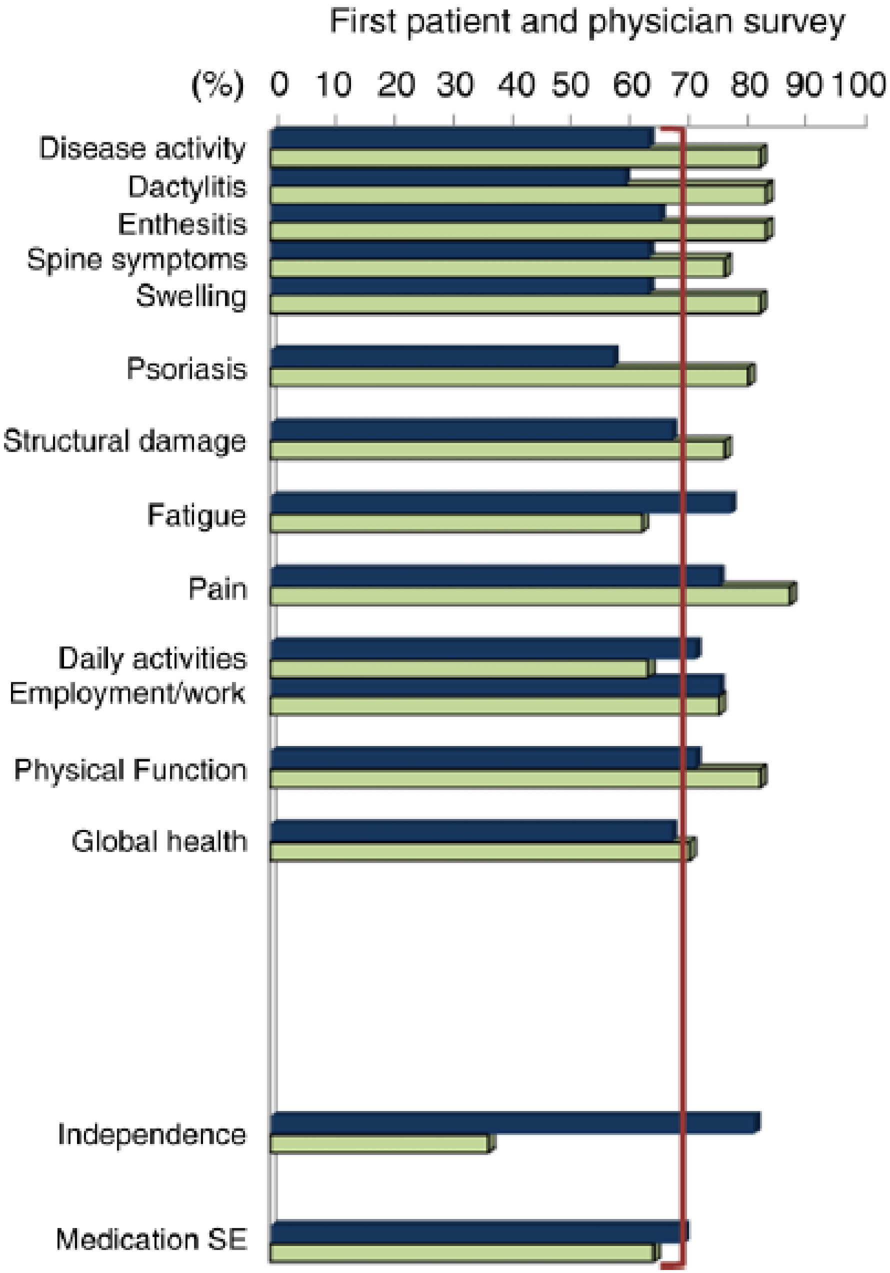
3/2016

4/2016

OMERACT 2016

- Full day consensus meeting
- Equal representation
- Independent facilitator
- Open dialogue

Preliminary consensus on the updated PsA core domain set



MSK disease activity

Skin disease activity

Structural damage

Fatigue

Pain

Participation

Physical Function

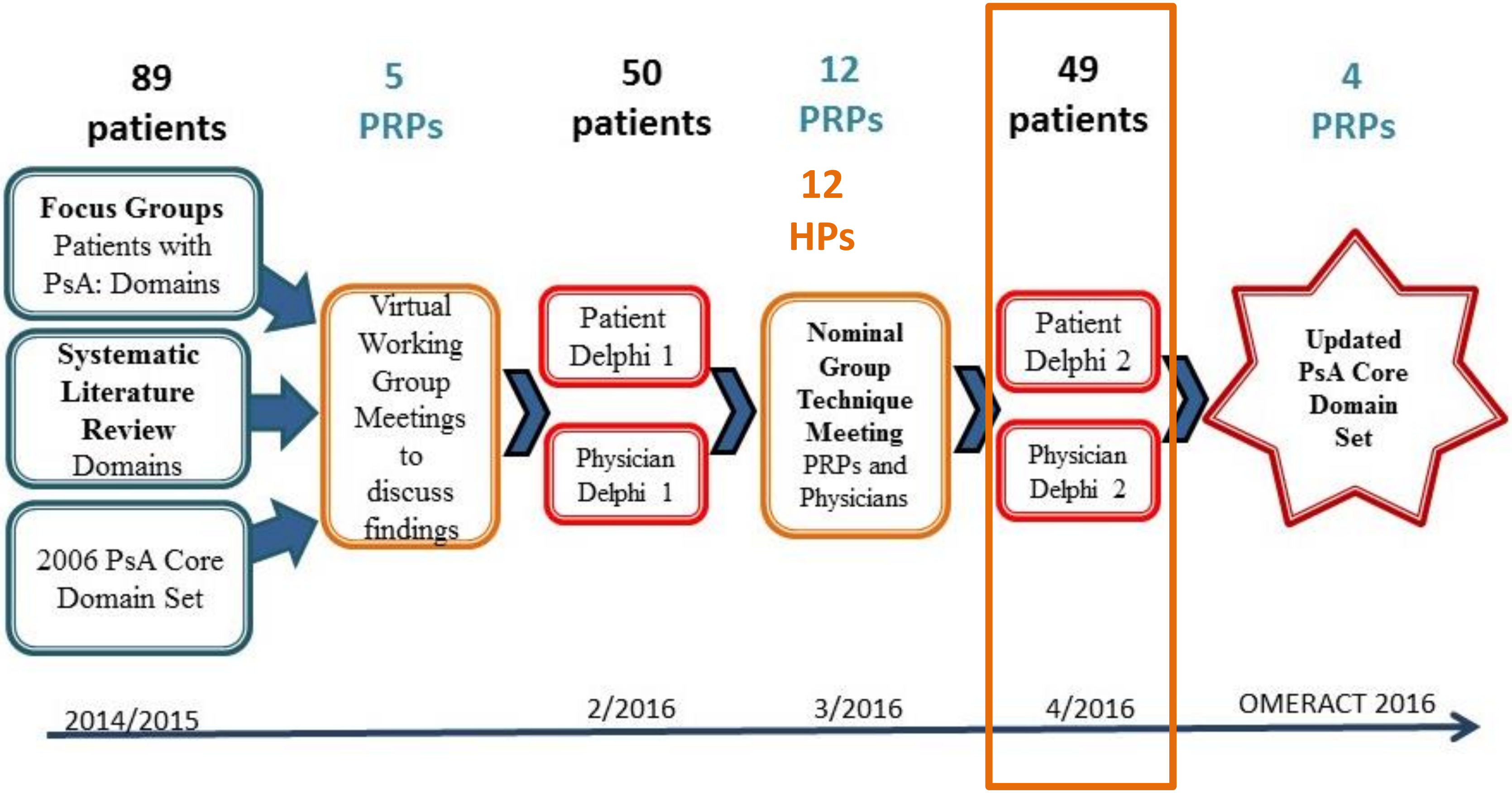
Patient Global

Systemic inflammation

Emotional well-being

Economic cost

Independence
Sleep
Stiffness
Treatment burden



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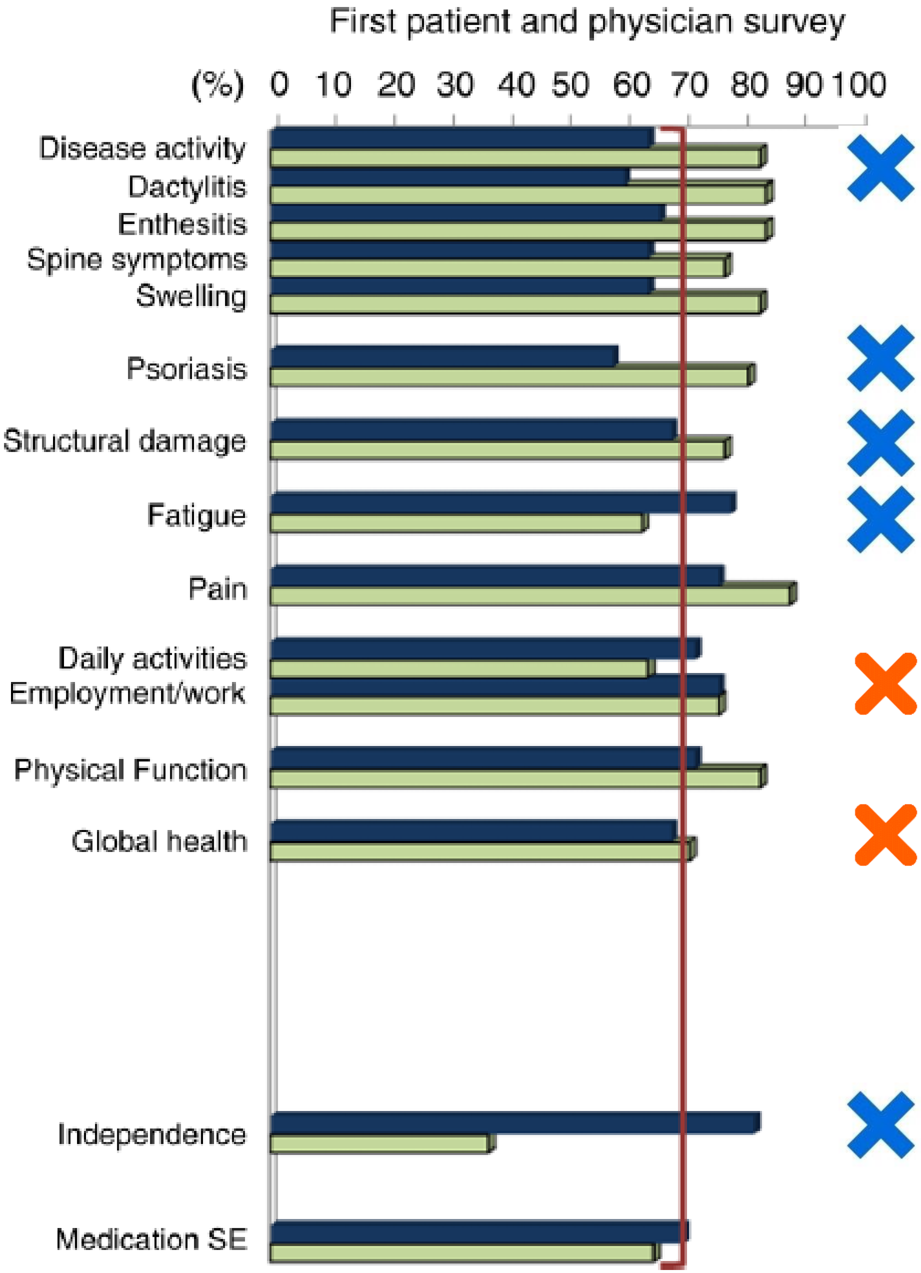
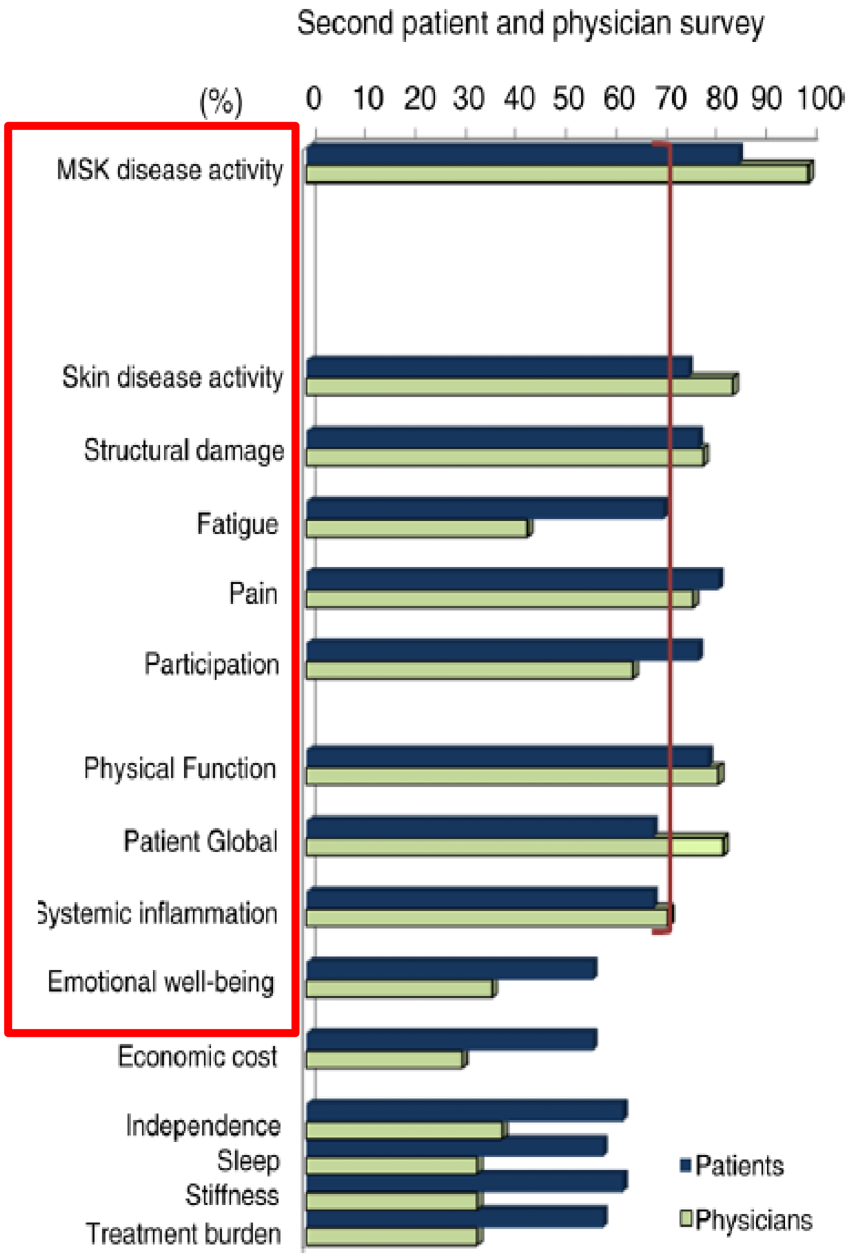
OMERACT 2016



First patient and physician survey



Second patient and physician survey

What has been the impact of patient and PRP involvement?

- 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.

How can this be explained?

Potential factors to look at….

- Risk of patient representatives aligning with physicians views and priorities?
- Influence of existing power imbalances?
- Influence of clinical relationships?
- Lack of proportional representation in numbers?

Conclusion

- PRP involvement is needed to ensure face validity of a core domain set.
- Attributes for an effective consensus meeting are:
  - Equal numbers of patients and other stakeholders
  - An independent facilitator
  - Open dialogue
  - Inclusion of opinions of all participants via consensus techniques (Nominal Group Technique).
- More research is needed to explore strategies to preserve the patients' perspectives in the consensus building process and final core domain set.

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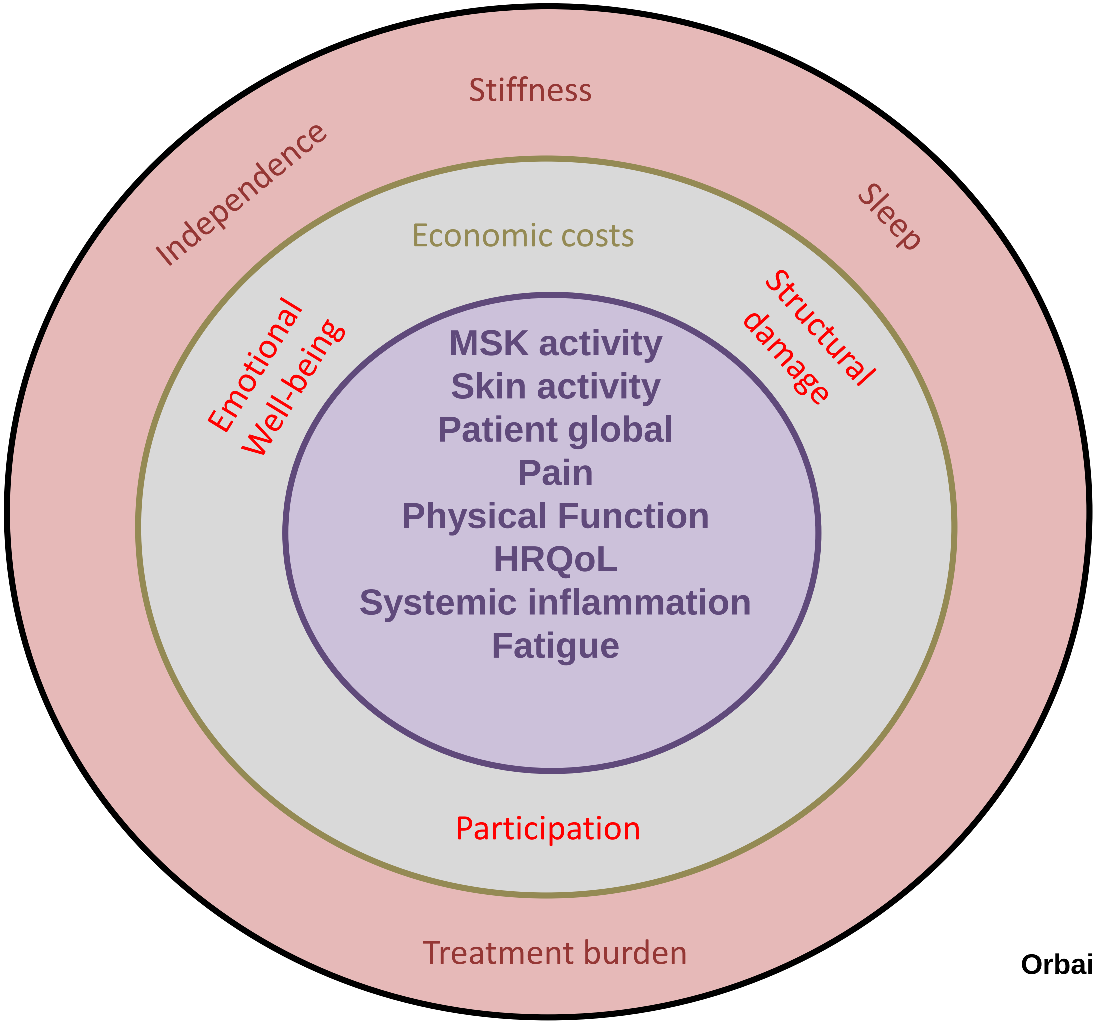
2013

2014

2015

2016



Stiffness

Independence

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Economic costs

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Structural
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OMERACT meeting
2016

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martinusdewit@hotmail.com