



The OMERACT Initiative PRO experience

Peter Tugwell

1992-2013

OMERACT
Outcome Measures in
Rheumatology

“Trials are only as good as their endpoints”

OMERACT

Outcome Measures in Rheumatology

- 12 International Meetings since 1992
- Over 40 topics
- Over 300 papers
- Over 3000 Citations
- Outcomes adopted by trialists
- Approval Agencies [EG FDA [and Canada] ,Europe , Australasia
- Cochrane Collaboration
- ICF

The OMERACT Filter

[TDF : 'To Die For']

- **TRUTH:**

Face, content, construct and criterion validity

Is the measure truthful, free from bias, relevant?

Does it measure what is intended?

Does it show convergence with appropriate variables and divergence between groups?

Can it be compared with a gold standard measure?

- **DISCRIMINATION:**

Reliability, reproducibility and sensitivity to change

Is it internally consistent and stable?

Does the measure discriminate between states of interest –
at one timepoint, different timepoints?

- **FEASIBILITY:**

Can the outcome easily be measured given constraints of time and costs?

Is it easy to score and interpret?

The OMERACT 'Umbrella'

1. Ankylosing Spondylitis
2. Fatigue
3. Gout
4. JIA :
5. Myositis
6. Osteoarthritis
7. Pain
8. PMR

9. PsA
10. Rheumatoid Arthritis:
 - ACR20/50/70/n
 - Flare
11. SLE:
12. Vasculitis
13. Worker Productivity

How does OMERACT work?

Achieving consensus over measures involves:

■ Content

- Education in methodology

- Agreeing on:

 - Purpose

 - Domain(s)

 - Applicability of specific measures

- Iteration

How does OMERACT work?

Achieving consensus over measures involves:

■ Content

■ Process

■ Data-driven

■ Iterative, stepwise

■ Inclusivity

● **Patients treated as equals**

● Important role for dissenters

● Harsh data softened by political considerations

The Journal of Rheumatology 2005; 32:11

OMERACT 7 Workshop

Incorporating the Patient Perspective into Outcome Assessment in Rheumatoid Arthritis — Progress at OMERACT 7

JOHN R. KIRWAN, SARAH E. HEWLETT, TURID HEIBERG, ROD A. HUGHES, MAGGIE CARR, MAGGIE HEHIR, TORE K. KVIEN, PATRICIA MINNOCK, STANTON P. NEWMAN, ENID M. QUEST, ERIK TAAL, and JANNEY WALE

OMERACT Conferences

- **OMERACT 1:** Maastricht, Netherlands [RA]
- **OMERACT 2:** Ottawa, Canada
- **OMERACT 3:** Barrier Reef , Australia
- **OMERACT 4:** Cancun , Mexico
- **OMERACT 5:** Toulouse, France
- **OMERACT 6 :** Brisbane, Australia
 - Patient Group Formalised;
- **OMERACT 7:** Asilomar, USA
- **OMERACT 8:** Malta
- **OMERACT 9:** Kananaskis , Canada
- **OMERACT 10:** Kota Kinabalu Borneo
- **OMERACT 11:** June 2012 Pinehurst USA
- **OMERACT 2014 ;** May 7-11 2014 Budapest Hungary
- **OMERACT 2016 :**Canada -suggestions welcome !



SAVE THE DATE

OMERACT 12

**Budapest, Hungary Hilton Budapest May 7 – 11,
2014**

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Rheumatoid Arthritis: OMERACT I, 1992

- Identified important 'domains' for assessment in RCTs.
- Facilitated development of ACR Response Criteria, Requires $\geq 20\%$ improvement in 5 of 7 measures
- **Q: WHAT ENDPOINTS DOES THE ACR 20 CONSIST OF?:**

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Rheumatoid Arthritis: OMERACT I, 1992

- Identified important ‘domains’ for assessment in RCTs.
- **Facilitated development of ACR Response Criteria, Requires $\geq 20\%$ improvement in 5 of 7 measures:**
 - 1.Tender Joint Count
 - 2. Swollen Joint Count
 - and 3 of the following 5:
 - I. **Pain by VAS**
 - II. **Patient Global**
 - III. **Physical Function: HAQ**
 - IV. MD Global
 - V. ESR and/or CRP

Three of the above are PROs [Patient Reported Outcomes]

Rheumatoid Arthritis: OMERACT I, 1992

- Identified important 'domains' for assessment in RCTs.
- Facilitated development of ACR Response Criteria, Requires $\geq 20\%$ improvement in 5 of 7 measures:
 - 1. Tender Joint Count
 - 2. Swollen Joint Count
 - and 3 of the following 5:
 - MD Global
 - Pain by VAS*
 - Patient Global *
 - Physical Function: HAQ [QOL-eg SF36] *
 - ESR and/or CRP
 - PLUS Xrays in >48 weeks
- **Emphasized importance of PRO [Patient Reported Outcomes] data when evaluating promising therapies**
- **Facilitated Guidance Documents from EMEA and FDA which led to approval of 10 new DMARDs in RA from 1998 – 2010.**

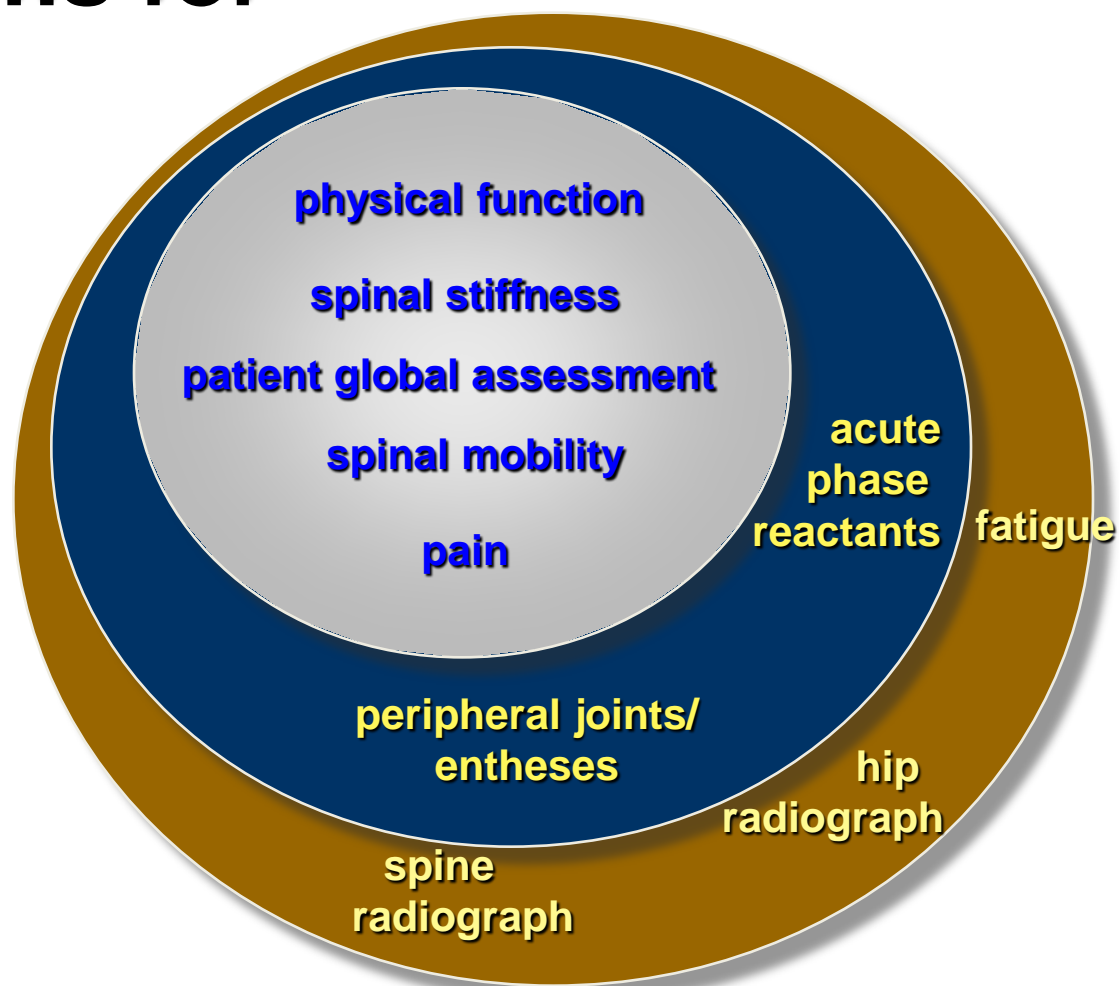
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 - 10.2.Flare
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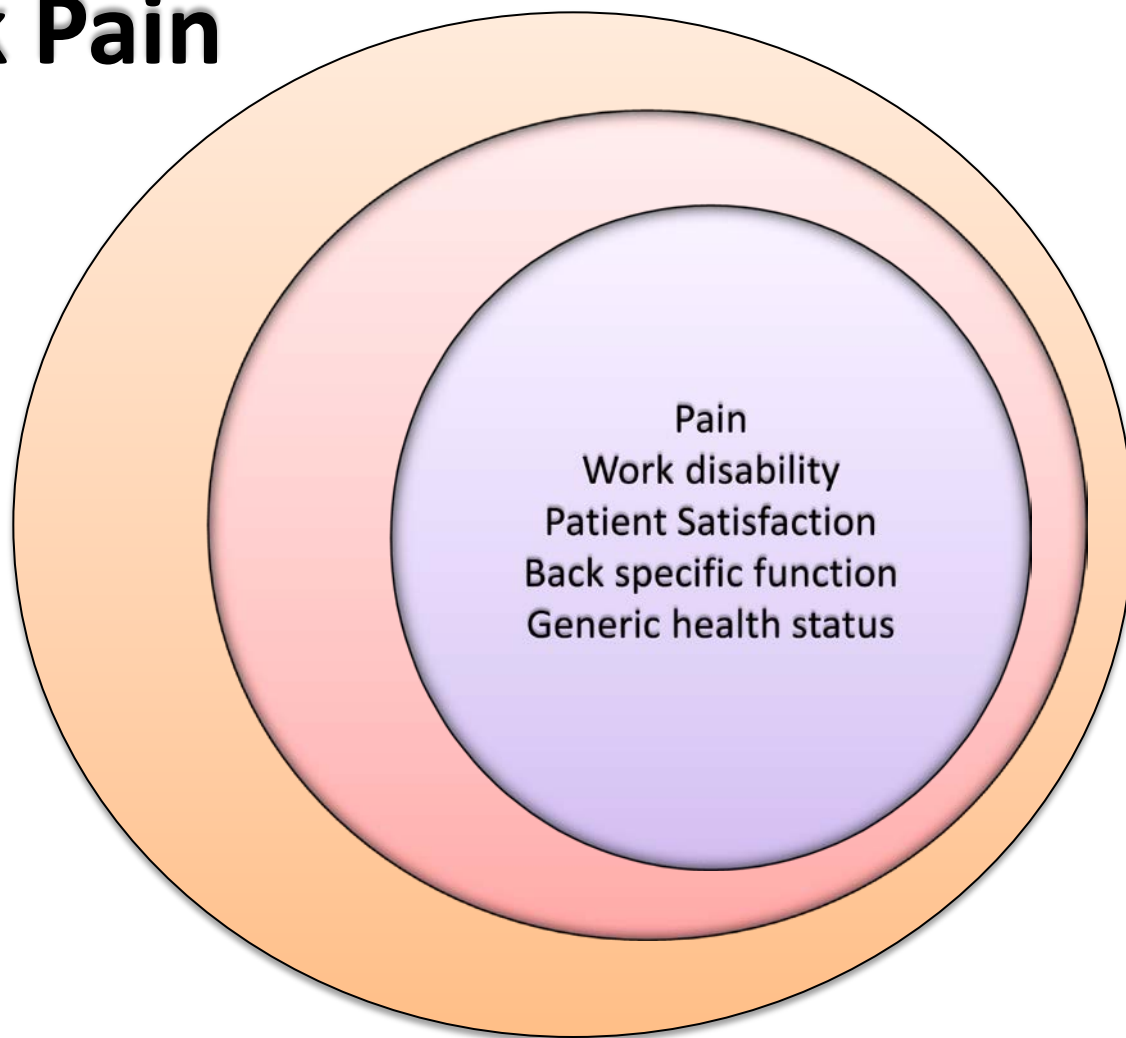
Example: ASAS/OMERACT

Core Domains for Ankylosing Spondylitis



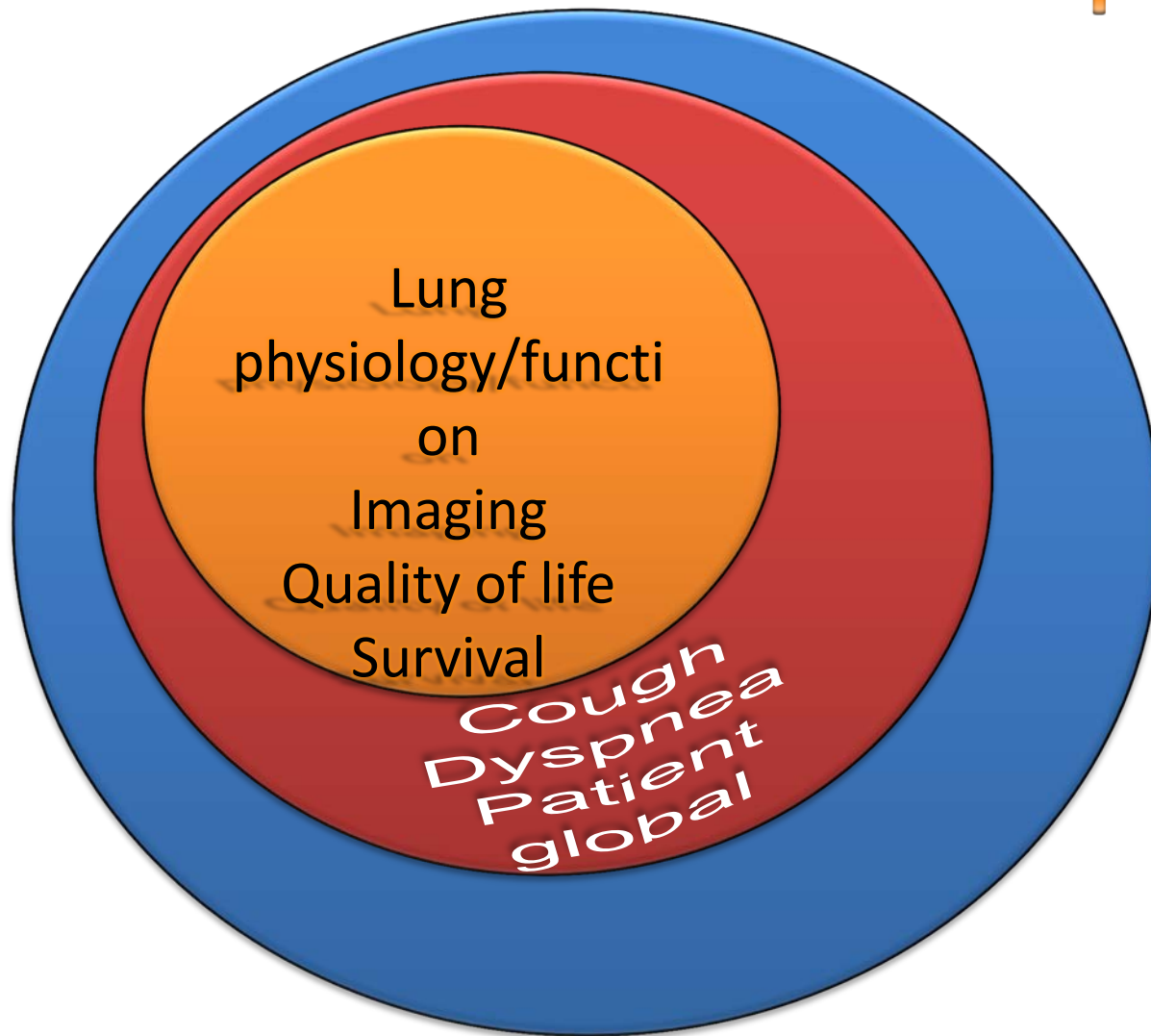
Example: Core Set

Back Pain

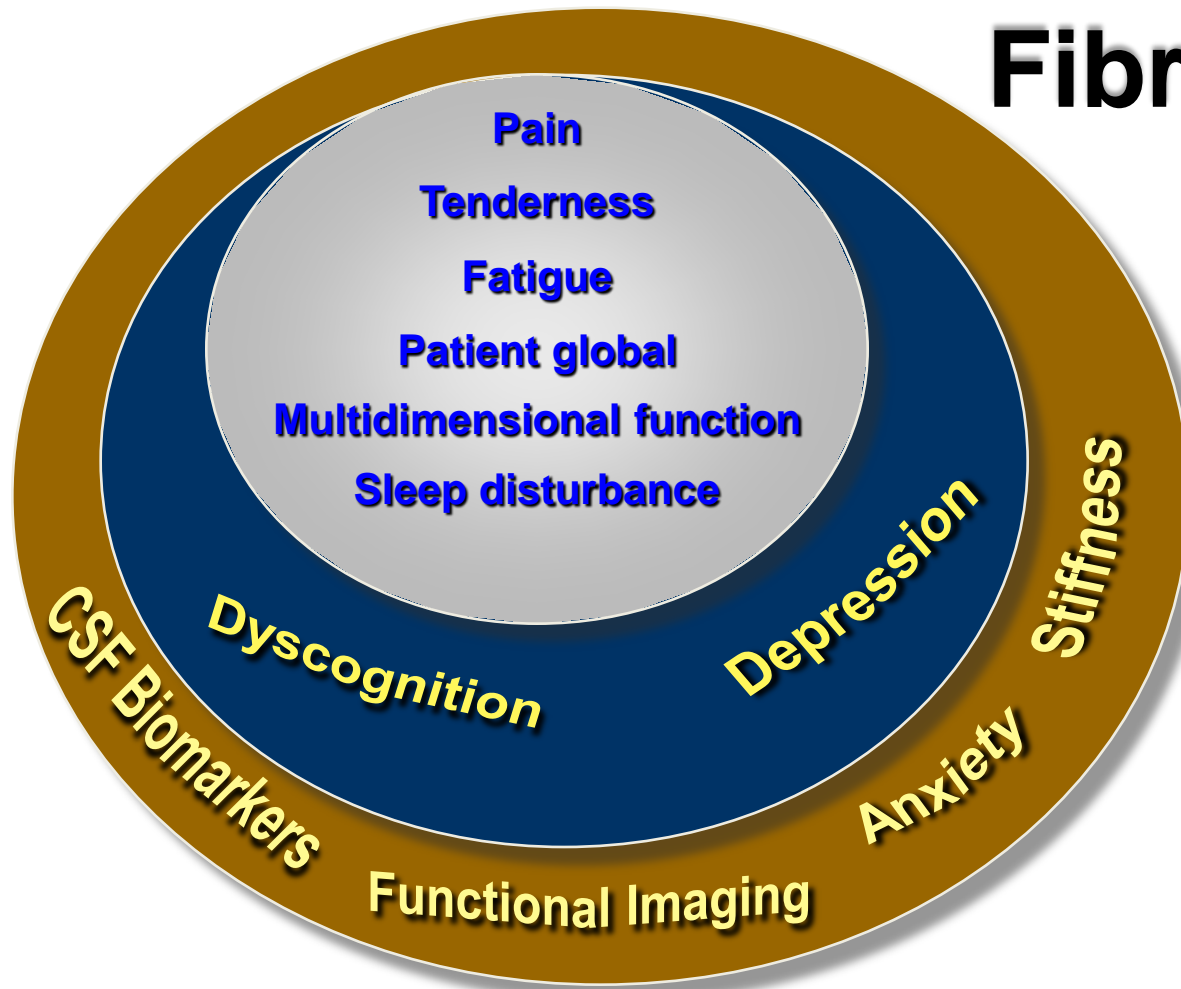


Example: Core Set

CTD-ILD

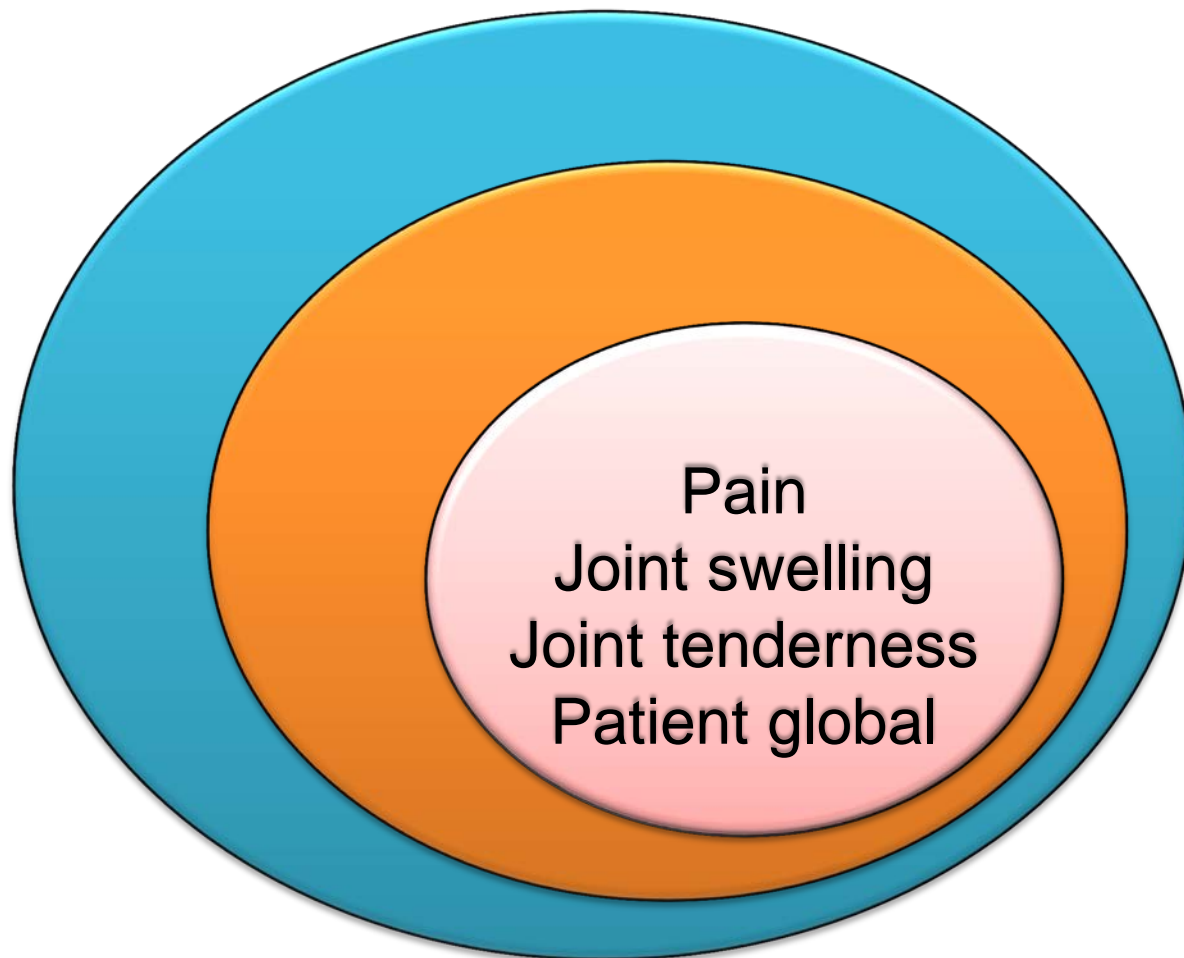


Core domains for Fibromyalgia

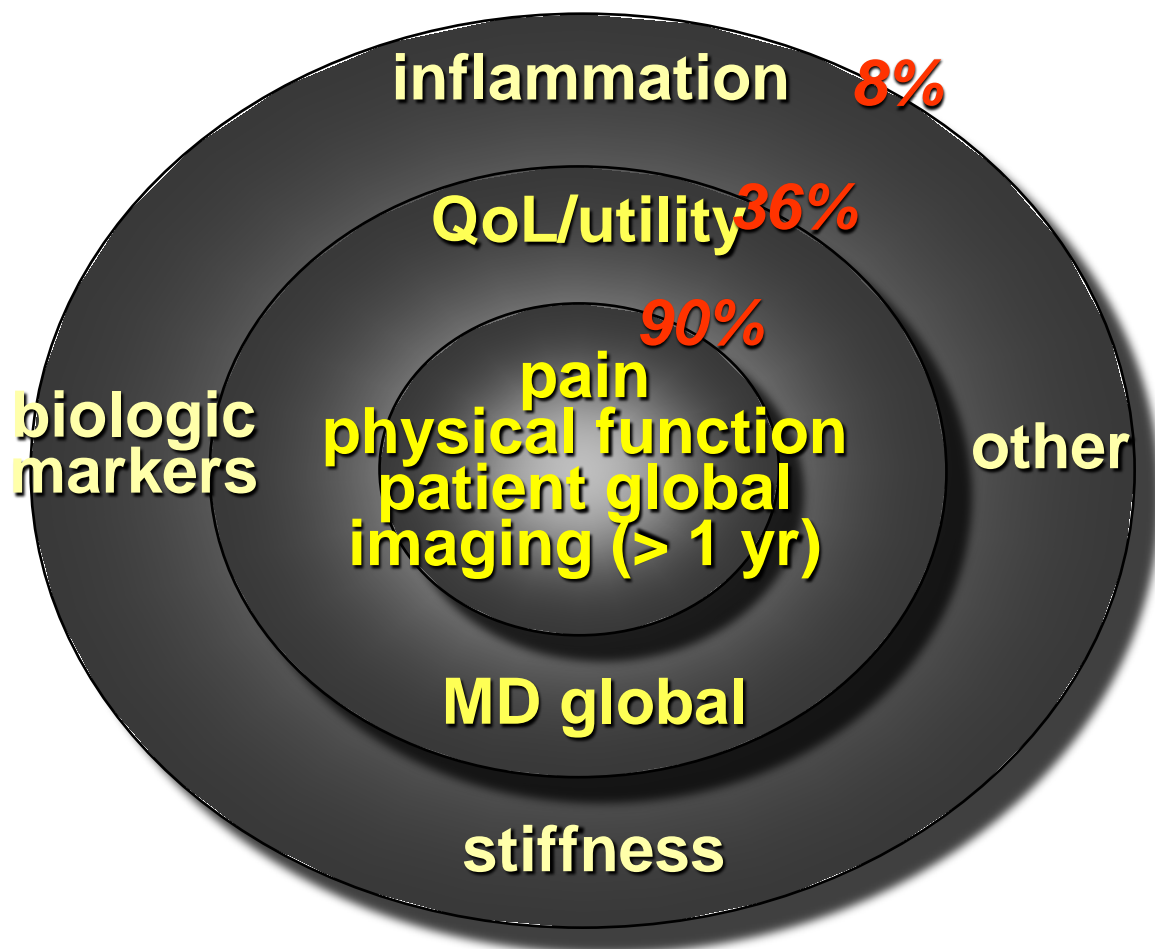


Example: Preliminary Core Set

Acute Gout

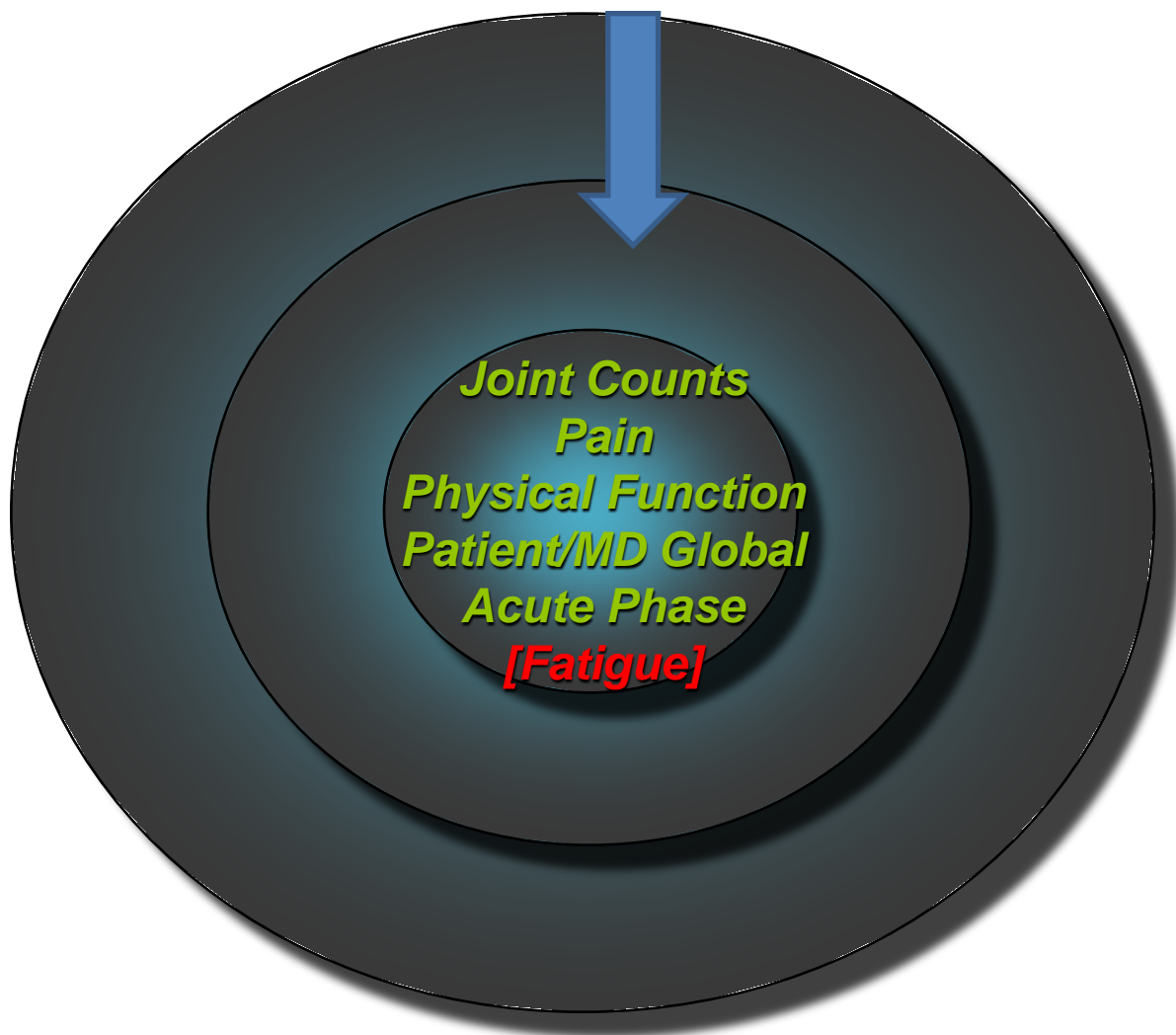


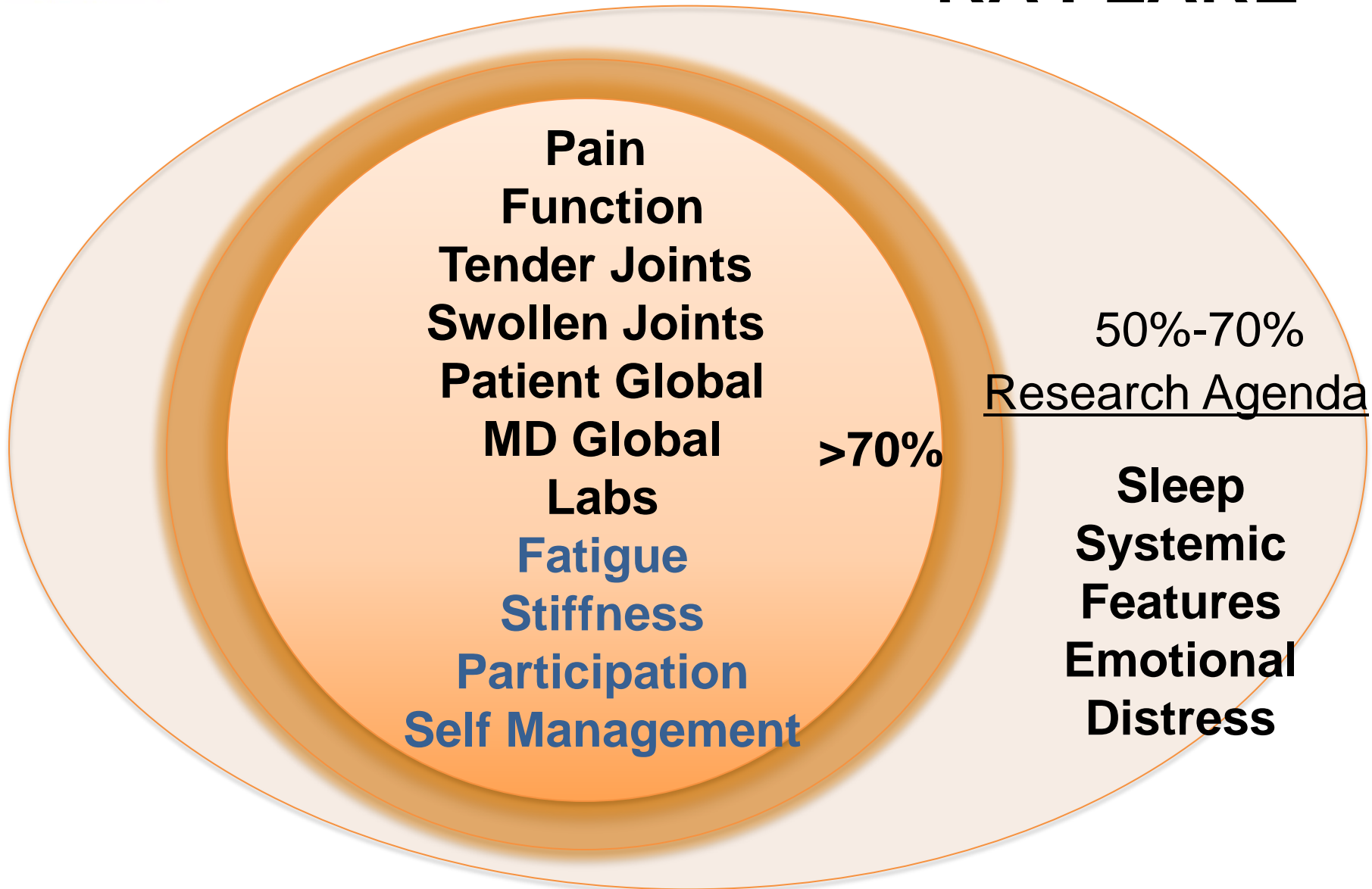
Example: Core Set Osteoarthritis



RHEUMATOID ARTHRITIS

OMERACT/ACR Outcome Core Set: Benefit

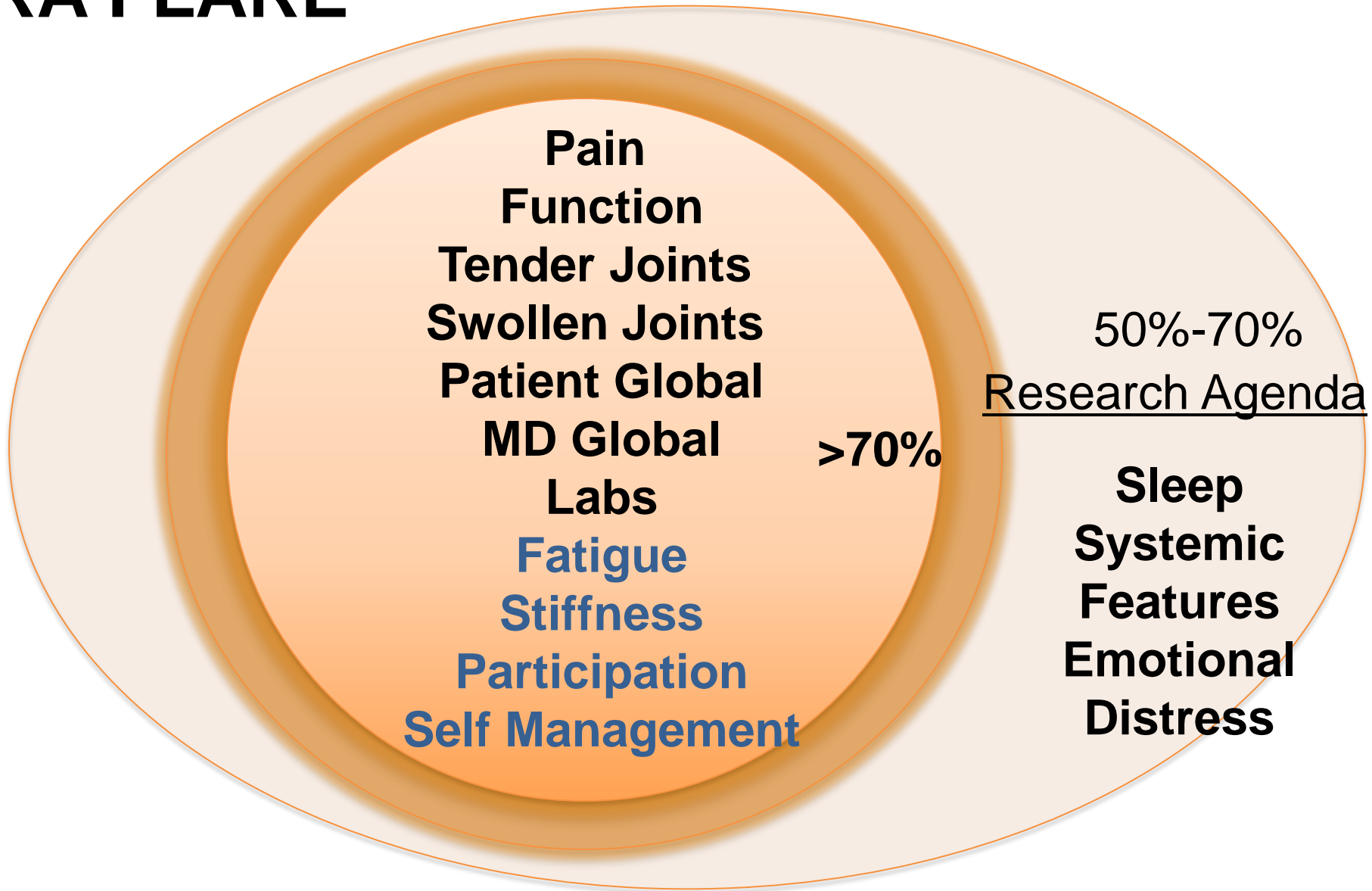




Flare: Our Process Has Involved Multiple Stakeholders (> 250 Individuals)

- **“HCPs”**
 - Clinicians (Rheumatologists, Nurses, PT, Allied Health Professionals) involved in Care of patients with RA
 - Researchers and Methodologists involved in RA outcomes
- **People living with RA**
 - Patients and patient research partners
- **Worldwide representation (23 countries)**

RA FLARE



Pain
Function
Tender Joints
Swollen Joints
Patient Global
MD Global
Labs
Fatigue
Stiffness
Participation
Self Management

>70%

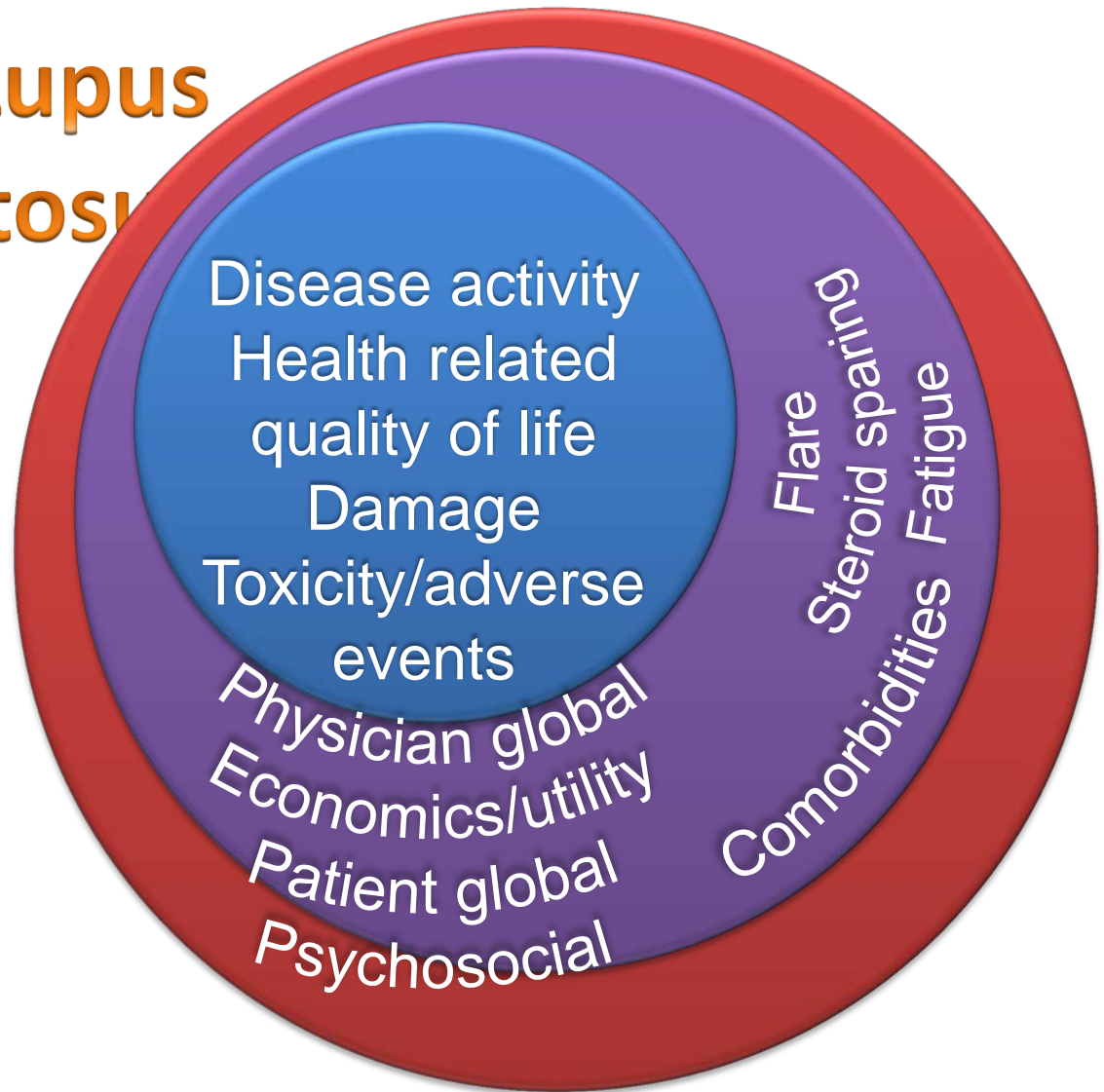
50%-70%
Research Agenda

Sleep
Systemic
Features
Emotional
Distress

Example: Core Set

Systemic Lupus

Erythematosus



Vasculitis Assessment

Activity

BVAS, BVAS 1 & 2
BVAS/WG, BVAS 3
DEI, FFS, VAI, JVAS

Damage

VDI, AVID, CDA

Function

SF 36
AVQOL
PROMIS/PROs

UPDATING The OMERACT Filter 1.0

The OMERACT Filter 1.0

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The OMERACT Filter 2.0

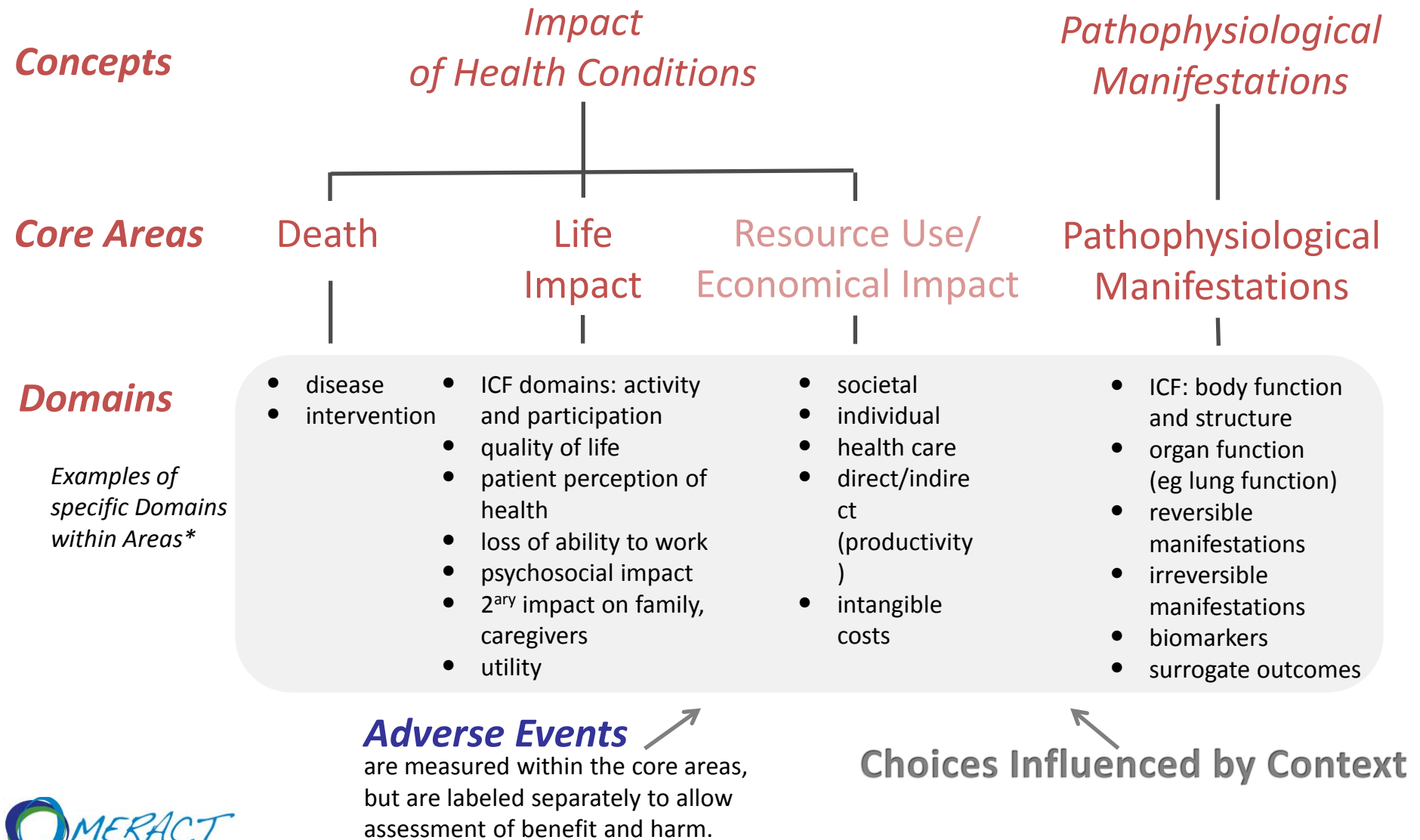
[TDF : 'To Die For']

5 Steps

1. Select and Validate Domains
2. **Then** seek endorsement of OMERACT
3. **Then** examine/develop instruments/measures
4. **Then** test the Core Set for Truth, Discrimination, Feasibility
5. **Then** seek endorsement of OMERACT for the Core Set

The OMERACT Filter 2.0

[Select and validate Domains]



Other linked PRO projects



- Cochrane Decision Aids

Decision aids for people facing health treatment or screening decisions (Review)

Stacey D, Bennett CL, Barry MJ, Col NF, Eden KB, Holmes-Rovner M, Llewellyn-Thomas H, Lyddiatt A, Légaré F, Thomson R



**THE COCHRANE
COLLABORATION®**



COCHRANE REVIEW DECISION AIDS

<http://musculoskeletal.cochrane.org/Decision-aids>

- Osteoarthritis

- [What are my options for managing hip or knee arthritis?](#)
- [Should I use TENS \(transcutaneous electrostimulation\) for osteoarthritis?](#)
- [Should I have ultrasound for osteoarthritis?](#)

- Osteoporosis

- [Should I take alendronate \(Fosamax[®]\) to prevent fractures?](#)
- [Should I take etidronate \(Didronel[®]\) to prevent fractures?](#)
- [Should I take risedronate \(Actonel[®]\) to prevent fractures?](#)

- Rheumatoid arthritis

- [Should I take abatacept \(Orencia[®]\) for rheumatoid arthritis?](#)
- [*Should I take etanercept \(Enbrel[®]\) for rheumatoid arthritis?*](#)
- [Should I take tocilizumab \(Actemra[®]\) for rheumatoid arthritis?](#)
- [Should I take methotrexate \(Rheumatrex[®]\) for rheumatoid arthritis?](#)
- [Should I take methotrexate \(Rheumatrex[®]\) for rheumatoid arthritis alone or with other disease-modifying anti-rheumatic drugs \(DMARDs\)?](#)



Cochrane Review Etanercept Decision Aid

- Take etanercept (Enbrel®): Once or twice a week, you have an injection under the skin in different parts of the thigh or abdomen. A nurse or doctor teaches you how to do this. A family member or friend can also learn. You store the drug in a refrigerator and warm it to room temperature prior to use. Most people who fear self-injection are able to give these injections with mild or no discomfort.
- Decline etanercept (Enbrel®). You may wish to discuss other treatment options with your doctor



Cochrane Review Etanercept Decision Aid

Step 1:

What are the benefits ?

Benefits ++

If people take etanercept (Enbrel®) along with their usual drugs for 1 year, 29 more of them will **have a major improvement in the pain, disability, and number of tender and swollen joints**. NNT =3*
(Improvement was measured by ACR 50 meaning that the people had at least 50% (major) improvement in their RA)

If people take etanercept (Enbrel®) for 10 years, 11 fewer people will have **serious joint damage** as seen on X-rays.



Take etanercept (Enbrel®) injection plus usual drugs



Take placebo injection plus usual drugs

50 Improve

50 Don't improve



21 Improve

79 Don't improve



96 Avoid permanent joint damage

4 get joint damage



85 Avoid permanent joint damage

15 get joint damage



Blocks of 100 faces show a 'best estimate' of what happens to **100 people** who either take etanercept (Enbrel®) or placebo (fake treatment). Each face (☹) stands for one person. The shaded areas show the number of people affected.



Step 1: Side Effects and Harms

Side Effects ++

Etanercept causes 3 more people to **stop treatment** due to side effects such as a skin reaction. Reactions include rash, burning, or itching at the injection site. This may last 10 to 14 days without leaving a scar.

13 Stop treatment

10 Stop treatment

87 Stay on treatment

90 Stay on treatment



Serious Harms +

If **10,000** people take etanercept (Enbrel®), 16 more people may get **tuberculosis** from taking it. Before starting etanercept, a TB test is usually done. The drug is stopped if people have a high fever or have an active infection and are taking an antibiotic.

9,980 Avoid tuberculosis

9,996 Avoid tuberculosis

20 Get tuberculosis

4 Get tuberculosis



Cochrane Review Etanercept Decision Aid

Step 3: What else do you need to prepare for decision making?

Find out how well this decision aid helped you learn the key facts.

Check the best answer.

| | Take etanercept (Enbrel®) | Decline etanercept (Enbrel®) | I don't know |
|---|---------------------------------|------------------------------------|--------------------------|
| 1. Which option <u>raises</u> your chance of improving pain, dysfunction, and the number of swollen joints? | <input type="checkbox"/> | <input type="checkbox"/> | <input type="checkbox"/> |
| 2. Which option <u>lowers</u> your chance of serious joint damage? | <input type="checkbox"/> | <input type="checkbox"/> | <input type="checkbox"/> |
| 3. Which option has a greater risk of skin reactions? | <input type="checkbox"/> | <input type="checkbox"/> | <input type="checkbox"/> |
| 4. Which option has the <u>lowest</u> chance of tuberculosis? | <input type="checkbox"/> | <input type="checkbox"/> | <input type="checkbox"/> |

Check your answers at the bottom of the page

Find out how comfortable you feel about deciding



Do you know enough on the benefits and harms of each option to make a choice?

Yes

No



Are you clear about which benefits and harms matter most to you?



Do you have enough support and advice to make a choice?



Do you feel sure about the best choice for you?

If you answered 'No' to any of these, discuss with your practitioner. (Adapted SURE Test © O'Connor & Légaré)



Cochrane Review Etanercept Decision Aid

Step 4: What are the next steps?

Check what you want to do next...

- Try etanercept (Enbrel®)
- Discuss other treatment options
- Other, please specify:

Step 3 answers: Questions 1, 2 and 3: Take etanercept (Enbrel®) Question 4. Decline etanercept (Enbrel®)



Strategy

Improve the individual experience by providing exceptional care and service to customers that is

Key 2010-11 Actions

- Develop and implement a provincial framework for patient- and family-centered care that will serve as an overarching guide for health care service delivery in Saskatchewan.
- Develop and implement a **Shared Decision Making** framework which will inform and engage patients in decisions about their treatment options, including surgical and non-surgical treatments.



Budapest, Hungary Hilton Budapest May 7 , 2014

**Partnerships Consensus PRO Meeting –COCHRANE,
OMERACT, IMMPACT, PROMIS, COMET**

Thank you!



