REiNS - Enhancing Patient Engagement in Neurofibromatosis Clinical Trials

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Washington
December 5, 2016

Increasing patient engagement in arthritis studies: the OMERACT experience
REiNS objectives

1. To educate neurofibromatosis researchers and clinicians about the utility of increased patient engagement in developing endpoints for clinical trials

2. To engage patients, family members, and other stakeholders in the NF community in the REiNS collaboration

3. To generate a roadmap for developing patient engagement guidelines in REiNS subgroups
Overview

Part 1
To educate neurofibromatosis researchers and clinicians about the utility of increased patient engagement in developing endpoints for clinical trials

Part 2
How to engage patients, family members, and other stakeholders in the NF community in the REiNS collaboration

Part 3
To generate a roadmap for developing patient engagement guidelines in REiNS subgroups
Part I

“Researchers can easily overlook the complexity and capriciousness of living with a chronic disease, reducing the meaning of life experiences to abstract themes and models”. *

The patient as passive recipient, following doctor’s prescription

One way communication

Patient is study participant

The patient as an equal partner in the decision making process

Two way communication

Patient as collaborative partner
Why actively involving patients in health research?

- Ethical argument
  In a democracy patients have the right to speak for themselves

- Content argument
  Experience based knowledge makes research outcomes more relevant and fitting better with the context of daily life

- Political argument
  Legitimacy and chances for implementation increase

- Empowerment of patients and researchers

Abma & Broerse
2007
Biannual worldwide conference on: Outcome Measurement in Rheumatology Clinical Trials

❄ First conference in 1992 in Maastricht
❄ Aim: To achieve consensus about endpoints for clinical trials in rheumatology
❄ Characteristics:
  ❄ Data-driven, iterative consensus process
  ❄ Inclusive, interactive, non-commercial
  ❄ A broad stakeholders approach

OMERACT agrees ‘core sets’ for measuring outcome in rheumatic diseases

At OMERACT 1 (1992) the core set for rheumatoid arthritis (RA) was agreed

- Pain
- Swollen joints
- Tender joints
- Physician global assessment
- Patient global assessment
- Physical function
- Acute phase response

Why involving patients in outcome research?

- In 2000 OMERACT participants discussed the definition of a ‘clinically important change’ in response to treatment.
- In the final voting session participants agreed that this questions could only be answered by patients.

For OMERACT 6 (2002) 11 patients were invited to review the OMERACT core set.
Involving patient research partners has a significant impact on outcomes research: a responsive evaluation of the international OMERACT conferences

Maarten de Wit, John A Gatsonis, Koelewijn-van Loon, Sarah Collins


ARTICLE SUMMARY

Since 2002, patients have participated as collaborative partners in the biannual conference on Outcome Measures in Rheumatology (OMERACT).

Although the contribution of patients has been praised and there is a widespread call for scientific publications on the impact of engaging with patients, no systematically obtained evidence has been published to support the idea that the structural involvement of patients in research conferences is beneficial.

Our qualitative study reports the combined results of a thematic document analysis and 32 semistructured interviews with all stakeholders including researchers, patient participants and
The example of fatigue in RA

Early descriptions at OMERACT 6 & 7 led to substantial qualitative research establishing the importance of RA fatigue

“Fatigue is overwhelming and different from normal tiredness; it permeates every sphere of life; and self-management is variable, but professional support is rare”


Qualitative work showed that measuring fatigue adds new information to the existing core set for RA.
Contribution of different variables to measuring rheumatoid arthritis

Red: Variance in measure that can be accounted for (explained) by variation in the other measures
Contribution of different variables to measuring rheumatoid arthritis

Green: Variance in measure that cannot be explained by variation in the other measures

Red: Variance in measure that can be accounted for (explained) by variation in the other measures

John Kirwan, 2006
Result

- Fatigue was added to the core-set, to be included as outcome in every new clinical trial in RA.
- More powerful instruments for measuring fatigue in RA have now been devised, and are increasingly used in clinical trials.

This would never have happened without the direct involvement of patient delegates in the OMERACT process.
“We were first discussing on fatigue and to be honest I never ever had before heard of fatigue being a problem in rheumatology. So it got into my mind and then I got thinking about it and then, when I was back, I asked patients if they felt fatigue and I got nearly a 100% positive response. So it was like a coming out, you know. I listened to the patients before but bringing it to a specific topic, that was really what I learned at OMERACT.” [RC]
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Conclusion

“Clinical trials are only as credible as their endpoints” *

* Peter Tugwell & Maarten Boers, The Journal of Rheumatology 1993; 20:3
Core domain set development according to the OMERACT filter 2.0

OMERACT disease specific core domain sets

**Concepts**
- Impact of Health Conditions
- Pathophysiological Manifestations

**Core Areas**
- Death
- Life
- 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
  - 2\textsuperscript{any} impact on family, caregivers
  - utility
  - societal
  - individual
  - health care
  - direct/indirect (productivity)
  - intangible costs
  - ICF: body function and structure
  - organ function (e.g., 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

Stakeholder input

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

Draft Core Domain Set

consensus

Match Domains to Core Areas

Literature review List of Domains & Instruments

Source:
OMERACT Handbook
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
COMET– Core Outcome Measures in Effectiveness Trials

Core outcome set (COS) is an agreed standardized set of outcomes to be measured and reported as a minimum in all trials in a specific health-related area.*

COMET database is an international repository of studies relevant to the development of COS, planned, ongoing and completed.**

** www.comet-initiative.org
Fig 3. Number of COS developed in each disease category (n = 227).

doi:10.1371/journal.pone.0146444.g003
Acute phase reactants
Ultrasound

Peripheral joint activity
Skin activity
Patient global
Pain
Physical Function
HRQoL

OMERACT-GRAPPA
Psoriatic Arthritis
core set

Peripheral joint activity
Skin activity
Patient global Pain
Physical Function
HRQoL

Why updating the PsA COS?

Core set discussed with patients

Peripheral joint activity
Skin activity
Patient global Pain
Physical Function
HRQoL
Dactylitis
Acute phase reactants
Tissue analysis
Enthesitis
Physician Global
Spinal
Nails
Ultrasound
MRI
CT
Radiology
Participation
Fatigue

Need to update the PsA core set with active patient involvement

A best practice of patient participation in COS development

Updating the core domain set for 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

- 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

- **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
  - Patient Delphi 1
  - Physician Delphi 1

- **50 patients**
  - Nominal Group Technique Meeting PRPs and Physicians
  - Patient Delphi 2
  - Physician Delphi 2

- **12 PRPs**
  - 49 patients

- **4 PRPs**

Work streams Update core outcome set for Psoriatic Arthritis
Following OMERACT recommendations

The Journal of Rheumatology


Peter P. Cheung, Maarten de Wit, Clifton O. Bingham 3rd, John R. Kirwan, Amye Leong, Lyn M. March, Pam Montie, Marieke Scholte-Voshaar, and Laure Gossec

ABSTRACT. Objective. Patient participation in research is increasing; however, practical guidelines to enhance this participation are lacking. Specifically within the Outcome Measures in Rheumatology (OMERACT) organization, although patients have participated in OMERACT meetings since 2002, consensus about the procedures for involving patients in working groups has not been formalized. The objective is to develop a set of recommendations regarding patient research partner (PRP) involvement in research working groups.
## What about PRPs Tasks?

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What about PRPs’ recognition?

Co-authorship

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PRP participation as integral part of research

Evolving Patient Roles

Steering group member

Patient research partners

Delphi participants

Focus group participants
New domains identified through SLR and the qualitative studies

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

Systemic inflammation
- Independence
- Stiffness
- Sleep
- Treatment burden
- Emotional wellbeing
- Economic costs

New domains identified through SLR and the qualitative studies

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

Systemic inflammation
Independence
Stiffness
Sleep
Treatment burden
Emotional wellbeing
Economic costs

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

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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

Patient Delphi 1

Physician Delphi 1

50 patients

12 PRPs

Nominal Group Technique Meeting PRPs and Physicians

Patient Delphi 2

Physician Delphi 2

12 HPs

49 patients

4 PRPs

Updated PsA Core Domain Set
• Full day consensus meeting
• Equal representation
• Independent facilitator
• Open dialogue

Preliminary consensus on the updated PsA core domain set
89 patients

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Updated PsA Core Domain Set

2014/2015

2/2016

3/2016

4/2016

OMERACT 2016
89 patients

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Updated PsA Core Domain Set

2014/2015

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3/2016

4/2016

OMERACT 2016
Emotional wellbeing
Participation
Systemic inflammation
Structural damage
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?
Part III

One says, "Well, this may be a revolutionary pill..."

The other responds, "But I'm not sure the public will be willing to swallow it..."


Brett J et al. The PIRICOM Study: A systematic review of the conceptualisation, measurement, impact and outcomes of patients and public involvement in health and social care research. The University of Warwick, 2009.


de Wit M, Elberse JE, Broerse JE, Abma TA. Do not forget the professional - the value of the FIRST model for guiding the structural involvement of patients in rheumatology research. *Health expectations*. 2015; 18: 489-503

De Wit M, Kvien T, Gossec L. Patient participation as an integral part of patient reported outcomes development guarantees the representativeness of the patient voice – A case-study from the field of rheumatology. RMD-open 2015;1.

Conditions for participation

- Participation is a process
- The role of the principal investigator is key in providing adequate support to patients
- Participation should always be tailor made, there is no concept that fits all
- It requires multiple forms of participation
- It requires always an extra effort: in time, money and energy
- A structural approach guarantees sustainability
- Willingness for mutual learning
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.
Thanks for your attention

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