



## **The HOME Roadmap:**

Paving the way to core outcomes for patient-reported symptoms in eczema trials

### **COMET meeting Nov 2016**

Joanne R. Chalmers, Louise A.A. Gerbens, Eric Simpson, Christian J. Apfelbacher, Kim S. Thomas, Cecilia A.C. Prinsen, Laura B. von Kobyletzki, Hywel C. Williams, Jochen Schmitt and Phyllis I. Spuls

- What is HOME
- What is the HOME roadmap
- How HOME followed the roadmap to agree a core outcome measurement instrument for patient-reported symptoms
- Similarities and differences between the HOME roadmap and the COSMIN/COMET guidance



## HOME = Harmonising Outcome Measures for Eczema

- Founded in 2010
- Multiple, unvalidated outcome measures for eczema severity
- Evidence synthesis
- Need for COS
- Evidence driven, evidence generating
- Consensus methodology
- Open membership
- OMERACT



*Professors Hywel Williams  
and Jochen Schmitt*



- HOME roadmap, 2014
- To provide framework for others working in COS in skin disease
- No published guidance on whole process
- Included current guidance:
  - OMERACT filter (version 1)
  - COSMIN quality criteria for assessing measurement properties of instruments

## The Harmonizing Outcome Measures for Eczema (HOME) Roadmap: A Methodological Framework to Develop Core Sets of Outcome Measurements in Dermatology

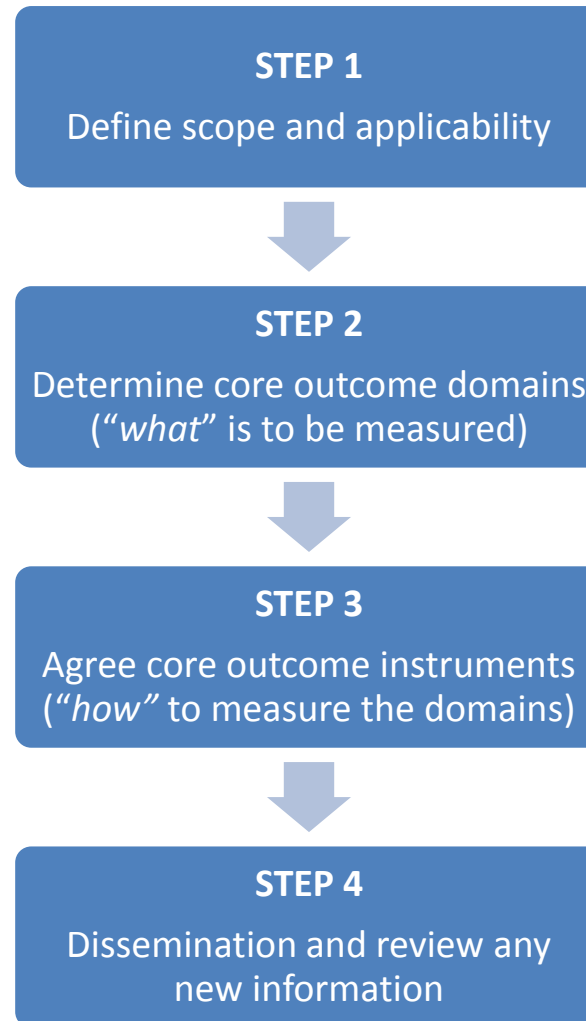
Jochen Schmitt<sup>1</sup>, Christian Apfelbacher<sup>2</sup>, Phyllis I. Spuls<sup>3</sup>, Kim S. Thomas<sup>4</sup>, Eric L. Simpson<sup>5</sup>, Masataka Furue<sup>6</sup>, Joanne Chalmers<sup>4</sup> and Hywel C. Williams<sup>4</sup>

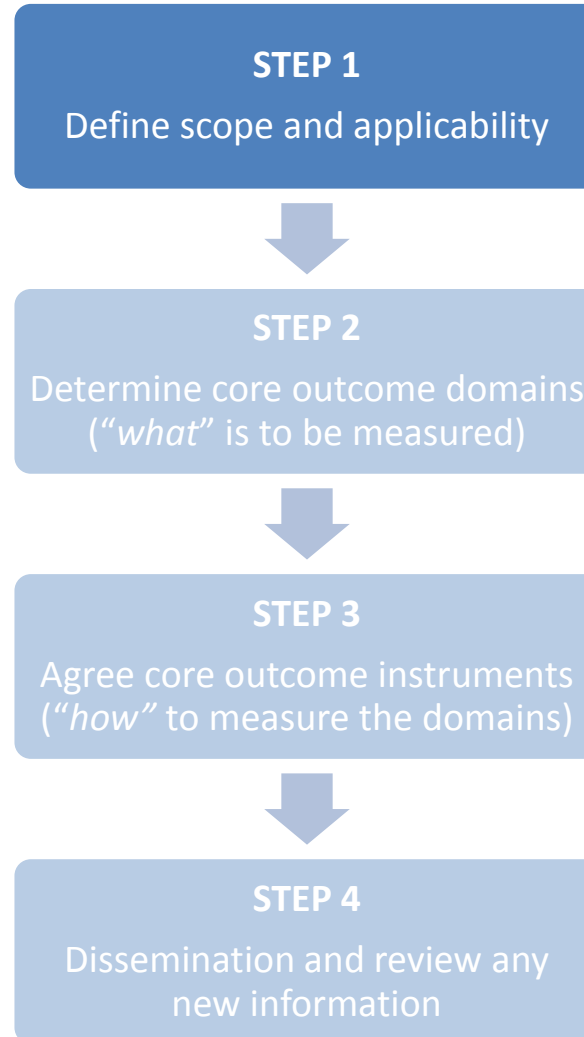
Core outcome sets (COSs) are consensus-derived minimum sets of outcomes to be assessed in a specific situation. COSs are being increasingly developed to limit outcome-reporting bias, allow comparisons across trials, and strengthen clinical decision making. Despite the increasing interest in outcomes research, methods to develop COSs have not yet been standardized. The aim of this paper is to present the Harmonizing Outcomes Measures for Eczema (HOME) roadmap for the development and implementation of COSs, which was developed on the basis of our experience in the standardization of outcome measurements for atopic eczema. Following the establishment of a panel representing all relevant stakeholders and a research team experienced in outcomes research, the scope and setting of the core set should be defined. The next steps are the definition of a core set of outcome domains such as symptoms or quality of life, followed by the identification or development and validation of appropriate outcome measurement instruments to measure these core domains. Finally, the consented COS needs to be disseminated, implemented, and reviewed. We believe that the HOME roadmap is a useful methodological framework to develop COSs in dermatology, with the ultimate goal of better decision making and promoting patient-centered health care.

*Journal of Investigative Dermatology* (2015) 135, 24–30; doi:10.1038/jid.2014.320; published online 4 September 2014

Schmitt *et al* **JID** (2014)

Covers whole  
process of  
COI selection

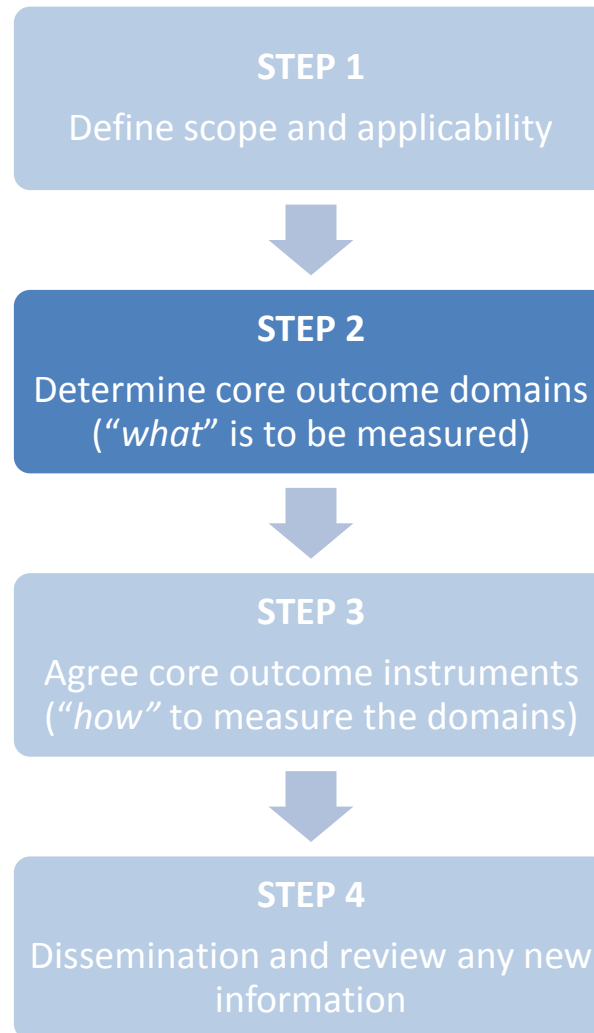






## Agreed at HOME II consensus meeting, Amsterdam 2011

<b>Population</b>	Children and adults with eczema
<b>Setting</b>	Clinical trials (initial focus), clinical practice (when capacity)
<b>Geographical scope</b>	<b>Global</b>
<b>Stakeholders</b>	<i>Clinicians, patients, methodologists, pharma, regulatory</i>



- eDelphi (JID, 2011) presented to the HOME II consensus meeting
  - Preliminary set of core domains
  - Plenary and breakout group discussions
- Consensus rules agreed *a priori*
  - Anonymous, keypad voting
  - Specialised software for real time results
  - Consensus reached if less than 30% of voters disagree
    - *At least 70% of voters agree or are unsure*





Working  
group per  
domain

Clinician-  
reported signs

Patient-  
reported  
symptoms

Quality of Life

Long-term  
control of  
flares

Schmitt J *et al* **Allergy** (2012)





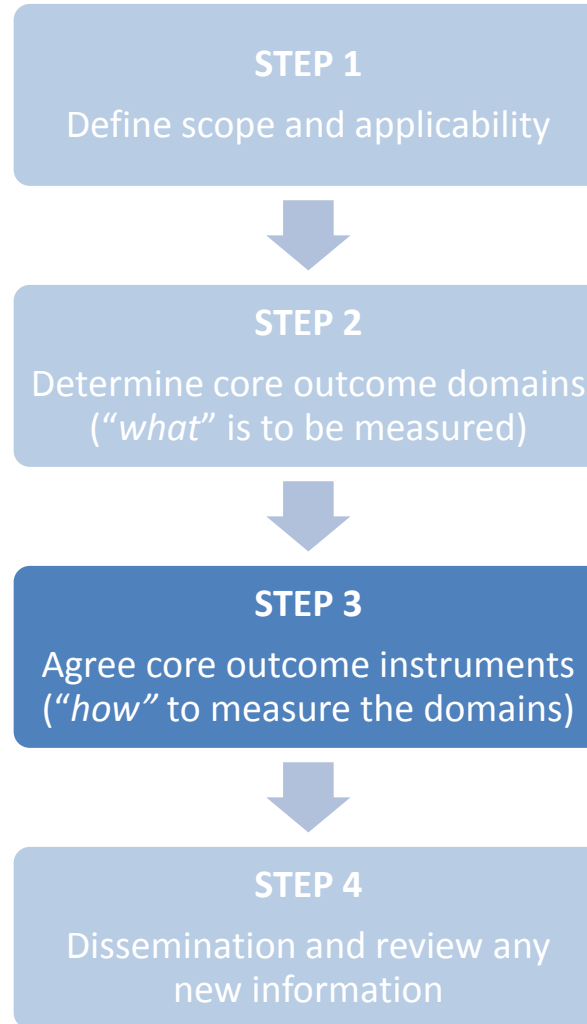
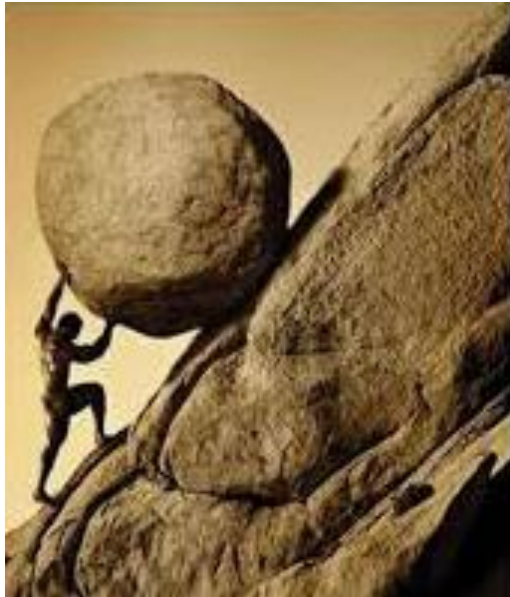
Clinician-  
reported signs

Patient-  
reported  
symptoms

Quality of Life

Long-term  
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flares





## Step 3 comprises 5 stages in the HOME roadmap

### STEP 3

Agree core outcome instruments (“*how*” to measure the domains)

#### STAGE 1

Identify all instruments previously used to measure the domain.

#### STAGE 2

Establish the extent and quality of testing of the identified instruments.

#### STAGE 3

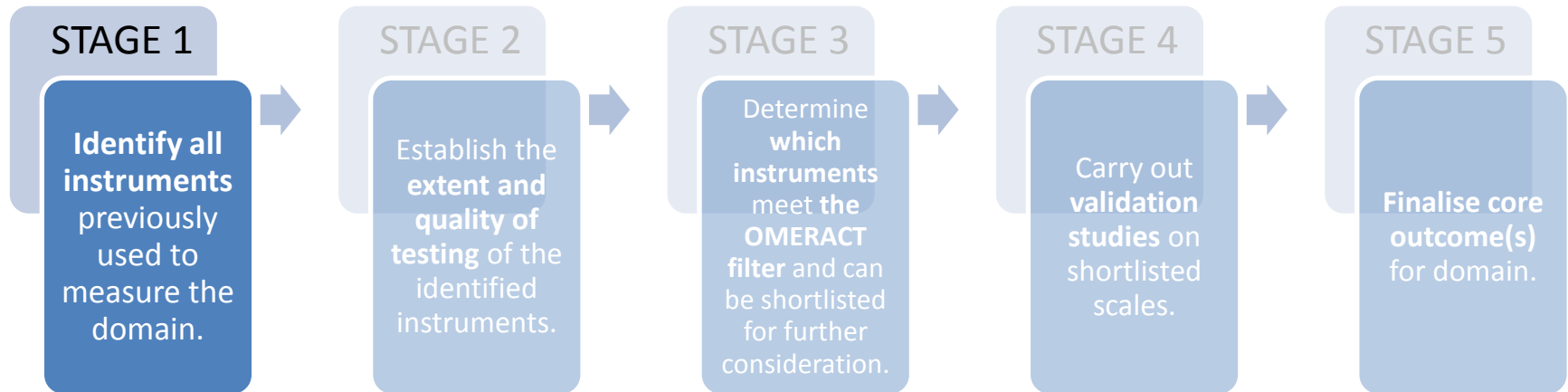
Determine which instruments meet the **OMERACT filter** and can be shortlisted for further consideration.

#### STAGE 4

Carry out **validation studies** on shortlisted scales.

#### STAGE 5

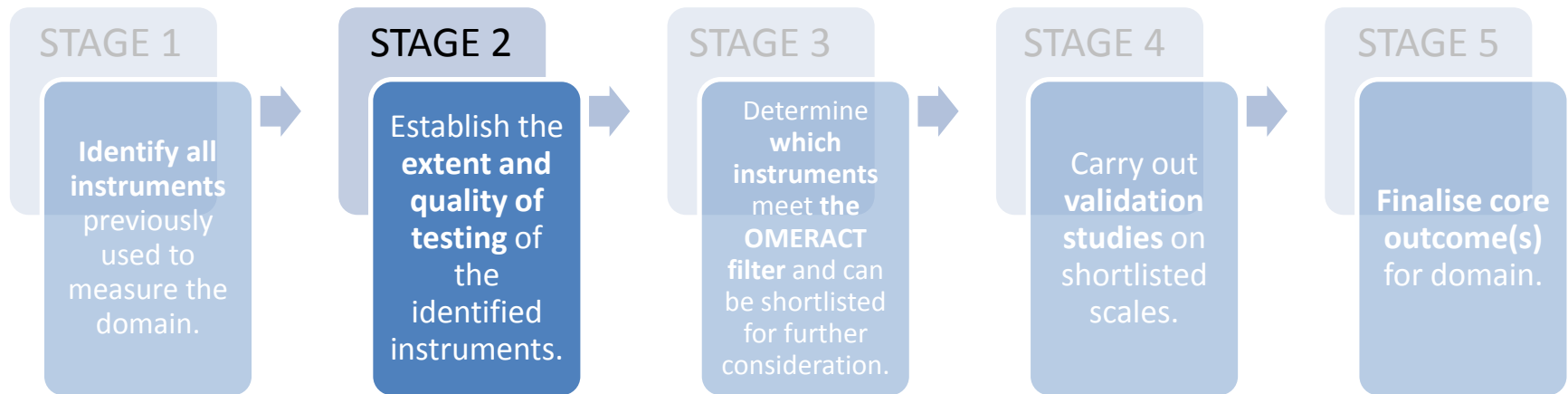
Finalise **core outcome(s)** for domain.



Gerbens *et al* **BJD** 175;678-686 (2016)

## Systematic review

- Long-list of 30 instruments identified (18 un-named)
- 20 composite instruments of clinician-reported signs and patient-reported symptoms



- Extent, quality and results of the testing of measurement properties rated for each instrument (COSMIN methodology)
  - ✓ Has the measurement property been tested?
  - ✓ Did it pass the test?
  - ✓ Were the testing methods of sufficient quality to trust the results?
- COSMIN sensitive search strategy for validation studies
- 29 included studies evaluating 18 instruments



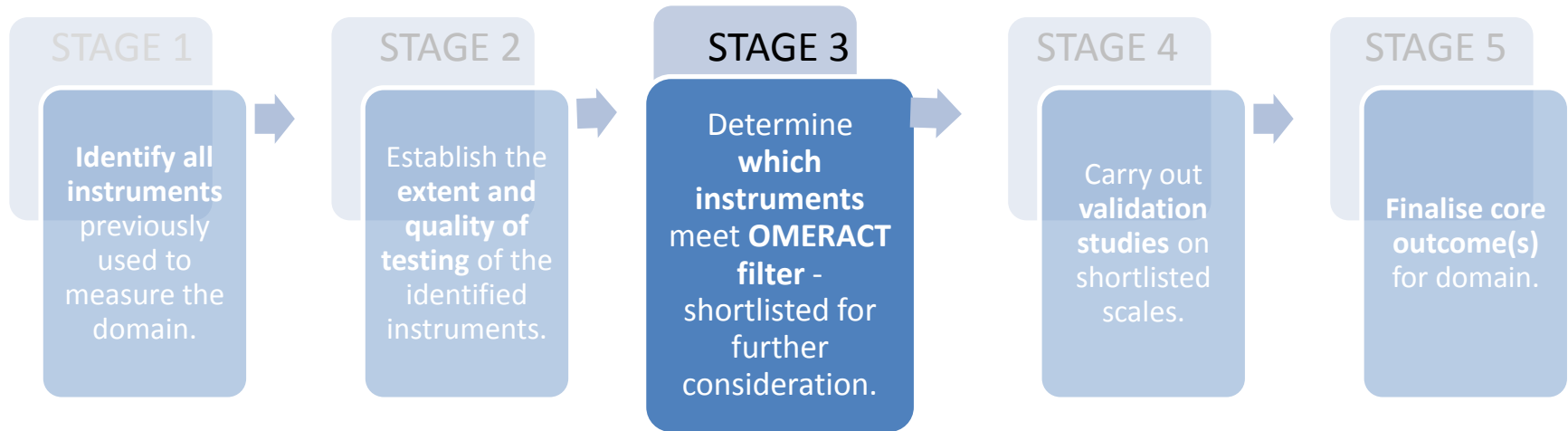
ORIGINAL ARTICLE SKIN AND EYE DISEASES

**Evaluation of the measurement properties of symptom measurement instruments for atopic eczema: a systematic review**

L. A. A. Gerbens<sup>1</sup>, C. A. C. Prinsen<sup>2</sup>, J. R. Chalmers<sup>3</sup>, A. M. Drucker<sup>4,5</sup>, L. B. von Kobylitzki<sup>6</sup>, J. Limpens<sup>7</sup>, H. Nankervis<sup>8</sup>, A. Svensson<sup>9</sup>, C. B. Terwee<sup>2</sup>, J. Zhang<sup>1</sup>, C. J. Apfelbacher<sup>1</sup> & P. I. Spuls<sup>1</sup> on the behalf of the Harmonising Outcome Measures for Eczema (HOME) initiative

<sup>1</sup>Department of Dermatology, Academic Medical Center, University of Amsterdam, Amsterdam, <sup>2</sup>Department of Epidemiology and Biostatistics, University of Nottingham, Nottingham, United Kingdom, <sup>3</sup>Department of Dermatology, University of Queensland, St. Andrew's Hospital, Brisbane, Australia, <sup>4</sup>Department of Dermatology, University of Nottingham, Nottingham, United Kingdom, <sup>5</sup>Department of Dermatology, University of Nottingham, Nottingham, United Kingdom, <sup>6</sup>Department of Dermatology, University of Nottingham, Nottingham, United Kingdom, <sup>7</sup>Department of Dermatology, University of Nottingham, Nottingham, United Kingdom, <sup>8</sup>Department of Dermatology, University of Nottingham, Nottingham, United Kingdom, <sup>9</sup>Department of Dermatology, University of Nottingham, Nottingham, United Kingdom

Gerbens *et al* **Allergy** 2016



Results of SR of measurement properties used to determine which instruments pass the OMERACT filter (Boers *et al* 1998)

<b>Truth</b>	<b>Discrimination</b>	<b>Feasibility</b>
“Is the measure truthful, does it measure what it intends to measure? Is the result unbiased and relevant?”	“Does the measure discriminate between situations that are of interest?”	“Can the measure be applied easily in it’s intended setting, given constraints of time, money, and interpretability?”
<ol style="list-style-type: none"> <li>1. Face validity</li> <li>2. Content validity</li> <li>3. Construct validity (structural validity, hypothesis testing, cross-cultural validity)</li> <li>4. <i>(Criterion validity)</i></li> </ol>	<ol style="list-style-type: none"> <li>1. Reliability (internal consistency, reliability, measurement error)</li> <li>2. Sensitivity to change (responsiveness)</li> </ol>	<ol style="list-style-type: none"> <li>1. Time taken</li> <li>2. Cost</li> <li>3. Interpretability</li> </ol>



**Short-list of potential instruments**



**HOME IV consensus meeting**

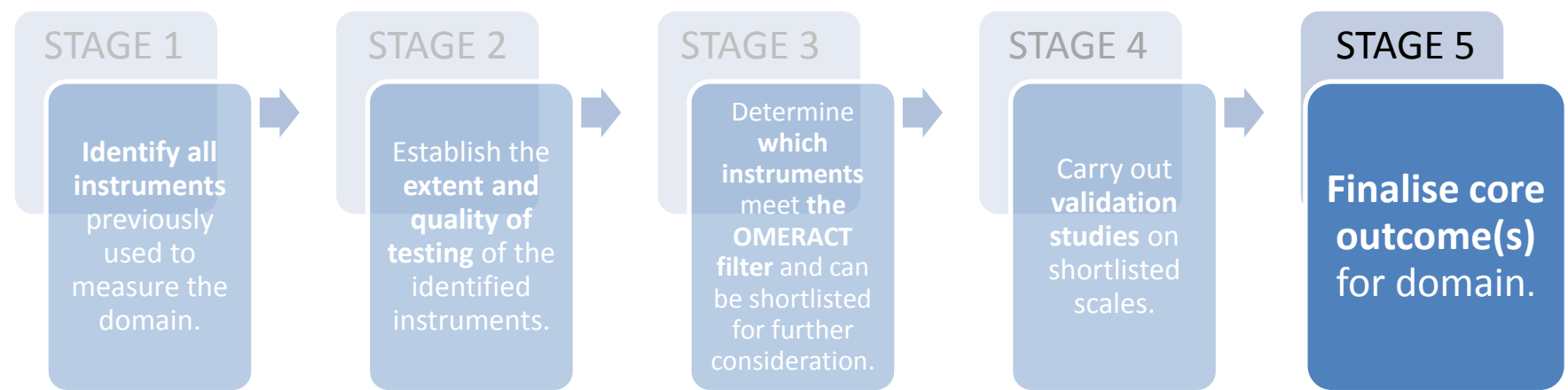
**Malmö, Sweden April 2015**

- Attendees reflected agreed scope and applicability
  - 70 attendees (12 patients, 38 clinicians, 7 methodologists, 13 pharma)
  - North and South America, Europe, Japan and Australia
- Independent moderator (OMERACT)



- Instruments with inadequate quality in *at least one* measurement property excluded from further consideration
  - Shortlist of 5 partially validated instruments (unclear in some areas)
  - Plenary / breakout group discussions of shortlisted instruments
    - ✓ Patient Oriented Eczema Measure (POEM) preferred instrument by all 6 breakout groups
    - ✗ No group preferred the patient versions of clinical rated signs instruments or single symptom instruments
- vote on which (if any) of the potential instruments could be recommended (stage 5)





**“POEM should be recommended for the COS”**

- 87.5% agreed
- 9.4% unsure
- 3.1% disagreed

*Less than 30% disagreed that the POEM should be recommended for the core set*

→ No requirement to develop a new instrument

**POEM**  
Patient-Oriented Eczema Measure

The University of Nottingham  
UNITED KINGDOM · CHINA · MALAYSIA

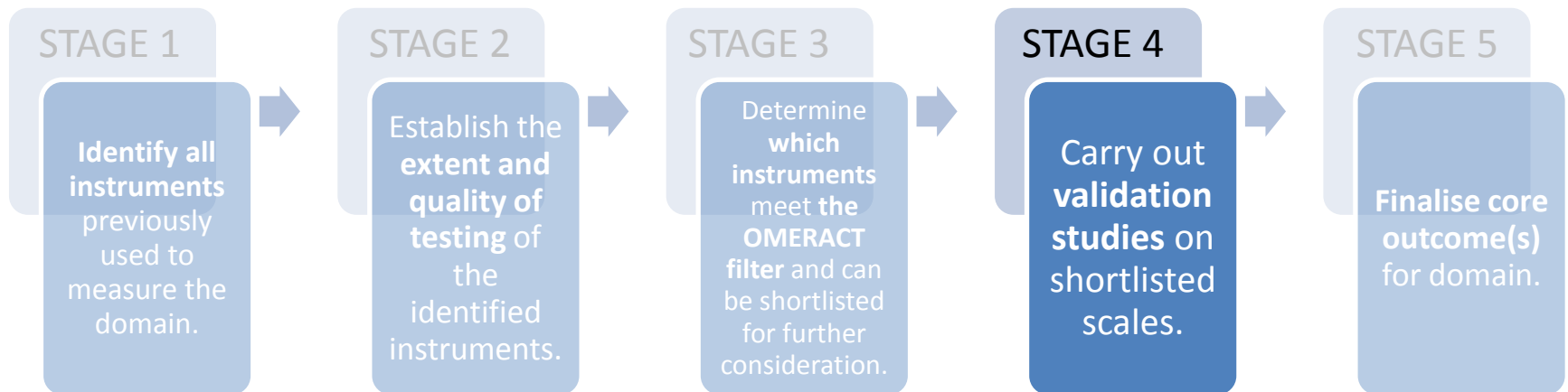
POEM for proxy completion (e.g. by parent)

Patient Details: \_\_\_\_\_  
Date: \_\_\_\_\_

Please circle one response for each of the seven questions below about your child's eczema. If your child is old enough to understand the questions then please fill in the questionnaire together. Please leave blank any questions you feel unable to answer.

- Over the last week, on how many days has your child's skin been itchy because of their eczema?  
No days    1-2 days    3-4 days    5-6 days    Every day
- Over the last week, on how many nights has your child's sleep been disturbed because of their eczema?  
No days    1-2 days    3-4 days    5-6 days    Every day
- Over the last week, on how many days has your child's skin been bleeding because of their eczema?  
No days    1-2 days    3-4 days    5-6 days    Every day
- Over the last week, on how many days has your child's skin been weeping or oozing clear fluid because of their eczema?  
No days    1-2 days    3-4 days    5-6 days    Every day
- Over the last week, on how many days has your child's skin been cracked because of their eczema?  
No days    1-2 days    3-4 days    5-6 days    Every day
- Over the last week, on how many days has your child's skin been flaking off because of their eczema?  
No days    1-2 days    3-4 days    5-6 days    Every day
- Over the last week, on how many days has your child's skin felt dry or rough because of their eczema?  
No days    1-2 days    3-4 days    5-6 days    Every day

Total POEM Score (Maximum 28): .....



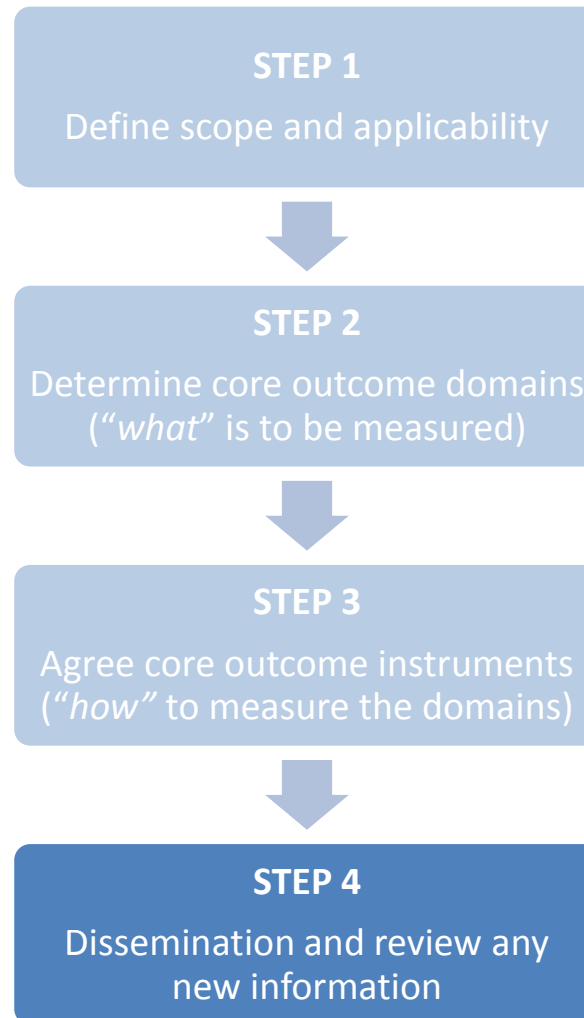
- Some validation gaps in POEM...
- Acceptable quality
- Agreed further work would be carried out...





- Structural validity of POEM – additional items?
- Intensity of symptoms to be added?
- Interpretability
  - Cross-cultural validity
  - MCID in different trial settings and populations





## Dissemination - work in progress...

- Available on website
- Publication in leading journals
- Wider dissemination (industry, funders, regulatory)

## Review of further validation studies

- To be discussed at HOME V (June 2017)

### POEM – Patient Oriented Eczema Measure

The Patient Oriented Eczema Measure (POEM) is a tool used for monitoring atopic eczema severity. It focuses on the illness as experienced by the patient.

Suitable for use in the outpatient clinic, audit, epidemiological studies and clinical trials, POEM has been recommended for use by clinical guidelines including those issued by the National Institute for Health and Care Excellence (NICE).

POEM is recommended by the **Harmonising Outcome Measures for Eczema (HOME)** initiative as the core outcome instrument for measuring patient-reported symptoms in eczema trials.

See the reference section for studies relating to the validation of the scale and the Minimal Clinically Important Difference (MCID).



#### Downloads

Click on the links below to download POEM for free .

POEM for adults 

Also available as a [Word file](#)

**For self-completion:** This version is suitable for patients who are able to read and understand the questions. This will include both adults and older children.

POEM for children 

Also available as a [Word file](#)

**For proxy-completion:** This version is for parents/guardians to fill out on their child's behalf. There are no specific age cut-offs for using this version.

POEM for all ages 

Also available as a [Word file](#)

**For self/proxy-completion:** This version is suitable for both completion by patients and for completion by parents/guardians on their child's behalf.



- HOME roadmap published to guide COS in skin disease
- COSMIN/COMET guidance on selecting COI Prinsen *et al* Trials 2016
  - Consensus study
  - Framework for all areas of medicine
- HOME roadmap and COSMIN/COMET guidance similar
  - Continue with Roadmap for HOME
  - Two helpful differences...



## HOME Roadmap

## COMET/COSMIN guidance

### Completion of validation studies

**Before** provisionally accepting an instrument

**Minimal validation requirements** for provisionally accepting an instrument

- content validity
- internal consistency or test/retest or inter-rater reliability

**REALITY: POEM recommended with some validation gaps**

### Number of instruments per domain

**Ideally one instrument** but more than one permitted

**Only one instrument** recommended

**REALITY: HOME has recommended only one instrument**

- Agreed scope of COS
- Evidence-based consensus process:
  - Domains to be measured
  - Instrument to recommend
  - Further validation studies
- Instrument selection is resource intensive
- Implementation is vital
- HOME roadmap supported by new COSMIN/COMET guidance

For more information on HOME go to  
[www.homeforeczema.org](http://www.homeforeczema.org)

