



**THE JAMES LIND ALLIANCE**  
Tackling treatment uncertainties together

## **Outcomes in clinical research – whose responsibility?** Thursday 20 November 2008 Institute of Education

A meeting organised jointly by the James Lind Alliance  
the Social Science Research Unit, Institute of Education, University of London  
and the Royal College of Nursing Research Institute,  
School of Health and Social Sciences, University of Warwick

### **Report of:** **Discussion themes from the afternoon session**

THE UNIVERSITY OF  
**WARWICK**



Leading education  
and social research  
Institute of Education  
University of London

**Outcomes in clinical research – whose responsibility?  
20<sup>th</sup> November 2008 Institute of Education**

**Discussion themes from afternoon session**

## **1. Acknowledgements**

The James Lind Alliance would like to thank the facilitators who helped to gather this feedback, and facilitated the groups through the discussions:

- ❑ Sophie Staniszweska and Liz Tutton, Royal College of Nursing Research Institute, University of Warwick
- ❑ Ann Jackson and Anne Benson, Royal College of Nursing Institute, London
- ❑ Gillian Stoke, Ruth Stewart and Kathryn Oliver, Social Science Research Unit, Institute of Education, University of London
- ❑ Suzanne Hagen, Glasgow Caledonian University
- ❑ Philippa Yeeles, UK Clinical Research Collaboration
- ❑ Roger Steele, UK Clinical Research Network
- ❑ Maryrose Tarpey, INVOLVE Support Unit
- ❑ Diana Rose, Service User Research Enterprise, Institute of Psychiatry, King's College London

The James Lind Alliance would especially like to thank the poster presenters who produced excellent summaries of their work, then gave poster-related presentations:

- ❑ Michael Power, Sowerby Centre for Health Informatics at Newcastle
- ❑ Iain Sinha, Medicines for Children Research Network Clinical Trial Unit, Institute of Child Health, Alder Hey Children's NHS Foundation Trust, Liverpool
- ❑ Ralf Strobl, Sylvia Lawry Centre for Multiple Sclerosis Research, Munich
- ❑ Mark Fenton, Database of Uncertainties about the Effects of Treatments, James Lind Initiative, Oxford
- ❑ Kirstie Haywood, Royal College of Nursing Research Institute, Warwick University
- ❑ Jo Evans and Caroline Laker, Service User Research Enterprise (SURE), Institute of Psychiatry, London
- ❑ Brian Buckley, Bladder and Bowel Foundation
- ❑ Kim Thomas, Centre of Evidence Based Dermatology, University of Nottingham
- ❑ Alex Wyke, PatientView
- ❑ Lelia Duley, Obstetric Epidemiology, University of Leeds
- ❑ Rebecca Rees, Social Science Research Unit, Institute of Education, University of London
- ❑ Allison Tong, Centre for Kidney Research, Children's Hospital, Westmead, Australia

Posters are available at:

[http://www.lindalliance.org/JLA\\_SSRU\\_RCNI\\_Outcomes\\_Conference\\_Nov\\_2008.asp](http://www.lindalliance.org/JLA_SSRU_RCNI_Outcomes_Conference_Nov_2008.asp)

## **2. Introduction**

The structure of the conference was presentations and discussion in the morning, followed by poster presentations and themed discussion groups in the afternoon. A separate report and commentary on the morning's activities are reported on the James Lind Alliance Website [http://www.lindalliance.org/JLA\\_SSRU\\_RCNI\\_Outcomes\\_Conference\\_Nov\\_2008.asp](http://www.lindalliance.org/JLA_SSRU_RCNI_Outcomes_Conference_Nov_2008.asp)

The groups focussed on content relating to particular areas of outcomes development in the following conditions: Back Pain, Children's Health, Multiple Sclerosis, Schizophrenia, Mental Health, ME/Chronic Fatigue Syndrome, Urinary Incontinence, Skin Disease, Epilepsy, Pregnancy and Childbirth, Sexual Health and Kidney Disease.

All the posters were available to look at throughout the day, and participation in specific discussion groups was based on self-selection. All the groups were facilitated, and notes were taken. All groups used the same format and trigger questions for their discussion. This report highlights the discussion themes from these groups. Quotes from facilitator notes are *italicised*.

### 3. Key feedback messages from each discussion group

At the end of the day, the chairperson asked each group to share their most interesting discussion point/conclusion with the other conference participants. These are set out in the box below.

**MS** – Are patients interested in pictures of their brain? Probably not – but this is a clinical outcome. Patient outcome measures need to match clinical ones.

**Pregnancy and childbirth** - Defined by power struggles between women and professionals. Need outcomes on safety, truth and risk with input from women's experiences of pregnancy and childbirth.

**Mental Health** – The synergy of outcomes is combining the input from several sources for outcomes development.

**Epilepsy** – Translating outcomes of processes to dovetailed tools for trials is the next big area of work to be done. Often outcomes are either too general or too specific to be useful in trials.

**Schizophrenia** - We need to identify patient important outcomes when someone is well, and then they make sense to apply when ill. Symptoms mean different things to different people and this needs to be taken into account in outcome development.

**Skin** – Patient-Reported Outcome Measures (PROMs) that are used in research must have a profile in the publication – preferably the abstract. Trial management groups must take account of the input on outcomes from patients.

**Urinary Incontinence** – Using standardised appropriate outcome measures in both research and clinical use (they can be the same!), and developing a large body of work/database to use for further studies.

**Kidney disease** - When looking at outcomes we need to be asking – what difference will this make to the patient? What is the impact and how do we manage expectations in outcomes?

**Back Injury** – We need to guard against losing the patient input/experience in the process of research. Can we reconcile outcome information for research with outcome information for clinical decision making?

**ME/Chronic Fatigue** – There are poor tools in existence, measuring inappropriate things in the wrong way. Mismatch between what PROMs measure and what people with ME/CFS think are important.

**Sexual Health** – Outcomes selected by stakeholders should be used for systematic reviews. Outcomes need to be defined clearly and sufficiently for literature searches. If we prioritise outcomes are we then prioritising treatments?

**Children's outcomes** - To develop children's outcomes in research we need a collaborative effort of children, parents, trialists, and funding bodies.

#### 4. More detailed feedback

##### 4.1 What sort of tools to measure outcomes have group participants used before?

- ❑ International Classification of Function (ICF)
- ❑ SUDEP (Sudden Unexplained Death in Epilepsy) – *“death is the ultimate BAD outcome after all”*.
- ❑ Threshold measures – however, they present challenges: at what point and how does ‘visibility’ of disease become ‘important’?
- ❑ SF 36 (Short Form 36, a well known outcomes tool)
- ❑ Standard measures for clinical trials including death and morbidity
- ❑ WHO QOL (World Health Organisation Quality of Life)
- ❑ Quality of Life tools
- ❑ Measures of relapse and remission

##### 4.2 What approaches have group participants used to develop outcomes?

- ❑ Mixed methods – ensuring that you get the same answers when you ask what needs to be measured in different ways (called ‘triangulation’ in research)
- ❑ Delphi methods (series of questionnaires, that help identify consensus on outcomes to be measured)
- ❑ Focus groups – mixed (with carers/clinicians) or single focus
- ❑ *“May need to run several methods alongside each other until patient importance in outcomes development is accepted”*
- ❑ Looking at % change in pain over time
- ❑ Reporting over a period of time and returning to study participants, i.e. presenting a cumulative as well as individual ‘snap shot’ in time picture of pain
- ❑ Simple practical outcome measures feels like they are:
  - Agreed by the patient and clinical groups
  - Use SNO-MED-CT (Systematised Nomenclature of Medicine Clinical Terms)
  - Access aggregated data (from more than one source)

##### 4.3 What do group participants think outcome measures are for?

- ❑ To measure outcomes in side effects of treatments, this is a gap in our understanding.
- ❑ Mixed use - sometimes this presents problems for patients where outcomes are developed for use in research/clinical evaluation but are used for other purposes e.g. assessing social welfare claims.
- ❑ In rare conditions – need to get the outcomes right first time as very limited options for funding.
- ❑ The difference between developing outcomes for research purposes and for more routine practice – is this the problem, or is it the solution?
- ❑ OR do they need to be different - as there are different drivers for measuring outcomes in research and clinical practice?

#### 4.4 What do participants measure and how?

- ❑ Exploring the psychological states of outcomes e.g. infection and (in the case of skin disease) fear of infection.
- ❑ The play of generic versus specific outcome measures – is this where patient involvement could really help?
- ❑ Patients frequently report their health status. This can be spontaneous, especially when they are describing a health problem and seeking treatment. However, it may not be spontaneous. It may be as a part of 'history taking' for diagnosis or as a part of patient reported outcome measures, where the importance to this particular patient, or patients generally, may not be clear.
- ❑ Patients can also report their own values. This is widely recognised as an important element of shared decision-making.
- ❑ Patients can also report judgements - having preferences for particular interventions not necessarily dependent on outcomes.
- ❑ Concerns for patient-important but 'invisible' outcomes. These may be very visible to key individuals (e.g. people suffering with incontinence) but not to the wider public, and therefore not receive the attention and sensitivity they deserve in a public arena.
- ❑ Alternatively, they may be invisible to key individuals (e.g. sudden death in epilepsy) and not therefore receive the attention they deserve from individual patients and carers.
- ❑ Outcome measures that can capture what is 'endurable' (e.g. pain, skin conditions).
- ❑ Balance of statistical power (numbers) versus the more in-depth qualitative measure?
- ❑ Some outcome measures should be disease and treatment specific.
- ❑ Some outcomes are very difficult to measure e.g. oedema in pregnancy or when the next epileptic fit is coming.
- ❑ What about pragmatic questions such as 'are you still in hospital?' or 'using services?' or 'are you still self harming?'
- ❑ In MS outcomes are likely to be the ability to keep working, doing hobbies, new hobbies, childbearing (age dependant), predicting when moving between phases of severity, avoiding wheelchair and stick use for mobility, driving, independence in self care, hope and optimism.
- ❑ Hope and fear are really important aspects of ill health, chronic disease and decision making – how much can outcome measures address these issues?

#### 4.5 What did group participants think of the process of developing outcome measures with patients?

- ❑ The value of 'triangulation' of information to inform outcomes, from clinical, patient/carer and researcher perspectives.
- ❑ Getting this mix right at the start of the process, and having the discussions at trial/study feasibility stage.
- ❑ Being open to using a mixed methods approach to ensure that outcomes feedback is sufficiently specific (where needed) and generalised.
- ❑ In chronic conditions – outcome measurement depends on health states at the time of collection – value of knowing relative 'wellness' and 'un-wellness' states.
- ❑ More collaboration – outcomes development could be seen as a specialist science in its own right – does this make it elitist?
- ❑ The more people work together on outcomes development (from a variety of perspectives) the more rounded the result?
- ❑ A shared language is crucial - clinicians think and talk more easily about treatment response and success, whilst patients think and talk more easily about visibility of skin conditions that can or cannot be covered up.
- ❑ Trials and other studies must use the views surfaced through focus groups into trial management, at feasibility stage. Often not enough time to use all of the information to inform the studies.

#### **4.6 How did group participants measure the effectiveness of patient involvement in outcomes development?**

- ❑ Backward audit trail – what outcomes were discussed at the beginning of the study and how are they reflected in the results?
- ❑ More inclusion of patients/carers in publishing research on outcomes development.
- ❑ Routine reporting of patient important outcome measures used in abstracts of published papers – bringing them culturally to prominence in research reporting.
- ❑ When patients get the rare opportunity to review patient reported outcome measures, they often appear to find a mismatch between what patients consider important and what is actually being measured.
- ❑ The challenges of using scales to measure outcomes.
- ❑ The need for tools to be succinct, accessible, easy and quick to use.
- ❑ Really important we get this right, as measures are used in RCTs to evaluate effectiveness, therefore if the wrong measures are being used what is the value of the research overall and to whom?
- ❑ Preferences for treatment can be very strongly held opinions regardless of clinical need.

#### **4.7 What comments did group participants make on measuring the effectiveness of using patient reported outcome measures?**

- ❑ Involve patients more fully in the evaluation of existing measures so that patients can contribute to / participate in the evaluation of patient reported outcome measures, and make recommendations for selection etc.
- ❑ Explore more fully the fundamentals of patient reported outcome measures - that is, just how patient-based are they? How involved have patients been in their development? This is often not reported and where it is, it is often cursory and of poor quality.
- ❑ Need a new generation of patient reported outcome measures that really do place patients at the heart of evaluation and reflect what is genuinely important.