

Outcome measures for patients: experiences of the Centre of Evidence Based Dermatology



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Summary

- The Centre of Evidence Based Dermatology values the opinions of service users in designing and conducting research.
- Involvement of service users in defining and designing outcome measures can be particularly rewarding.
- Clinicians tend to focus on signs and symptoms of disease.
- Patients may be more holistic, and place more emphasis of side effects.

What is the Centre of Evidence Based Dermatology (CEBD)?

- The CEBD is based at the University of Nottingham. The director of the Centre is Professor Hywel Williams and its working principles are quality, clinical relevance and independence.
- The research strategy of the CEBD is based on the concept of three closely related research cogs that drive each other. These cogs:



- **IDENTIFY THE KNOWLEDGE GAPS**
The Cochrane Skin Group produces high quality systematic reviews of interventions for the treatment and prevention of skin disease.
- **FILL THE KNOWLEDGE GAPS**
The UK Dermatology Clinical Trials Network conducts independent, high-quality, multi-centre clinical trials on topics of relevance to patients and clinicians.
- **DISSEMINATE KNOWLEDGE**
The National Library for Health Skin Disorders Specialist Library provides a one-stop shop for quality assured electronic information.

How do service users contribute?

We aim to involve service users at every stage of the research process. This includes:

- Conducting systematic reviews
- Commenting on systematic reviews
- Membership of management committees
- Consumer panels for individual trials
- Focus groups during trial design
- Patient-oriented outcome measures

Skin disease is visible, so its assessment is easy!



How do you measure diffuse disease?

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Do hair counts reflect how the patient feels?

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What does an improvement of 2.5 points on a severity scale mean?

Treatment may be very successful, but at what cost?

Side effects are important too.

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Systematic review of patient-reported outcomes in dermatology trials

METHODS:

- Systematic review of 125 randomised controlled trials (published 1994 – 2001).
- Trials identified from *Archives of Dermatology, Br J Dermatol, Clin & Exp Dermatol, J Dermatol Treat and J Am Acad Dermatol*.

RESULTS:

- Participant efficacy outcomes were mentioned in 32 (26%) of the trials
- Only 53% of the 36 trials reported the results of participant efficacy outcomes in the abstract.
- Only 17 papers clearly declared their primary outcome in advance.
- There was insufficient information to assess agreement between patient and assessor's outcomes.

CONCLUSION:

- More patient-oriented outcome measures are needed.

Townshend AP, Chen CM, Williams HC How prominent are patient-reported outcomes in clinical trials of dermatological treatments? *Br J Dermatol* 2008; Aug [epub ahead of publication]

What can be done?

DEFINING OUTCOME MEASURES

- Talk to patients at an early stage (focus groups, structured interviews, questionnaires).
- Establish what is important to them.
- Example:
Focus group discussions resulted in changes to our study protocol (STOP GAP trial):
 - o The importance of pain as an outcome measure was emphasised.
 - o Patients and clinicians may not agree on the relative importance of different side effects e.g. patients worry about weight gain on oral steroids, just as much as potentially life threatening side effects.

CREATING OUTCOME MEASURES

- Use patient experiences to inform the development of new validated scales.
- Patient-Oriented Eczema Scale (POEM).
- Developed following structured interviews with patients and carers.
- Now used extensively in clinical trials and in normal clinical practice

RAISING THE STATUS OF PATIENT OUTCOMES

- Use patient reported outcomes as the primary outcome when possible.
- This may be difficult for studies when patients are aware of their treatment allocation
- **EXAMPLES:**
 - o Topical steroids for eczema trial – one of the first to use patient reported symptoms as primary outcome (Thomas et al BMJ: 2001).

- o Softened Water Eczema Trial (SWET)



- o Participants know when they have a water softener installed. This makes patient reported outcomes subject to bias – included as secondary outcomes only.

Conclusion

- Trial design and conduct is difficult!
- Talking to patients and their carers helps to ensure that you measure the important things.
- Focus groups can help to establish the most important aspects of the disease and its treatment.
- In general patient-oriented outcomes are poorly reported in trials of dermatological treatments.
- Involving service users in the design and conduct of trials has changed the outcomes that we use.
- The burden of side effects can be underestimated or trivialised by clinicians.