Precision in pemphigus treatment assessment: Establishing minimal clinically important differences (MCIDs) for the pemphigus disease area index (PDAI)

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BACKGROUND

• Autoimmune blistering diseases (AIBDs) are rare conditions characterised by autoantibodies targeting intercellular adhesion molecules.1
• MCIDs represent the smallest change in a particular outcome measure that is considered clinically significant.2
• To date, MCIDs have not been established for the Pemphigus Disease Area Index (PDAI).
• MCIDs provide essential benchmarks for clinical trials, treatment evaluation, and research design optimization.

OBJECTIVE

• To calculate MCIDs for both improvement and deterioration in PDAI scores in patients with pemphigus vulgaris (PV) and pemphigus foliaceous (PF) using the anchor-based method.

METHODS

• A total of 41 pemphigus patients were recruited in Sydney, Australia, with 35 meeting the MCID analysis criteria.
• Generated a robust dataset with 185 pairs of change scores for comprehensive MCID analysis.
• The anchor-based method was employed.
• Anchors: the 15-point Likert scale and the Physician Global Assessment Visual Analogue Scale (PGA-VAS) anchors.
• Receiver operating characteristic (ROC) curves were employed to determine optimal MCID cutpoints with the highest Youden Index (J).

RESULTS

• MCID for Improvement:
  • 15-point Likert scale anchor (Fig. 1a): 2.65 points (78.7% correct classification; sensitivity 75.9%; specificity 73.5%)
  • PGA-VAS anchor (Fig. 2a): 2.5 points (78.0% correct classification; sensitivity 84.4%; specificity 68.2%).
• MCID for Deterioration:
  • Consistently 2.5 points for both anchors (Fig. 1b, 2a) (81.0% correct classification; sensitivity 72.7%; specificity 81.1%).

DISCUSSION

• This study represents the first attempt to publish MCIDs for the PDAI.
• Global implication: MCIDs can be used for assessing pemphigus intervention effectiveness and cost-effectiveness comparisons in clinical trials.
• Limitations and future directions:
  • Future studies should explore MCID estimates across different sites and cultural contexts, ensuring applicability across the spectrum of pemphigus severity.
  • In addition, sample size can be further expanded through international collaboration.

CONCLUSION

This study’s MCIDs are a pivotal step towards tailored interventions, informed clinical trials, and efficient resource utilization in the pursuit of enhanced patient outcomes.

References