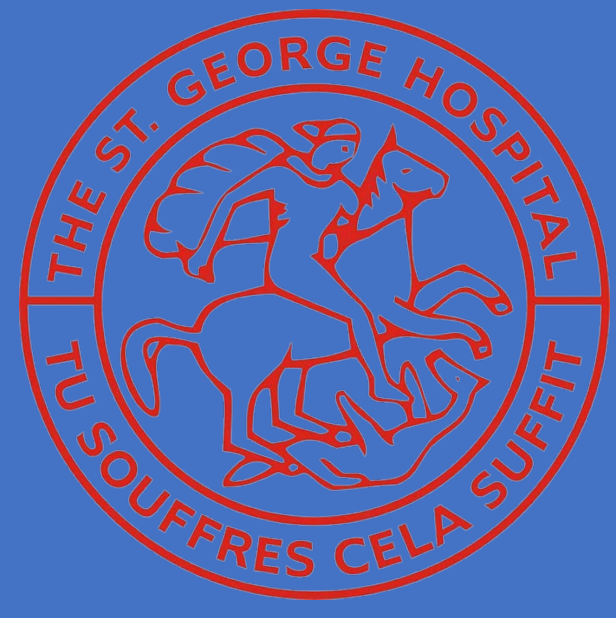# Precision in pemphigus treatment assessment: Establishing minimal clinically important differences (MCIDs) for the pemphigus disease area index (PDAI)

Henry Tseng[1,2], Corey Stone[1,2], Boaz Shulruf[2], Dédée F. Murrell[1,2]

[1]Department of Dermatology, St George Hospital, Sydney, Australia
[2]University of New South Wales, Sydney, Australia

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 81.0%).
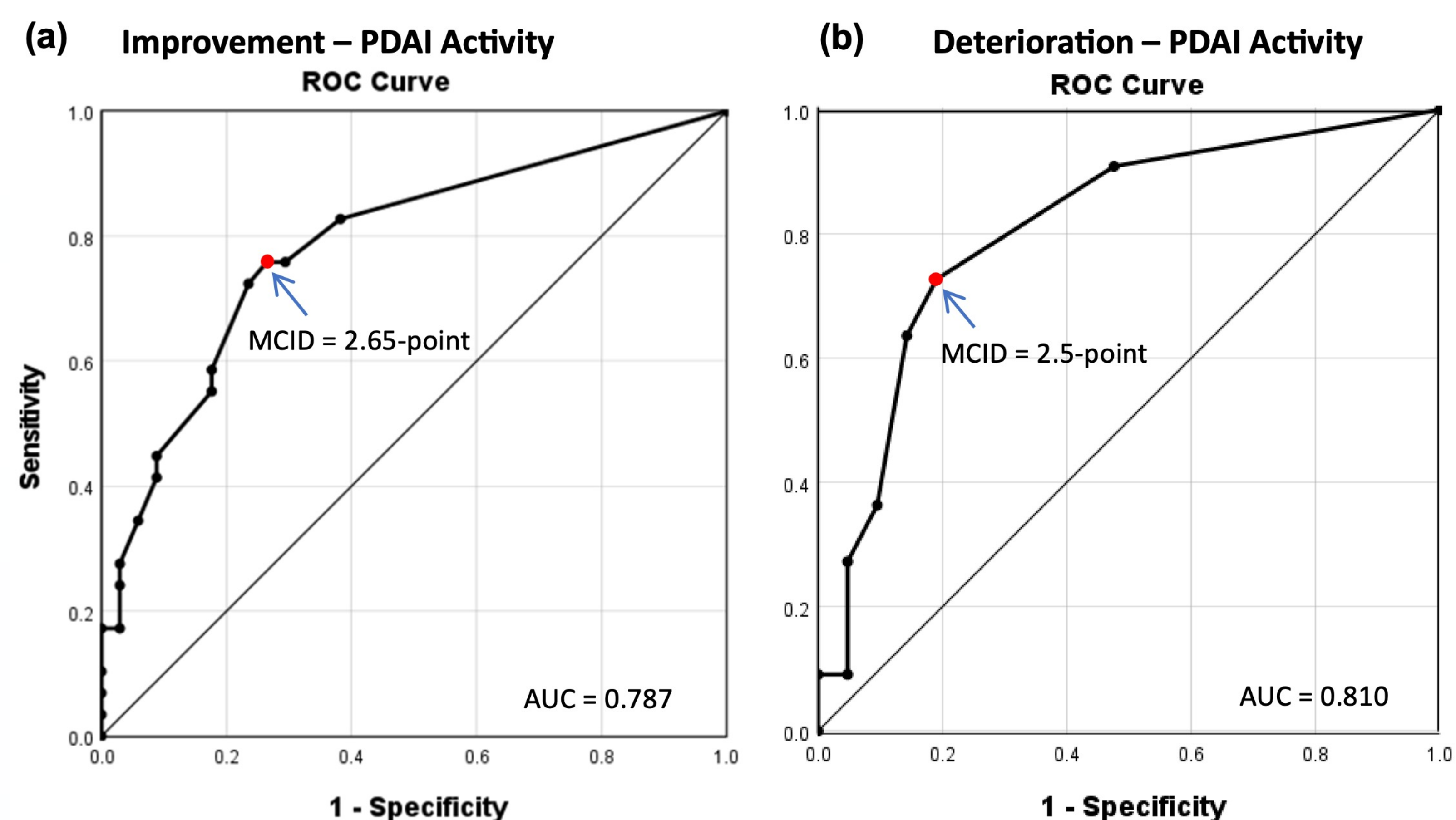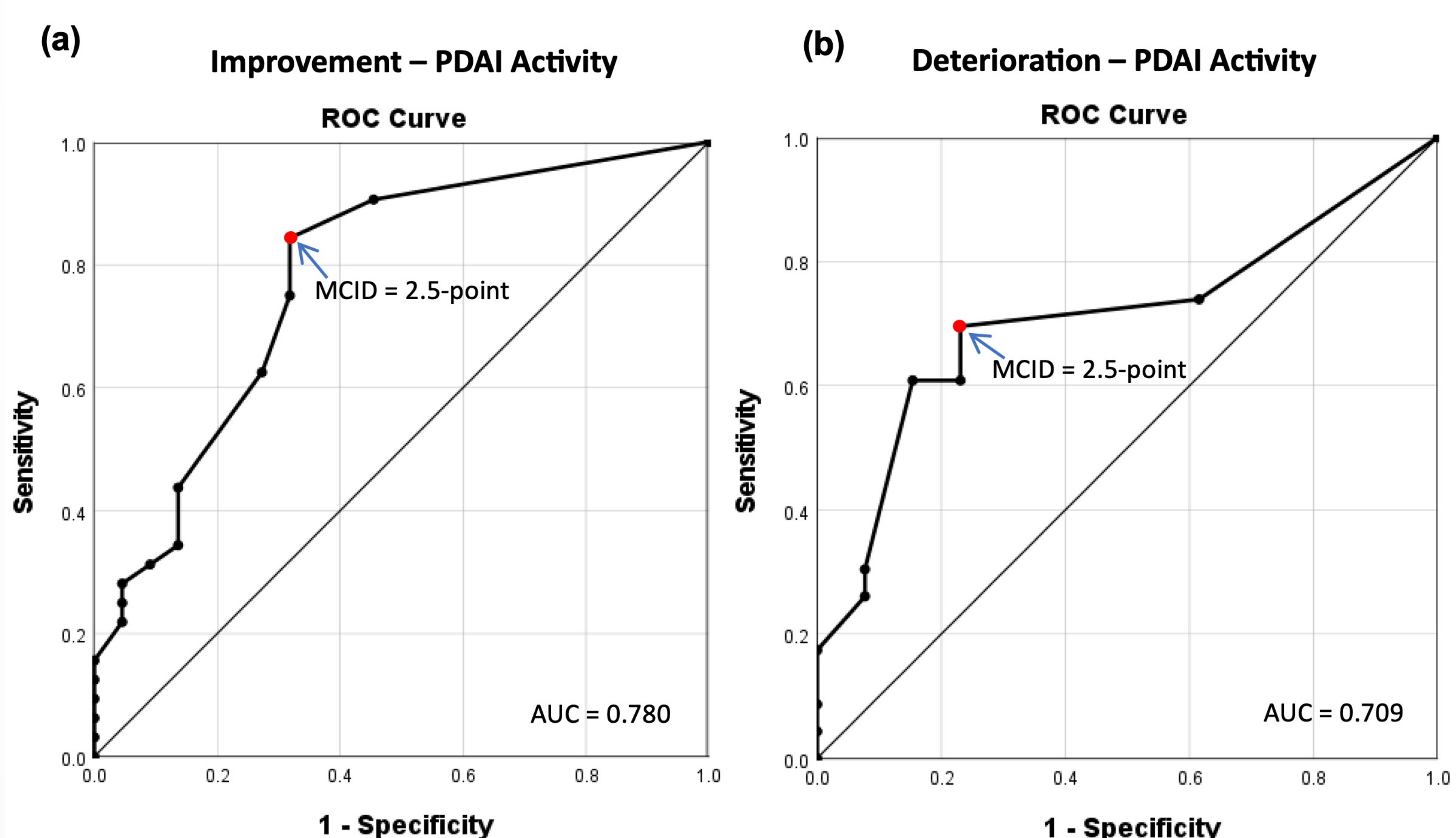
Figure 1

(a) Improvement – PDAI Activity ROC Curve — MCID = 2.65-point; AUC = 0.787
(b) Deterioration – PDAI Activity ROC Curve — MCID = 2.5-point; AUC = 0.810

Figure 2

(a) Improvement – PDAI Activity ROC Curve — MCID = 2.5-point; AUC = 0.780
(b) Deterioration – PDAI Activity ROC Curve — MCID = 2.5-point; AUC = 0.709

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

1. Schmidt E, Kasperkiewicz M, Joly P. Pemphigus. *Lancet* 2019; 394:882-94.
2. Salas Apaza JA, Franco JVA, Meza N et al. Minimal clinically important difference: The basics. *Medwave* 2021; 21:e8149.

d.murrell@unsw.edu.au
henry.tseng@student.unsw.edu.au