P1070

PCDAI Eligibility Criteria Into Clinical Trials: Post Hoc Analysis of the UNITI Jr Study of Ustekinumab in Paediatric Crohn's Disease

Improving Alignment Between Paediatric and Adult Clinical Trial Inclusion Criteria

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Key Takeaways

- **We revisited the suitability of the PCDAI threshold, with an emphasis on concordance or equity with adult studies to assess whether PCDAI eligibility criteria represent the same severity of disease as the CDAI cutoff used in the adult reference population**
- **This post hoc analysis of the UNITI Jr trial provides evidence supporting inclusion of paediatric participants 2 to 17 years old with moderately-to-severely active CD defined by the lowered threshold of PCDAI ≥25 compared with ≥30**
- **A PCDAI threshold of ≥25 with objective evidence of inflammation (per centrally read Simple Endoscopic Score for Crohn's Disease; SES-CD) balances inclusivity and while preserving the ability to detect a relevant change in disease activity**
  - **Recruitment for paediatric CD trials is often difficult.[6] Adopting this reduced threshold could permit inclusion of children with clinically meaningful disease who would have been otherwise excluded from participation in paediatric CD trials, thereby increasing the pool of eligible participants and leading to earlier study completion**
  - **Further analyses in larger sample sizes and more in-depth scrutiny of variables contributing to CDAI and PCDAI are needed**

Background

The multi-item Pediatric Crohn's Disease Activity Index (PCDAI)[1] is used to assess disease activity in paediatric Crohn's Disease (CD),[2] and provides improved differentiation between disease activity levels in this population compared with the Crohn's Disease Activity Index (CDAI)

- Moderately-to-severely active CD has been defined by a baseline PCDAI score of ≥30 in clinical trials, based upon a cutoff established 35 years ago[1,2]

Initial validation prioritized independent association of the PCDAI and CDAI with the purported gold standard of "physician global assessment"[1,3]

- Since PCDAI and CDAI cut-offs were developed separately, there is a risk that they do not identify the same severity of disease for inclusion in clinical trials

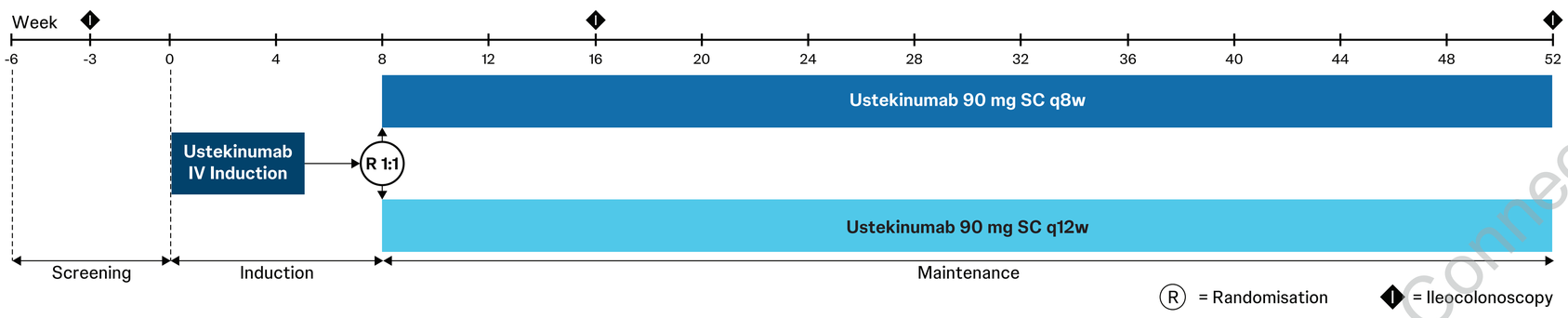
UNITI Jr (NCT04673357) was a phase 3, randomized, controlled trial that evaluated the efficacy and safety of ustekinumab in paediatric participants with moderately-to-severely active CD[4]

Objective

The objective of this post hoc analysis of UNITI Jr was to determine the PCDAI threshold that correlates best with a CDAI threshold of ≥220, which is used to define moderately-to-severely active disease for clinical trial entry in adult CD trials[5]

Methods

UNITI Jr – Study Design



Post hoc analysis:

- Data were from paediatric participants 2 to 17 years old with paired dairy PCDAI and CDAI data at baseline, Week 8, Week 16, or Week 52 (n=79 out of 101 enrolled participants)
- The 11-item PCDAI score was computed by summing all 11-items. The total PCDAI score ranges from 0 to 100 with higher scores indicating greater disease activity
- The 8-item CDAI score was calculated using a predetermined multiplication factor and summed to derive the total score ranging from 0 to over 600, with higher scores indicating greater disease activity
- PCDAI and CDAI History and Symptom item data were collected from study participants prospectively using daily electronic home diaries

Induction dosing:

- <40 kg: ustekinumab 250 mg/m^2
- ≥40 kg to ≤55 kg: ustekinumab 260 mg
- >55 kg to ≤85 kg: ustekinumab 390 mg
- >85 kg: ustekinumab 520 mg

Randomization was stratified by baseline body weight (<40 kg/≥40 kg) and response status (response: PCDAI decrease ≥12.5 points with a total PCDAI score not >30. Nonresponse: PCDAI decrease <12.5. Nonresponders had the option to enroll in an exposure optimization substudy or to discontinue).
***IV**=intravenous, **q8w**=every 8 weeks, **q12w**=every 12 weeks, **SC**=subcutaneous.*

Outcomes / Assessments and Analyses

Post hoc outcomes analysed

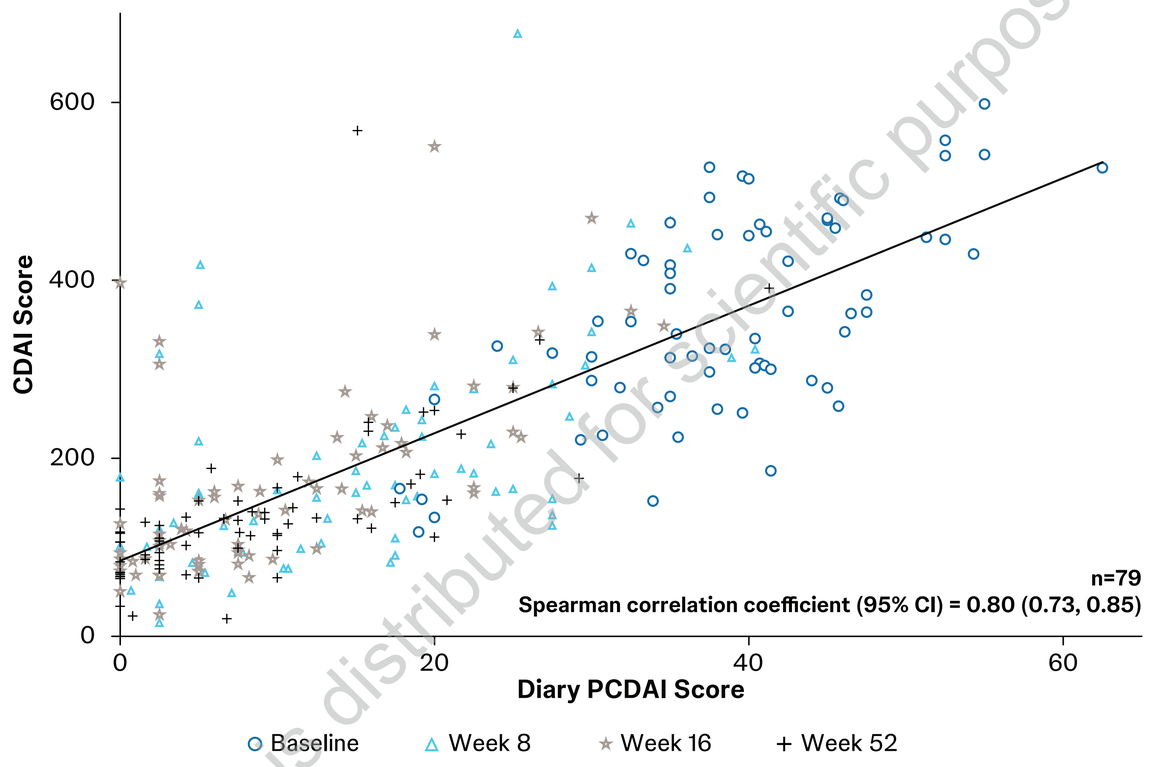
- Pooled baseline and post-baseline daily diary-derived PCDAI and CDAI scores through Week 52

Statistical analyses

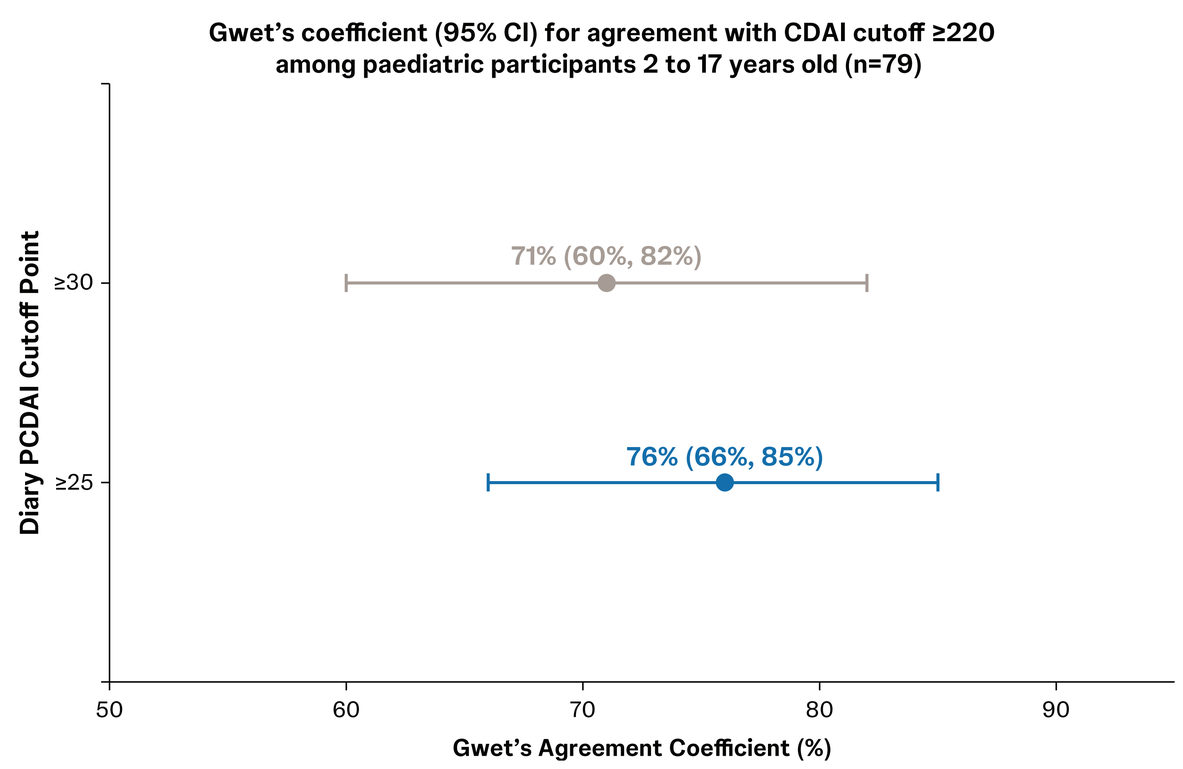
- Spearman's rank correlations between diary-derived PCDAI and CDAI scores and the associated 95% confidence intervals (CIs) were computed
- Gwet's agreement coefficients and 95% CIs were computed based on different dichotomized diary-derived PCDAI scores (<25 vs ≥25 and <30 vs ≥30) versus the dichotomized CDAI score (<220 vs ≥220)
- Given the longitudinal nature of the data, cluster bootstrapping was used to account for within-participant correlations

Results

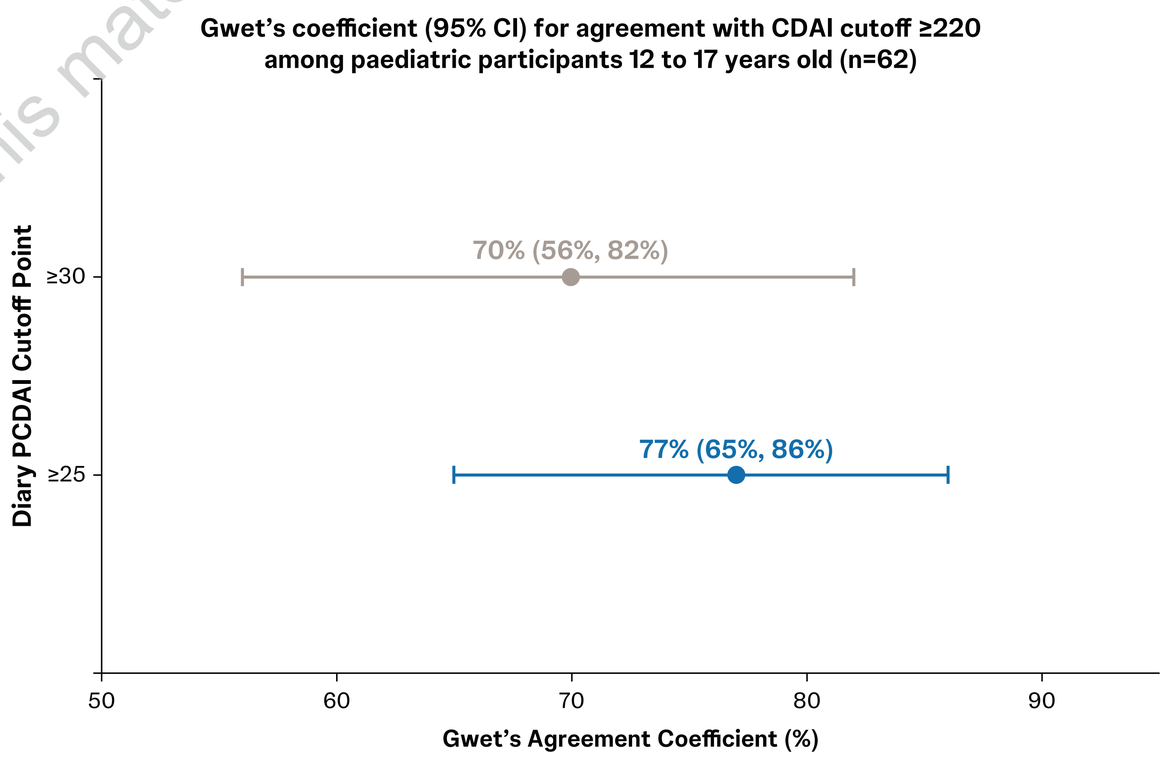
A strong positive correlation was demonstrated between the diary-derived PCDAI and CDAI scores



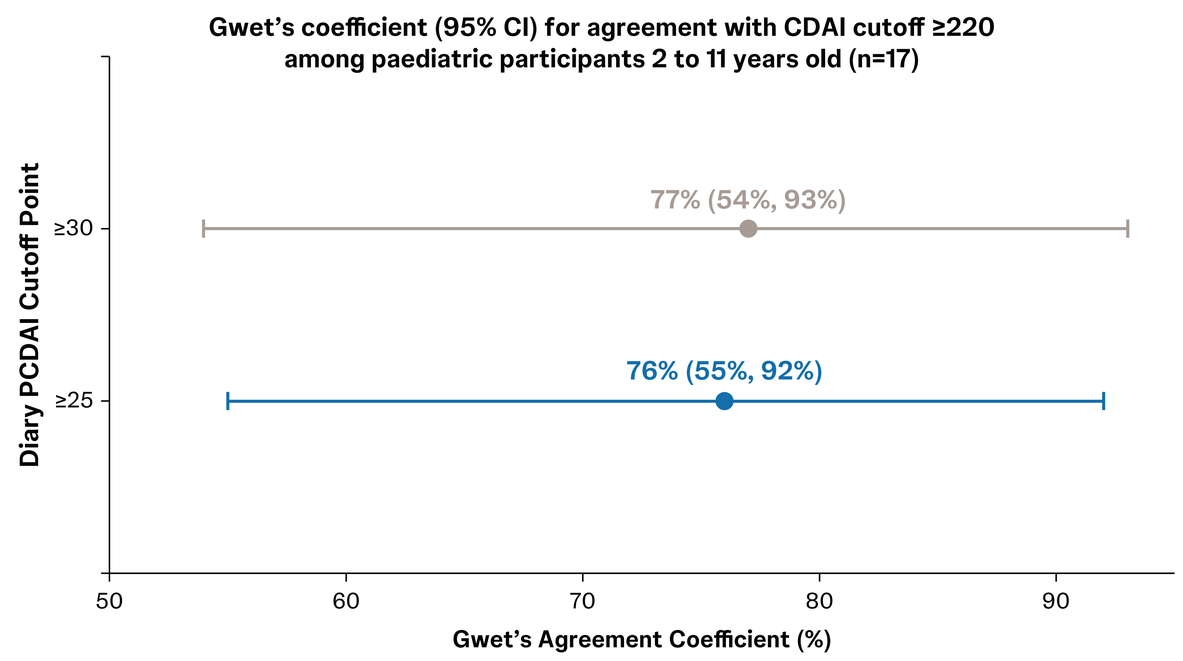
A PCDAI cutoff of ≥25 increased agreement with a CDAI score of ≥220 among all 79 paediatric participants



A PCDAI cutoff of ≥25 increased agreement with a CDAI score of ≥220 among adolescents 12 to 17 years old



Similar agreement was observed between PCDAI scores of ≥25 and ≥30 compared with a CDAI score of ≥220 among children 2 to 11 years old



Similarity of agreement between PCDAI scores of ≥25 and ≥30 versus a CDAI score of ≥220 among children 2 to 11 years old may be a result of limitations associated with CDAI in younger children and the small sample size evaluated

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