Determining the Minimal Important Difference of EQ-5D-5L Utility Values in CIDP Patients Using Data From a Large Clinical Trial

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BACKGROUND | METHODS

- Chronic Inflammatory Demyelinating Polyradiculoneuropathy (CIDP) is a rare autoimmune disorder characterized by distal/proximal weakness and/or sensory deficits.
- This study seeks to estimate the minimal important difference (MID) of EQ-5D-5L utility values in patients with CIDP.
- ADHERE is the largest CIDP trial to date, demonstrating the efficacy of efgartigimod. In stage A (open-label; n=322), all patients received efgartigimod for up to 12 weeks or until evidence of clinical improvement (ECI). Patients with ECI entered stage B (randomized withdrawal), receiving efgartigimod (n=111) or placebo (n=110) for up to 48 weeks or until clinical deterioration. A total of 229 patients continued in the open-label extension trial (OLE), receiving efgartigimod for up to two 48-week cycles.
- Different methods were applied to estimate the MID (Table 2) using data from stage A. Variables are described in Table 1.

TABLE 1 Measures

aINCAT	Disability was assessed using the adjusted Inflammatory Neuropathy Cause and Treatment (aINCAT) score, ranging from 0 (no functional impairment) to 10 (inability to make any purposeful movement).
PGIC	Perceived improvement was assessed using the Patient Global Impression of Change (PGIC) at the final Stage A visit, rated on a 7-point Likert scale. The Patient Global Impression of Severity (PGIS) was not collected in ADHERE, although it is considered a more robust measure as it does not rely on a recall period[1,2].
EQ-5D-5L	The EQ-5D-5L was recorded at baseline and at the last assessment of stage A. Utility values were calculated using the crosswalk method and were anchored at 0 (death) and 1 (perfect health), with negative values corresponding to health states worse than death.

TABLE 2 Methods

Anchor-based methods

Anchor = global transition question
Mean utility change for patients reporting doing "a little better" on the PGIC

Clinical anchor = adjusted INCAT
- Mean utility change in patients with a 1-point decrease in aINCAT
- Regression coefficient of the change in utility versus change in aINCAT
- Optimal cut-off point of the receiver-operating characteristic (ROC) curve (maximizing sensitivity and specificity)

Distribution-based method

Fractions of the standard deviation of utility values at baseline

RESULTS

1. Demographics

- The majority of patients included in stage A of ADHERE (n=322) were male (65%) and most had a diagnosis of typical CIDP (83%) **(Table 3).**

TABLE 3 Baseline patient characteristics

		(n=322)
Sex	Male	208 (65%)
	Female	114 (35%)
Age (years)	Mean (SD)	54.0 (13.9)
CIDP type	Typical CIDP	268 (83%)
	CIDP variant	54 (17%)
Total aINCAT score	Mean (SD)	4.6 (1.67)
CIDP treatment within 6 months prior to study entry	Corticosteroids	63 (20%)
	Immunoglobulins	165 (51%)
	Off treatment	94 (29%)

2. Utility values in Stage A

- Throughout stage A, the average EQ-5D-5L utility value increased from 0.41 to 0.54, corresponding to a mean change (SD) of 0.13 (0.29), indicating an improvement in health-related quality of life **(Table 4).**
- The median (IQR) time in stage A is 3.86 (5.00) weeks.

TABLE 4 Distribution of EQ-5D-5L utility values in stage A

	Baseline	Last assessment	Change from baseline
N obs	315	278	275
Mean (SD)	0.41 (0.27)	0.54 (0.29)	0.13 (0.29)
Median (Q1-Q3)	0.49 (0.23 - 0.60)	0.61 (0.41 - 0.72)	0.1 (0 - 0.24)

3. MID estimates

- The MID estimate based on the PGIC, reflecting the patient perspective, was 0.11. Other methods yielded estimates of 0.14 (mean change for 1-point decrease on aINCAT), 0.10 (regression coefficient), 0.10 (ROC curve), and 0.14 (distribution-based estimate) **(Table 5).**
- The clinician-based estimates aligned closely with the PGIC-based value, suggesting consistency between patient and clinician perspectives.
- The distribution-based MID estimate corresponding to a moderate effect size was 0.14, supporting the range identified through the anchor-based methods.

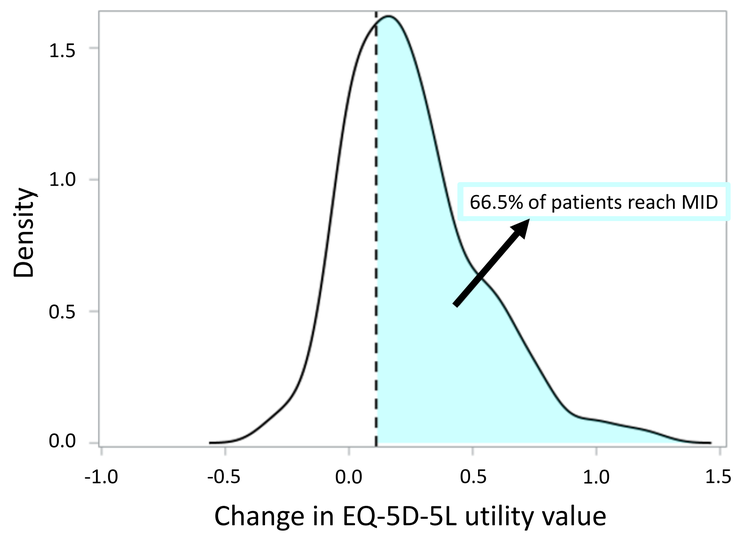
TABLE 5 MID estimates for the different methods

Method	MID estimate
Global transition question (PGIC)	
Mean change for patients doing "a little better"	0.11
Clinical anchor (adjusted INCAT score)	
Mean change for patients showing slight improvement	0.14
Slope of the regression	0.10
Optimal change value on the ROC curve	0.10
Distribution-based methods	
0.2·SD	0.05
0.5·SD	0.14

3. Achievement of MID in ADHERE

- Within stage A of ADHERE, 48% of patients achieved the MID of 0.11 between baseline and the last assessment. Stage A was dynamic: patients transitioned to Stage B as soon as clinical improvement was observed. Consequently, maximal improvement may not have been captured within the timeframe of Stage A.
- In the open-label extension study, 66.5% of patients achieved the MID between baseline stage A and week 12 (n=188), indicating that the majority of patients experienced a clinically meaningful improvement in quality of life with efgartigimod treatment. The OLE trial population was limited to patients responding to treatment during Stage A.

FIGURE 1 Density plot of the change in EQ-5D-5L utility value between baseline Stage A and week 12 of the OLE trial, with the dotted line indicating the MID estimate of 0.11



KEY TAKEAWAYS

- The MID estimate of 0.11 based on the PGIC is consistent with previously reported values for other health condition[3,4,5].
- The consistency across methods supports an MID of approximately 0.10–0.14 for CIDP patients. This range may facilitate interpretation of utility changes in future clinical and economic evaluations.
- The majority of patients in the open-label extension achieved the MID, indicating a sustained improvement in health-related quality of life with efgartigimod.
- A limitation is the absence of PGIS data for MID estimation, although PGIS is considered more robust than PGIC as it is not affected by recall bias[1,2].

PRESENTED AT THE PROFESSIONAL SOCIETY FOR HEALTH ECONOMICS AND OUTCOMES RESEARCH (ISPOR); NOV 9 – NOV 12, 2025; GLASGOW, SCOTLAND, UK

ABBREVIATIONS: CIDP: Chronic Inflammatory Demyelinating Polyneuropathy, **MID**: Minimal Important Difference, , **aINCAT**: adjusted Inflammatory Neuropathy Cause and Treatment, **EQ-5D-5L**: EuroQoL 5-Dimension 5-Level, **PGIC**: Patient Global Impression of Change, **ROC**: receiver-operating characteristic, **N**: Sample size, **SD**: standard deviation, **IQR**: Interquartile Range, **Q1**: first quartile (25% percentile), **Q3**: third quartile (75% percentile)

ACKNOWLEDGMENTS: This study used data from the ADHERE, an independently conducted survey. Argenx were one of multiple subscribers to the DSP, and funded the analysis described here. The material in this poster has not been previously presented or published. **FB, LV** and **SD** are paid consultants for and receive grant support from argenx. **CA**, **SP** and **GP** are employees of and hold stock in argenx.

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SCAN ME