Diagnostic Adjudication of Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) in the ADHERE Trial: First 200 Cases

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INTRODUCTION

- Diagnosing CIDP is challenging because it has various clinical presentations; the misdiagnosis rate is reported to be as high as ~50%[1]
- In clinical trials, the European Federation of Neurological Societies/Peripheral Nerve Society (EFNS/PNS) 2010 criteria[2] are widely used
- In **ADHERE**[3] (NCT04281472), a 9-member independent CIDP Confirmation Committee (CCC) was established to confirm an accurate diagnosis for enrollment,[4] a novel approach in global CIDP randomized controlled trials
  - ADHERE is an ongoing, phase 2, multicenter, randomized, double-blind, placebo-controlled trial investigating **efgartigimod PH20 SC** for CIDP and enrolls probable or definite CIDP cases but excludes pure sensory CIDP[4]
  - **Efgartigimod PH20 SC** is subcutaneous efgartigimod co-formulated with recombinant human hyaluronidase PH20, which increases dispersion and absorption of efgartigimod[4]

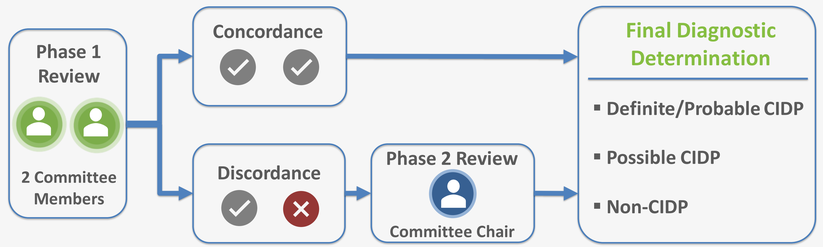
OBJECTIVE

- To report on the first 200 cases adjudicated by the CCC, an update on the first 100 cases in the AANEM 2021 poster[5]

SUMMARY AND PERSPECTIVE

- Use of a CCC may increase diagnostic accuracy in the recruitment of participants with CIDP for clinical trials
- Definite/Probable CIDP was confirmed in 58.5% (117/200) of all cases, of which 78% (92/117) were typical CIDP and 17.9% (21/117) were atypical CIDP
- Even among experts, it is challenging to accurately identify and diagnose CIDP per the 2010 EFNS/PNS criteria
  - There was concordance at phase 1 review in 52% of the 200 cases, consistent with previously reported data for the first 100 cases[5]
- The CCC was able to confirm Definite/Probable CIDP in 69% (20/29) of participants with concomitant diabetes

CCC ADJUDICATION PROCESS



RESULTS

Table 1. Baseline Demographics

CCC Adjudicated (N=200)	
Age (years), mean (SD)	56.4 (13.54)
Sex, n (%)	
Female	68 (34.0)
Male	132 (66.0)
Race, n (%)	
Asian	10 (5.0)
Black/African American	3 (1.5)
Native Hawaiian/Pacific Islander	1 (0.5)
Other/Not Reported	4 (2.0)
White	182 (91.0)
Ethnicity, n (%)	
Hispanic/Latino	13 (6.5)
Not Hispanic/Latino	185 (92.5)
Not Reported	2 (1.0)
Diabetes, n (%)	29 (14.5)

Table 2. Diagnosis, by Data Presented to CCC for Adjudication

	EFNS/PNS Clinical Criteria				EFNS/PNS Electrodiagnostic Criteria			
	Yes	No	Disagree	Not Avail	Yes	No	Disagree	Not Avail
CONCORDANCE (n=104), n (%)								
Definite/Probable (n=79)	79 (76.0)	0	0		79 (76.0)	0	0	
Possible CIDP (n=0)	0	0	0		0	0	0	
Non-CIDP (n=25)	3 (2.9)	13 (12.5)	9 (8.7)		4 (3.8)	15 (14.4)	6 (5.8)	
DISCORDANCE (n=96), n (%)								
Definite/Probable (n=38)	25 (26.0)	1 (1.0)	9 (9.4)	3 (3.1)	23 (24.0)	0	13 (13.5)	2 (2.1)
Possible CIDP (n=10)	4 (4.2)	0	5 (5.2)	1 (1.0)	5 (5.2)	0	4 (4.2)	1 (1.0)
Non-CIDP (n=48)	11 (11.5)	1 (1.0)	32 (33.3)	4 (4.2)	10 (10.4)	9 (9.3)	26 (27.1)	3 (3.1)

Yes and No indicate whether the EFNS/PNS criteria were met or not met. Disagree means the adjudicators did not agree on whether the criteria were met (Yes) or not met (No). Not Avail(able) indicates cases with missing data in the database. Percentages are based on CIDP diagnosis category.

Figure 1. Overall Adjudication (N=200)

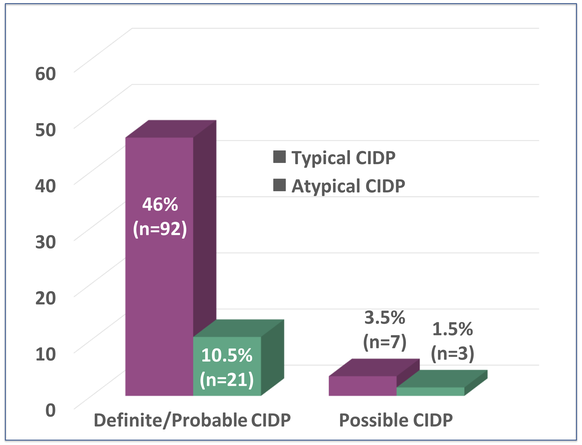


Figure 2. Adjudication in Diabetes (n=29)

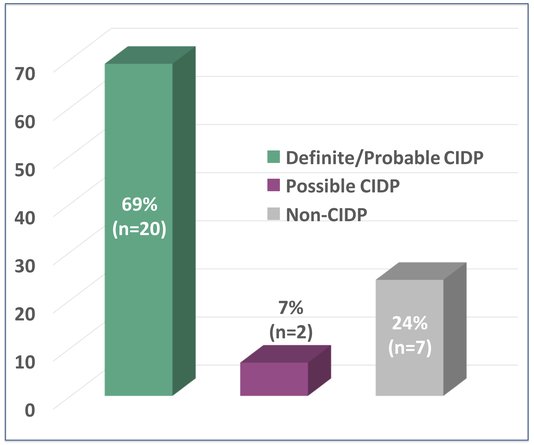
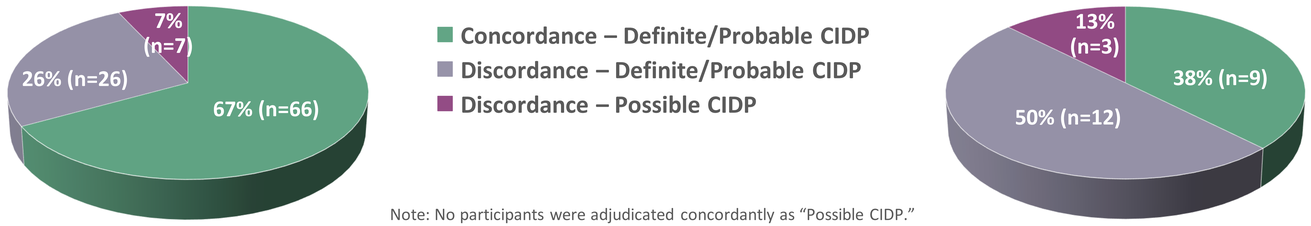


Figure 3. Adjudication, by CIDP Subtype



Note: No participants were adjudicated concordantly as "Possible CIDP."

REFERENCES: **1**. Allen JA, Lewis RA. *Neurology*. 2015;85:498-504. **2**. Van den Bergh PYK, et al. *Eur J Neurol*. 2010;17:356-363. **3**. ClinicalTrials.gov identifier: NCT04281472. Accessed August 24, 2022. https://clinicaltrials.gov/ct2/show/NCT04281472. **4**. argenx data on file, 2022. **5**. Donofrio PD, et al. Poster presented at: AANEM Annual Meeting; October 13-16, 2021; Aurora, Colorado. P169.

DISCLOSURES: This study was sponsored by argenx, the manufacturer of efgartigimod. Efgartigimod PH20 SC is an investigational agent that is not currently approved for use by any regulatory agency. **SA:** Has no COIs to disclose; **PDD:** Has received consulting honoraria from argenx; **KG:** Has received consulting honoraria from Annexon, argenx, Genentech, Momenta, Pfizer, UCB Pharma; **CK:** Has received consulting honoraria from Akcea, Alnylam, argenx, Biogen, CSL Behring, Sanofi; **JK:** Has received consultancy fees, speaking fees, and/or honoraria from argenx, Astellas Pharma, Boehringer Ingelheim, CSL Behring, Eisai, Mitsubishi Tanabe Pharma, Novartis Pharma, Otsuka Pharmaceutical, Pfizer Japan, Takeda Pharmaceutical Company, Teijin Pharma; **AK-P:** Has received consulting/lecture honoraria from argenx, Baxter, CSL Behring, Kedrion, Takeda; **J-ML:** Has received consulting honoraria from argenx, CSL Behring, Grifols, LFB, Pharnext, Sanofi; **EN-O:** Has received consulting honoraria from argenx, CSL Behring, Kedrion, LFB, Roche, Sanofi, Shire/Takeda; **EH, AT:** Are employees of argenx; **MM, ML:** Are employees of PPD, which was paid by argenx to conduct the study; **RAL:** Has received consulting honoraria from Akcea, Alnylam, argenx, Biotest, CSL Behring, Grifols, Momenta, Pfizer, Pharnext, Sanofi, Takeda.
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