Long-Term Efficacy of Efgartigimod PH20 SC in Patients With Chronic Inflammatory Demyelinating Polyneuropathy: Interim Results From the ADHERE+ Study

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CIDP Is a Severe and Progressing Immune-Mediated Polyneuropathy

- CIDP is an **autoimmune** peripheral neuropathy characterized by **progressive or relapsing muscle weakness and sensory disturbance** and associated with a **high treatment burden**[1–5]
- Although the exact pathophysiology of CIDP is yet to be fully understood, **IgG autoantibodies** play a key role in **demyelination**[6–9]
- **Efgartigimod PH20 SC** is a coformulation of efgartigimod and recombinant human hyaluronidase PH20 (rHuPH20), which allows for **rapid (30–90s single injection)** SC administration[10,11]

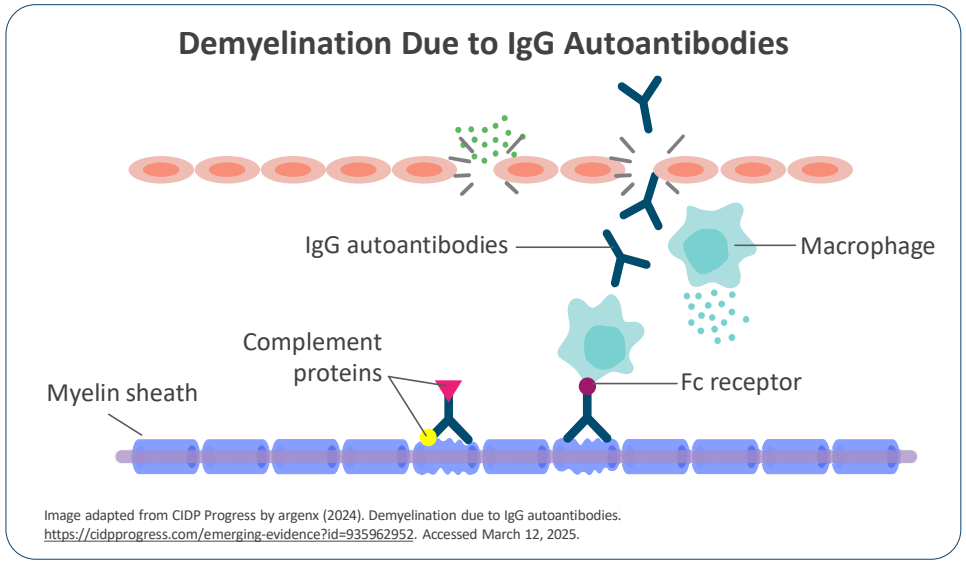
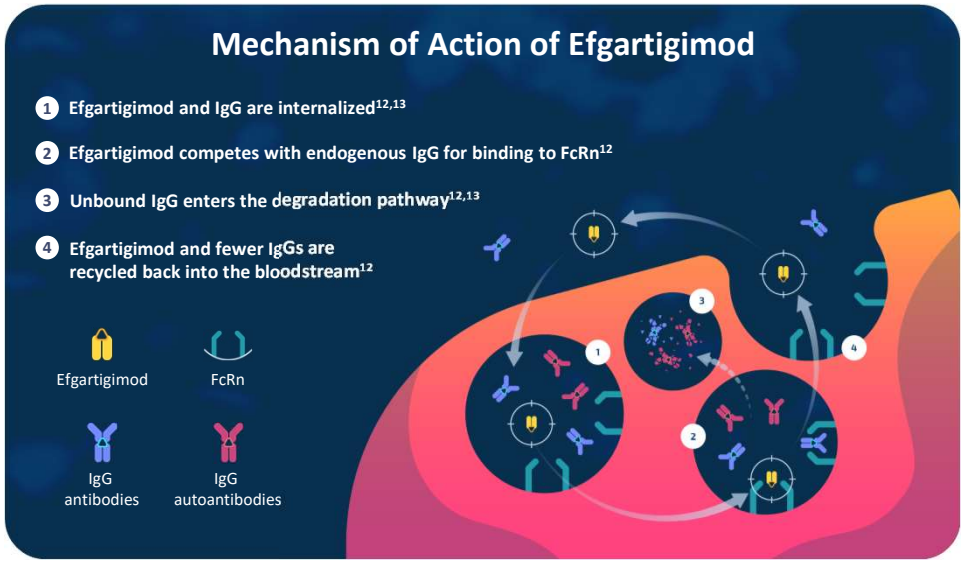


Image adapted from CIDP Progress by argenx (2024). Demyelination due to IgG autoantibodies. https://cidpprogress.com/emerging-evidence?id=935962952. Accessed March 12, 2025.



Efgartigimod has been shown to **reduce IgG antibody levels** in healthy volunteers and patients with other autoimmune diseases[13–18]

CIDP, chronic inflammatory demyelinating polyneuropathy; Fc, fragment crystallizable; FcRn, neonatal Fc receptor; IgG, immunoglobulin G; SC, subcutaneous.

1. Cox ZC, et al. *Clin Geriatr Med.* 2021;37(2):327–45. 2. Van den Bergh PYK, et al. *Eur J Neurol.* 2021;28(11):3556–83. 3. Brun S, et al. *Immuno.* 2022;2(1):118–31. 4. Bus SRM, et al. *J Neurol.* 2022;269(2):945–55. 5. Gorson KC. *Ther Adv Neurol Disord.* 2012;5(6):359–73.
6. Querol LA, et al. *Neurotherapeutics.* 2022;19(3):864–73. 7. Yan WX, et al. *Ann Neurol.* 2000;47(6):765–75. 8. Dziadkowiak E, et al. *Int J Mol Sci.* 2021;23(1):179. 9. Koike H, et al. *Neurol Ther.* 2020;9(2):213–27. 10. Locke KW, et al. *Drug Deliv.* 2019;26(1):98–106. 11. VYVGART HYTRULO. Prescribing information. argenx; 2024. https://www.argenx.com/product/vyvgart-hytrulo-prescribing-information.pdf. Accessed March 12, 2025. 12. Ulrichts P, et al. *J Clin Invest.* 2018;128(10):4372–86. 13. Sesarman A, et al. *Cell Mol Life Sci.* 2010;67(15):2533–50.
14. Vaccaro C, et al. *Nat Biotech.* 2005;23(10):1283–88. 15. Howard JF Jr, et al. *Lancet Neurol.* 2021;20(7):526–36. 16. Goebeler M, et al. *Br J Dermatol.* 2022;186(3):429–39. 17. Broome CM, et al. *Lancet.* 2023;402(10413):1648–59. 18. Howard JF Jr, et al. *Front Neurol.* 2024;14:1284444.

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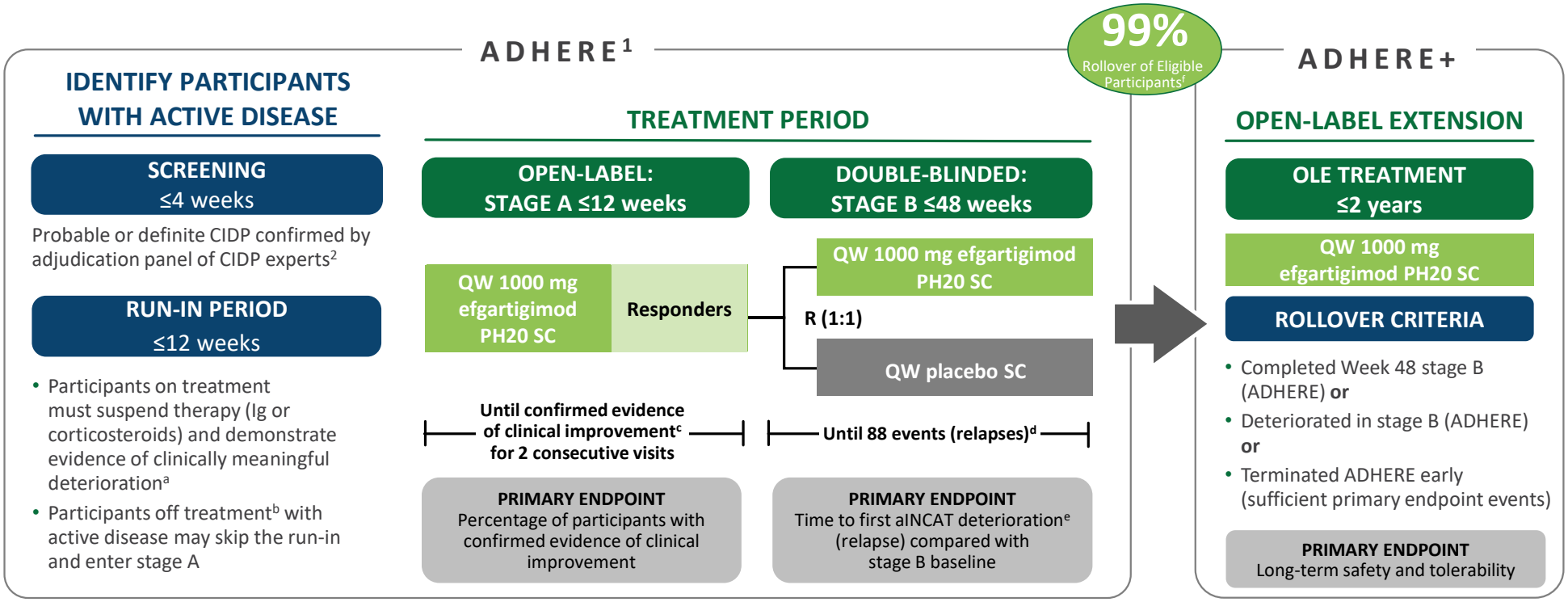
Efgartigimod in CIDP: Study Designs of ADHERE and ADHERE+



aINCAT, adjusted Inflammatory Neuropathy Cause and Treatment; CIDP, chronic inflammatory demyelinating polyneuropathy; ECI, evidence of clinical improvement; ECMD, evidence of clinically meaningful deterioration; HR, hazard ratio; Ig, immunoglobulin; INCAT, Inflammatory Neuropathy Cause and Treatment; I-RODS, Inflammatory Rasch-Built Overall Disability Scale; IVIg, intravenous immunoglobulin; OLE, open-label extension; PH20, recombinant human hyaluronidase PH20; QW, once weekly; R, randomization; SC, subcutaneous; SCIg, subcutaneous immunoglobulin.

[a]ECMD was defined as an aINCAT increase of ≥1 points, an I-RODS decrease of ≥4 points (centile metric), or a grip strength decrease of ≥8 kPa. [b]Off treatment was defined as participants who had never received CIDP treatment (treatment naïve) or who had not received CIDP treatment (corticosteroids, IVIg, or SCIg) within 6 months of trial entry. [c]ECI was defined as a clinical improvement on the parameters that the participant worsened in during run-in (≥4-point increase in I-RODS and/or ≥8-kPa increase in mean grip strength) or clinical improvement (≥1-point decrease) in INCAT. ECI was confirmed after these criteria were met after 4 injections and 2 consecutive visits. [d]The primary endpoint was assessed once 88 total relapses or events were achieved in stage B and was based on the HR for the time to first aINCAT deterioration (ie, relapse). [e]aINCAT deterioration was defined as a ≥1-point increase in aINCAT score compared with stage B baseline, which was confirmed at a consecutive visit after the first 1-point increase in aINCAT or not confirmed for participants with ≥2-point increase in aINCAT compared with stage B baseline. [f]n=228/229. 229 participants enrolled in ADHERE+, including 3 participants who inadvertently rolled over without meeting per-protocol inclusion criteria. The safety population for ADHERE+ included 228 participants who received ≥1 dose of efgartigimod PH20 SC in the OLE, as 1 participant discontinued before receiving the first dose of efgartigimod PH20 SC.

1. Allen JA, et al. *Lancet Neurol.* 2024;23(10):1013–24. 2. Van den Bergh PYK, et al. *Eur J Neurol.* 2010;17(3):356–63.

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Baseline Characteristics Were Similar Between ADHERE Stages A/B and ADHERE+, and Well-Balanced Between Treatment Groups in ADHERE Stage B

	ADHERE[1]			ADHERE+[e] Open-Label Extension
	Open-Label Stage A	Double-Blinded Stage B		
	Efgartigimod PH20 SC (N=322)	Efgartigimod PH20 SC (N=111)	Placebo SC (N=110)	Efgartigimod PH20 SC (N=228)
Scores shown were assessed at screening in ADHERE and baseline in ADHERE+				
Age, year, mean (SD)	54.0 (13.9)	54.5 (13.2)	51.3 (14.5)	53.2 (14.1)
Sex, male, n (%)	208 (64.6)	73 (65.8)	69 (62.7)	142 (62.3)
Time since diagnosis, years, mean (SD)	4.9 (6.1)	3.7 (4.4)	3.8 (4.7)	4.9 (5.6)
Typical CIDP diagnosis, n (%)	268 (83.2)	97 (87.4)	95 (86.4)	199 (87.3)
Unstable active disease (CDAS: 5),[a] n (%)	197 (61.2)	74 (66.7)	76 (69.1)	151 (66.2)
Prior treatment (within past 6 months), n (%)				
Corticosteroids	63 (19.6)	24 (21.6)	23 (20.9)	51 (22.4)
Immunoglobulins (IVIg, SCIg)	165 (51.2)	48 (43.2)	48 (43.6)	104 (45.6)
Off treatment[b]	94 (29.2)	39 (35.1)	39 (35.5)	73 (32.0)
Scores shown were assessed at beginning of each stage for ADHERE and at ADHERE stage A baseline for ADHERE+				
INCAT score, mean (SD)[c]	4.6 (1.7)	3.1 (1.5)	3.3 (1.6)	4.5 (1.6)
I-RODS score, mean (SD)[c]	40.1 (14.7)	53.6 (17.9)	51.2 (15.4)	41.2 (15.4)
Grip strength (dominant hand), kPa, mean (SD)[d]	38.5 (24.2)	54.9 (23.6)	58.0 (25.1)	39.0 (23.6)

CDAS, CIDP disease activity status; CIDP, chronic inflammatory demyelinating polyneuropathy; INCAT, Inflammatory Neuropathy Cause and Treatment; I-RODS, Inflammatory Rasch-built Overall Disability Scale; IVIg, intravenous immunoglobulin; PH20, recombinant human hyaluronidase PH20; SC, subcutaneous; SCIg, subcutaneous immunoglobulin; SD, standard deviation.

[a]Unstable active disease was defined as abnormal examination with progressive or relapsing course.[2] [b]Off treatment was defined as participants who had never received CIDP treatment (treatment naïve) or who had not received CIDP treatment (corticosteroids, IVIg, or SCIg) within 6 months of trial entry. [c]Lower scores represent improvement on INCAT, while higher scores represent improvement for I-RODS. [d]Grip strength scores in nondominant hand were similar. [e]Participants in ADHERE+ completed or deteriorated during ADHERE stage B, or terminated ADHERE early as the 88[th] event has been reached.

ADHERE+ data cut-off: February 16, 2024.

1. Allen JA, et al. *Lancet Neurol.* 2024;23(10):1013–24. 2. Gorson KC, et al. *J Peripher Nerv Syst.* 2010;15(4):326–33.

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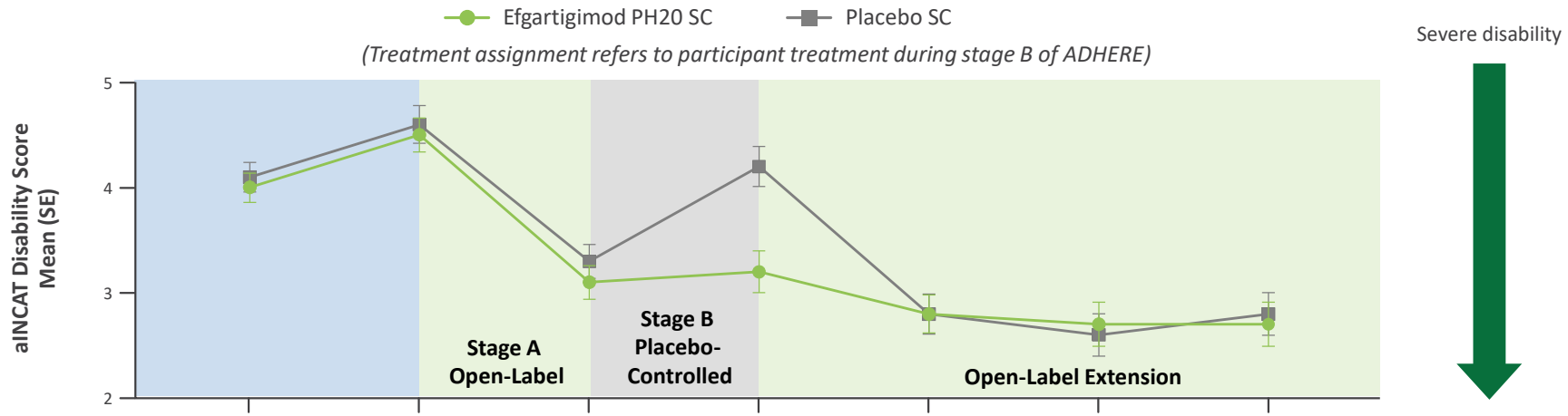
Among ADHERE Stage A Responders, Efgartigimod PH20 SC Treatment Resulted in Clinically Meaningful aINCAT Score[a] Improvements in ADHERE+



Participants, n	Run-in Baseline ADHERE	Stage A Baseline ADHERE	Stage B Baseline ADHERE	Stage B Last Assessment ADHERE	Week 12 ADHERE+	Week 24 ADHERE+	Week 36 ADHERE+
Efgartigimod PH20 SC	97	97	97	97	85	77	76
Placebo SC	94	94	94	93	79	78	74

Improvement coincides with time on and off efgartigimod treatment.
For stage A responders, mean efficacy scores on efgartigimod in ADHERE+ were better than at ADHERE run-in baseline.

aINCAT, adjusted Inflammatory Neuropathy Cause and Treatment; PH20, recombinant human hyaluronidase PH20; SC, subcutaneous; SE, standard error.

[a]A decrease of ≥1 points in aINCAT score[1] is considered a minimal clinically important difference[2].

Post hoc analysis included ADHERE stage A responders with run-in baseline values.

ADHERE+ data cut-off: February 16, 2024.

1. Breiner A, et al. *Muscle Nerve*. 2014;50(1):40–6. 2. Van den Bergh PYK. *Eur J Neurol.* 2021;28(11):3556–83.

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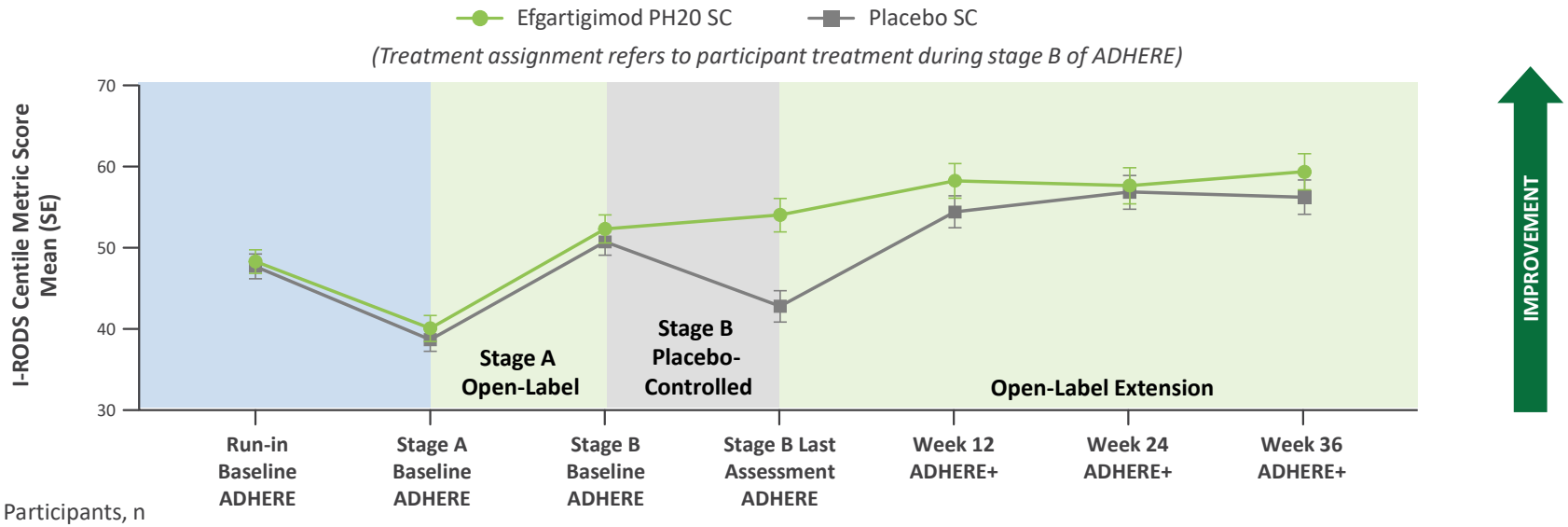
Among ADHERE Stage A Responders, Efgartigimod PH20 SC Treatment Resulted in Clinically Meaningful Improvements in I-RODS Centile Metric Score[a] in ADHERE+



Participants, n							
Efgartigimod PH20 SC	97	97	97	97	84	78	77
Placebo SC	94	94	94	92	80	78	73

Improvement coincides with time on and off efgartigimod treatment.
For stage A responders, mean efficacy scores on efgartigimod in ADHERE+ were better than at ADHERE run-in baseline.

I-RODS, Inflammatory Rasch-built Overall Disability Scale; PH20, recombinant human hyaluronidase PH20; SC, subcutaneous; SE, standard error.

[a]An increase of ≥4 points in I-RODS score[1] is considered a minimal clinically important difference[2].

Post hoc analysis included ADHERE stage A responders with run-in baseline values.

ADHERE+ data cut-off: February 16, 2024.

1. van Nes SI, et al. *Neurology*. 2011;76(4):337–45. 2. Van den Bergh PYK, et al. *Eur J Neurol*. 2021;28(11):3556–83.

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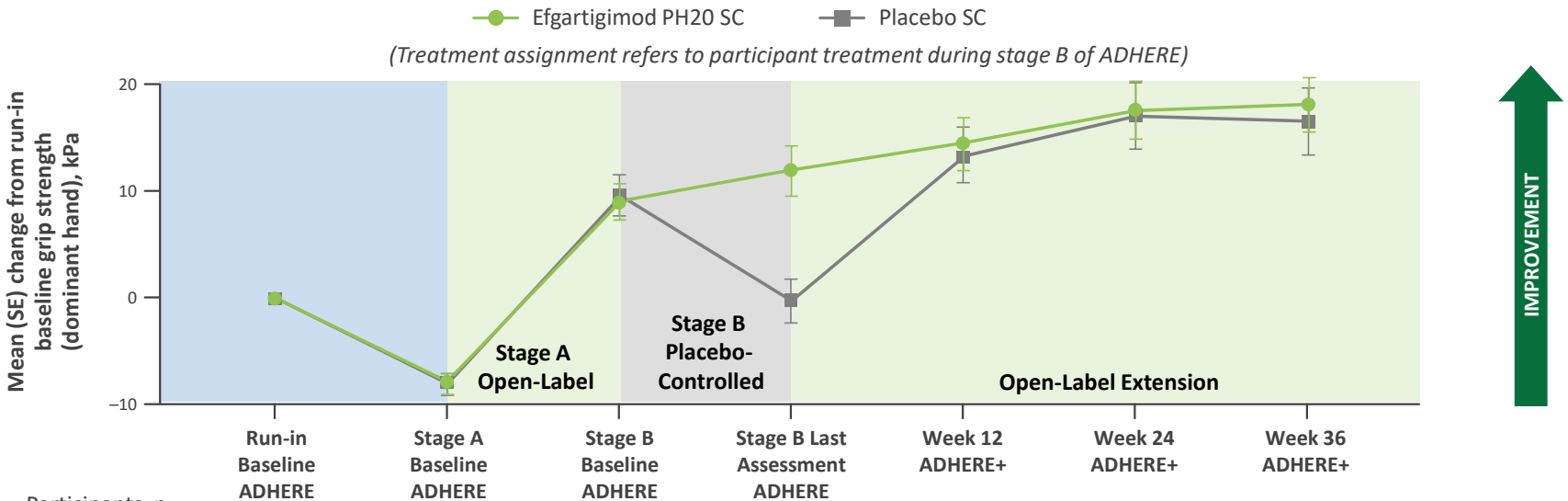
Among ADHERE Stage A Responders, Efgartigimod PH20 SC Treatment Resulted in Clinically Meaningful Improvement in Dominant Hand Grip Strength[a] in ADHERE+



Participants, n							
Efgartigimod PH20 SC	97	97	97	97	85	78	76
Placebo SC	94	94	94	93	78	78	73

Improvement coincides with time on and off efgartigimod treatment.
For stage A responders, mean efficacy scores on efgartigimod in ADHERE+ were better than at ADHERE run-in baseline.

PH20, recombinant human hyaluronidase PH20; SC, subcutaneous; SE, standard error.

[a]An increase of ≥8 kPa in grip strength[1] is considered a minimal clinically important difference[2].

Post hoc analysis included ADHERE stage A responders with run-in baseline values.

ADHERE+ data cut-off: February 16, 2024.

1. Vanhoutte EK. *Eur J Neurol.* 2013;20(5):748–55. 2. Van den Bergh PYK, et al. *Eur J Neurol.* 2021;28(11):3556–83.

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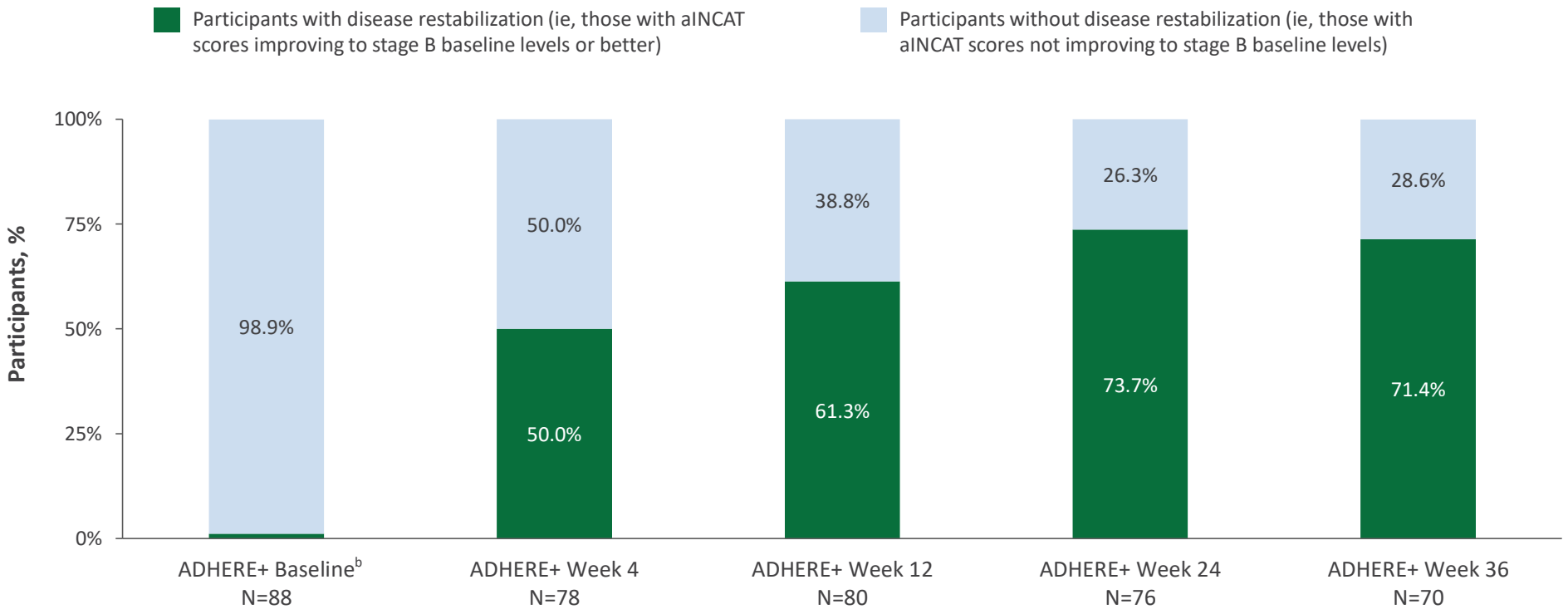
Among Participants With Disease Relapse[a] in ADHERE Stage B, Restabilization Occurred Early and Increased Over Time in ADHERE+



aINCAT, adjusted Inflammatory Neuropathy Cause and Treatment; PH20, recombinant human hyaluronidase PH20; SC, subcutaneous.

[a]Disease relapse was based on aINCAT deterioration, defined as a ≥1-point increase in aINCAT compared with stage B baseline, which was confirmed at a consecutive visit after the first 1-point increase in aINCAT or not confirmed for participants with ≥2-point increase in aINCAT compared with stage B baseline. An aINCAT score of ≤ −1 represents an improvement, a score of 0 represents no change, and a score of ≥ 1 represents deterioration. [b]ADHERE+ Baseline: efgartigimod PH20 SC, n=30; placebo SC, n=58; treatment assignment refers to participant treatment during Stage B of ADHERE.

Post hoc analysis included participants in ADHERE+ with disease relapse in ADHERE stage B.

ADHERE+ data cut-off: February 16, 2024.

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Efgartigimod Was Well Tolerated and Most TEAEs Were Mild or Moderate in Severity in ADHERE and ADHERE+

	ADHERE[1]			ADHERE+ Open-Label Extension
	Open-Label Stage A	Double-Blinded Stage B		Mean (SD) study duration[c]= 60.61 (32.87) weeks
n (%) [event rate[a]]	Efgartigimod PH20 SC (N=322; PYFU=46.9)	Efgartigimod PH20 SC (n=111; PYFU=56.7)	Placebo (n=110; PYFU=42.1)	Efgartigimod PH20 SC (N=228; PYFU=263.0)
Any TEAE	204 (63.4) [13.4]	71 (64.0) [3.5]	62 (56.4) [5.1]	171 (75.0) [3.1]
Any SAE	21 (6.5) [0.5]	6 (5.4) [0.1]	6 (5.5) [0.2]	35 (15.4) [0.25]
Any injection site reactions	62 (19.3) [2.6]	16 (14.4) [0.4]	7 (6.4) [0.2]	24 (10.6) [0.18]
Discontinued due to TEAEs	22 (6.8) [0.5]	3 (2.7) [0.05]	1 (0.9) [0.02]	18 (7.9) [0.14]
Deaths[b]	2 (0.6) [0.04]	0	1 (0.9) [0.02]	2 (0.9) [0.008]
Most common TEAEs (≥5% of participants in the total group in ADHERE+)				
COVID-19	7 (2.2) [0.17]	19 (17.1) [0.35]	14 (12.7) [0.33]	37 (16.2) [0.14]
Nasopharyngitis	5 (1.6) [0.11]	5 (4.5) [0.09]	3 (2.7) [0.07]	16 (7.0) [0.08]
Upper respiratory tract infection	11 (3.4) [0.26]	2 (1.8) [0.05]	11 (10.0) [0.26]	24 (10.5) [0.15]
Urinary tract infection	5 (1.6) [0.13]	2 (1.8) [0.05]	2 (1.8) [0.05]	12 (5.3) [0.06]
Headache	16 (5.0) [0.6]	4 (3.6) [0.11]	2 (1.8) [0.05]	14 (6.1) [0.09]

AE, adverse event; CIDP, chronic inflammatory demyelinating polyneuropathy; COVID-19, coronavirus disease 2019; PH20, recombinant human hyaluronidase PH20; PYFU, participants years of follow-up; SAE, serious adverse event; SC, subcutaneous; TEAE, treatment-emergent adverse event.

[a]Event rates were calculated as the number of events divided by the PYFU. [b]Two deaths (cardiac arrest and deterioration of CIDP) in ADHERE stage A were considered unlikely related to efgartigimod PH20 SC by the investigator; one death (pneumonia) in the placebo SC arm of ADHERE stage B was considered treatment related by the investigator; in ADHERE+, one participant had a fatal SAE of CIDP deterioration (considered to be related to efgartigimod PH20 SC by the investigator) and one participant had a fatal SAE of cardiac arrest (considered not related to efgartigimod PH20 SC or study procedures by the investigator and sponsor). [c]Study duration = (date of last contact − earliest date of informed consent form or date of rollover + 1 day) / 7.

ADHERE+ data cut-off: February 16, 2024.

1. Allen JA, et al. *Lancet Neurol.* 2024;23(10):1013–24 .

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Conclusions

Interim results from the ongoing ADHERE+ trial indicate that treatment with efgartigimod PH20 SC results in long-term clinical efficacy in participants with CIDP

- Clinically meaningful improvements in functional ability and dominant hand grip strength in ADHERE+, irrespective of ADHERE stage B treatment, were observed with efgartigimod PH20 SC

Majority of participants on efgartigimod PH20 SC who experienced disease relapse during ADHERE stage B restabilized, and half did so as early as Week 4 of ADHERE+

Weekly efgartigimod PH20 SC remained well tolerated

- A similar safety profile was observed between ADHERE and ADHERE+, with no increased rate or severity of TEAEs with longer exposure

CIDP, chronic inflammatory demyelinating polyneuropathy; PH20, recombinant human hyaluronidase PH20; SC, subcutaneous; TEAE, treatment-emergent adverse event.

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