

Efficacy and safety of Descartes-08 in AChR+ generalized myasthenia gravis: subgroup analysis of a Phase 2b randomized, placebo-controlled trial

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Presented by Tahseen Mozaffar MD

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Myasthenia gravis represents significant and enduring burden for patients

MG is a **chronic, autoimmune neuromuscular disorder** characterized by **fluctuating and debilitating muscle weakness and fatigue** that impacts



106,000+

people in the US alone*¹⁻³

Current treatment options rely on chronic immunosuppression rather than directly targeting pathogenic pathways, resulting in:

- **Suboptimal depth and durability of response, leading to incomplete symptom control**⁴⁻⁶
- **Reliance on chronic or cyclic treatment** to maintain disease control^{4,7}
- **Off-target effects** associated with prolonged immunosuppression that impact patient **quality of life**⁴

MG is a **chronic disease** with **severe patient burden** and **significant unmet need for durable, tolerable treatments**

*106,306 calculated from the MG prevalence rate of 37.0 per 100,000,¹ the US population of over 340 million in 2025,² and the proportion of patients with adult-onset MG (85–90%).³ MG, myasthenia gravis. 1. Rodrigues E, et al. *Muscle Nerve*. 2024;69(2):166–71; 2. US Census; 3. Orphanet. Adult-onset myasthenia gravis. Available at <https://www.orpha.net/en/disease/detail/391490>. Accessed February 2026; 4. Alhaidar MK, et al. *J Clin Med*. 2022;11:1597; 5. Jackson K, et al. *Neurol Ther*. 2023;12:107–28; 6. Smith et al. Poster presented at: American Academy of Neurology Annual Meeting; April 5–9, 2025; San Diego, CA, USA; 7. Stathopoulos P, et al. *Ann N Y Acad Sci*. 2018;1412:154–65.

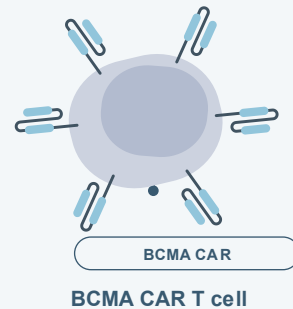
Descartes-08 is an mRNA CAR T-cell product targeting pathogenic cells expressing B-cell maturation antigen (BCMA)

Descartes-08

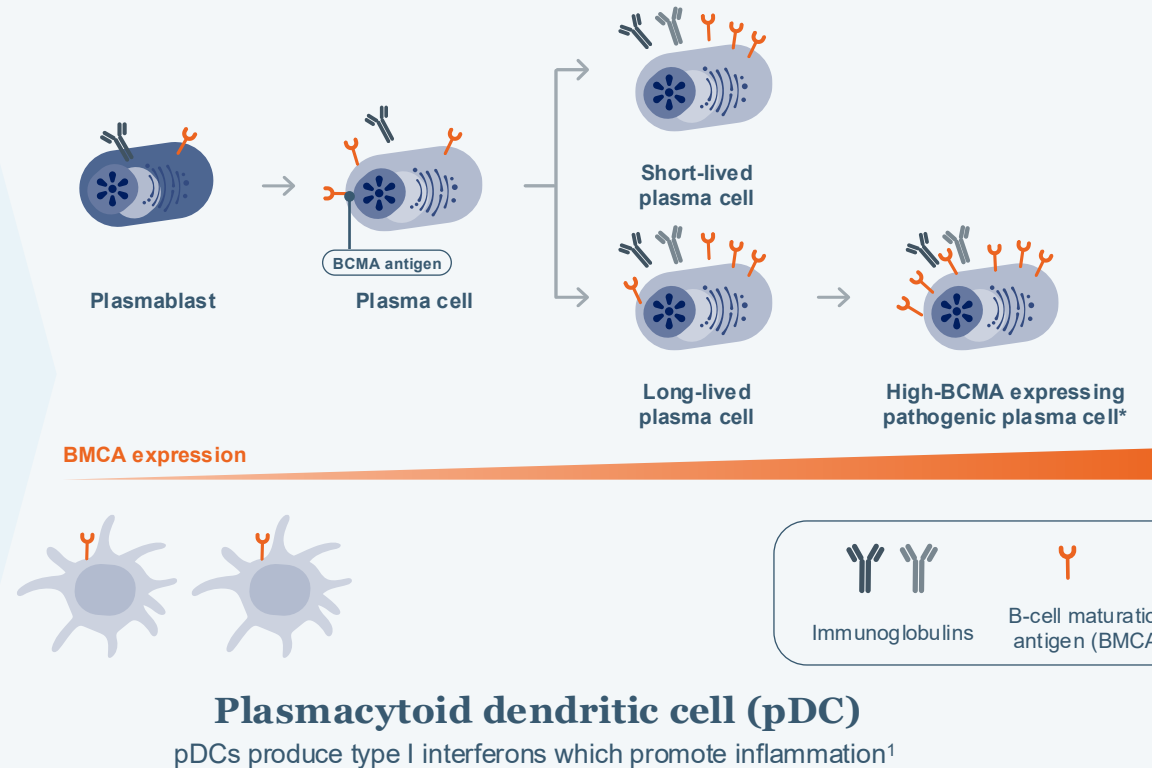
mRNA CAR T-cell therapy¹⁻⁴

- mRNA engineering (non-integrating) leads to transient expression of anti-BCMA CARs

Descartes-08 selectively targets dysregulated BCMA+ immune cells to precisely reset the immune system by reducing key drivers of MG pathogenesis^{2,3,5,6}



Terminally-differentiated antibody-secreting cells

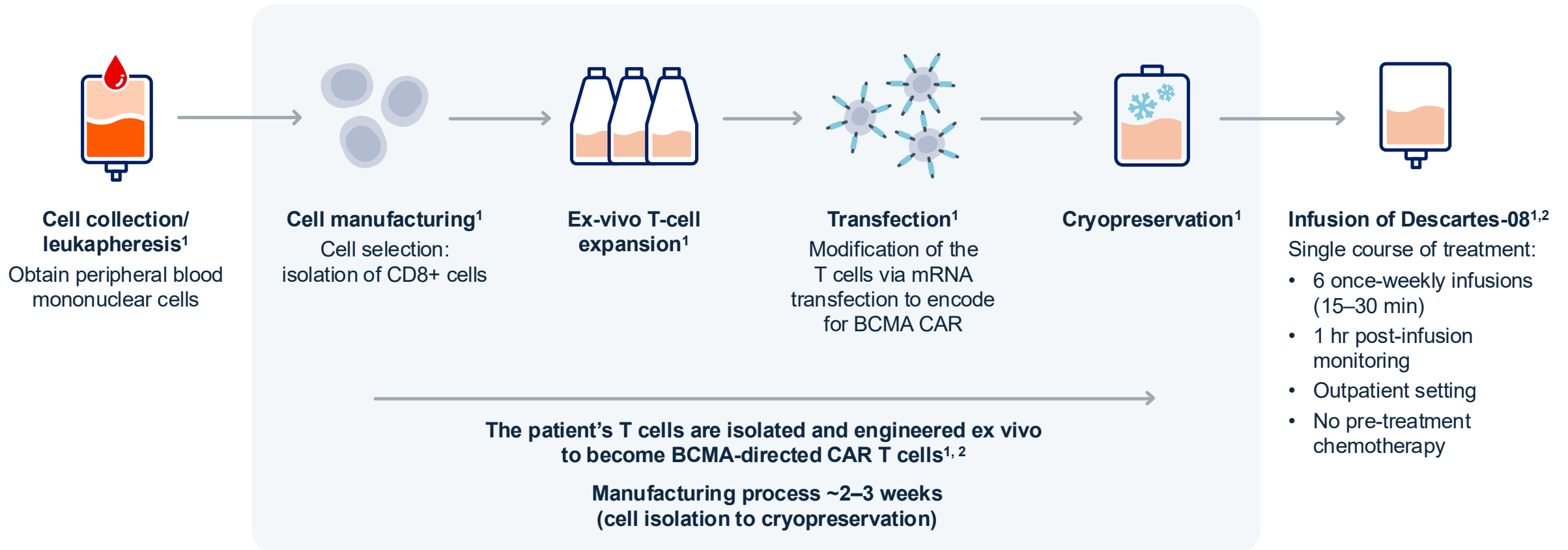


*High-BCMA expressing cells are associated with MG pathology.

BCMA, B-cell maturation antigen; CAR, chimeric antigen receptor; MG, myasthenia gravis; mRNA, messenger ribonucleic acid; pDC, plasmacytoid dendritic cell.

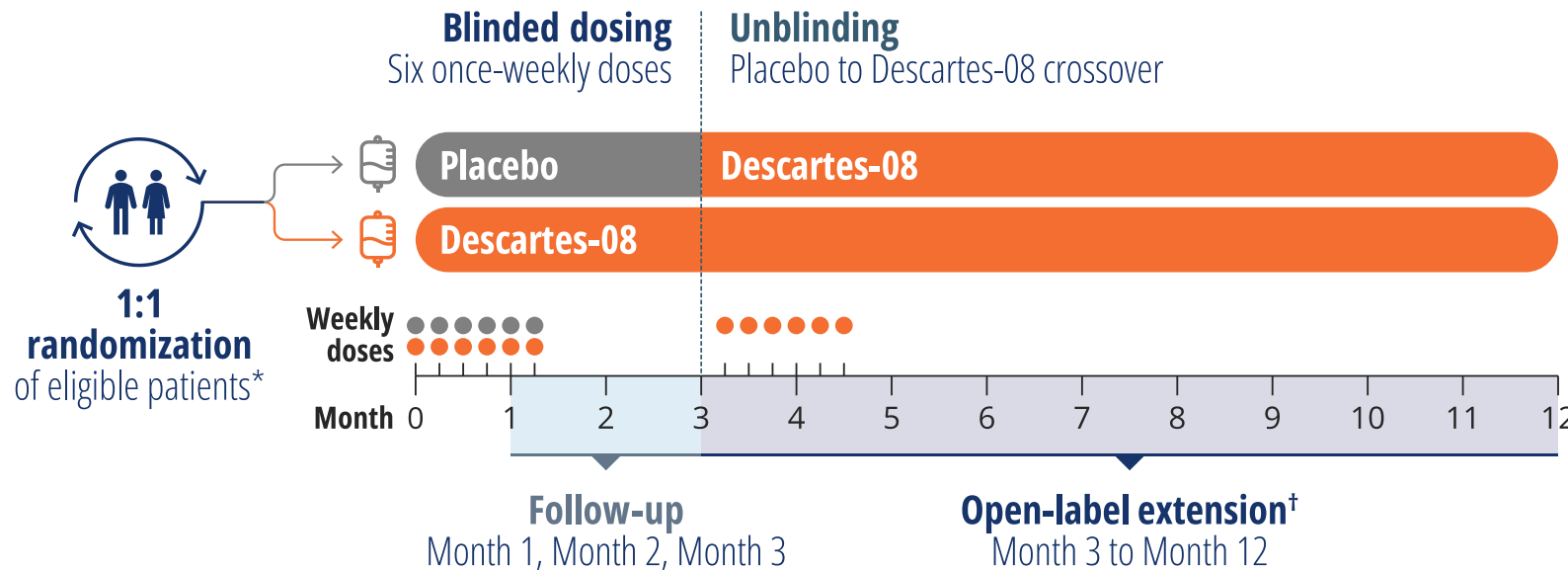
1. English EP, et al. *Sci Transl Med*. 2024;16:eado2084; 2. Granit V, et al. *Lancet Neurol*. 2023;22:578-90; 3. Chahin N, et al. *Ann Clin Transl Neurol*. 2025;12:2358-66; 4. Vu T, et al. *Nat Med*. 2026. doi: 10.1038/s41591-025-04171-y [online ahead of print]; 5. Fedak RR, et al. *Nat Med*. 2026. doi: 10.1038/s41591-025-04170-z [online ahead of print]; 6. Uzawa A, et al. *Clin Exp Immunol*. 2021;203:366-74.

Descartes-08 is an mRNA CAR T-cell product targeting pathogenic cells expressing B-cell maturation antigen



Study design

Eligible patients had **MG-ADL score ≥ 6 , MGFA Class II–IV, non-MuSK+ gMG[‡]**



Primary endpoint:

- The proportion of patients achieving a ≥ 5 -point decrease in MGC at Month 3 compared with baseline

Secondary endpoints:

- Mean change from baseline in MG-ADL, QMG, MGC, and MG-QoL-15r scores at each post-infusion visit
- Safety and tolerability of Descartes-08 in patients with gMG



Predefined subgroup analysis → AChR+ patients

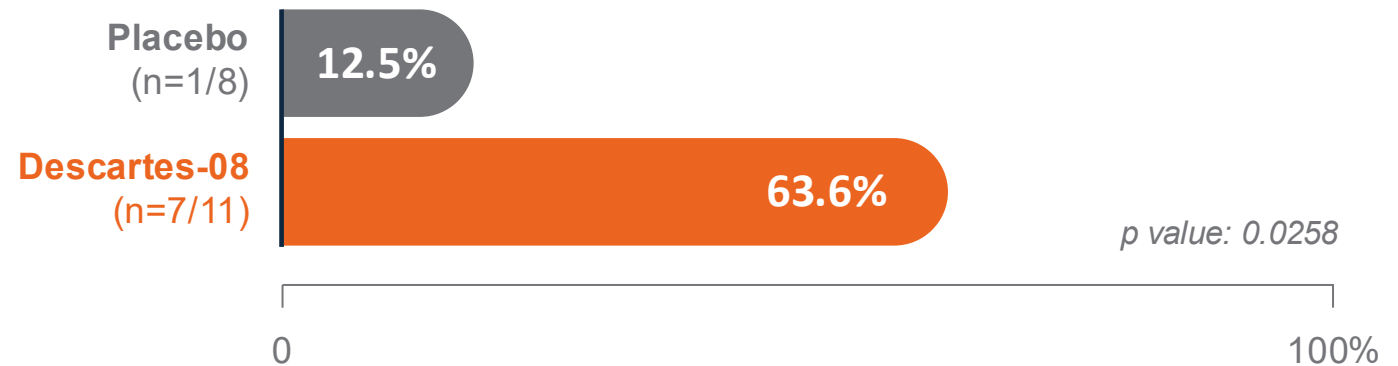
Patient demographics and baseline characteristics were comparable between treatment cohorts

AChR+, positive for autoantibodies against the acetylcholine receptor; gMG, generalized myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; MG-QoL-15r, Myasthenia Gravis Quality of Life 15 revised; MGC, Myasthenia Gravis Composite; MGFA, Myasthenia Gravis Foundation of America; MuSK+, positive for antibodies against muscle-specific tyrosine kinase; QMG, Quantitative Myasthenia Gravis. *Patients underwent leukapheresis for Descartes-08 manufacturing purposes ahead of randomization. †During open-label extension, follow-up for patients randomized to the placebo to Descartes-08 crossover treatment cohort occurred at Months 3, 4, 6, 9, and 12 post infusion; follow-up for those randomized to the Descartes-08 cohort occurred at Months 4, 6, 9, and 12. Permitted concomitant medications were pyridostigmine, corticosteroids (≤ 40 mg prednisone daily or equivalent), azathioprine, mycophenolate mofetil, and complement inhibitors, provided a stable dose at least 8 weeks prior to first infusion. Vu T, *et al. Nat Med.* 2026. doi: 10.1038/s41591-025-04171-y [online ahead of print].

A significantly higher proportion of AChR+ gMG patients treated with Descartes-08 achieved MGC response (≥ 5 -point improvement) at Month 3 versus placebo

The proportion of MGC score responders (≥ 5 -point improvement) was **significantly higher** in the **Descartes-08 AChR+ cohort** vs placebo at Month 3: **63.6%** vs **12.5%** in placebo group ($p=0.0258$)

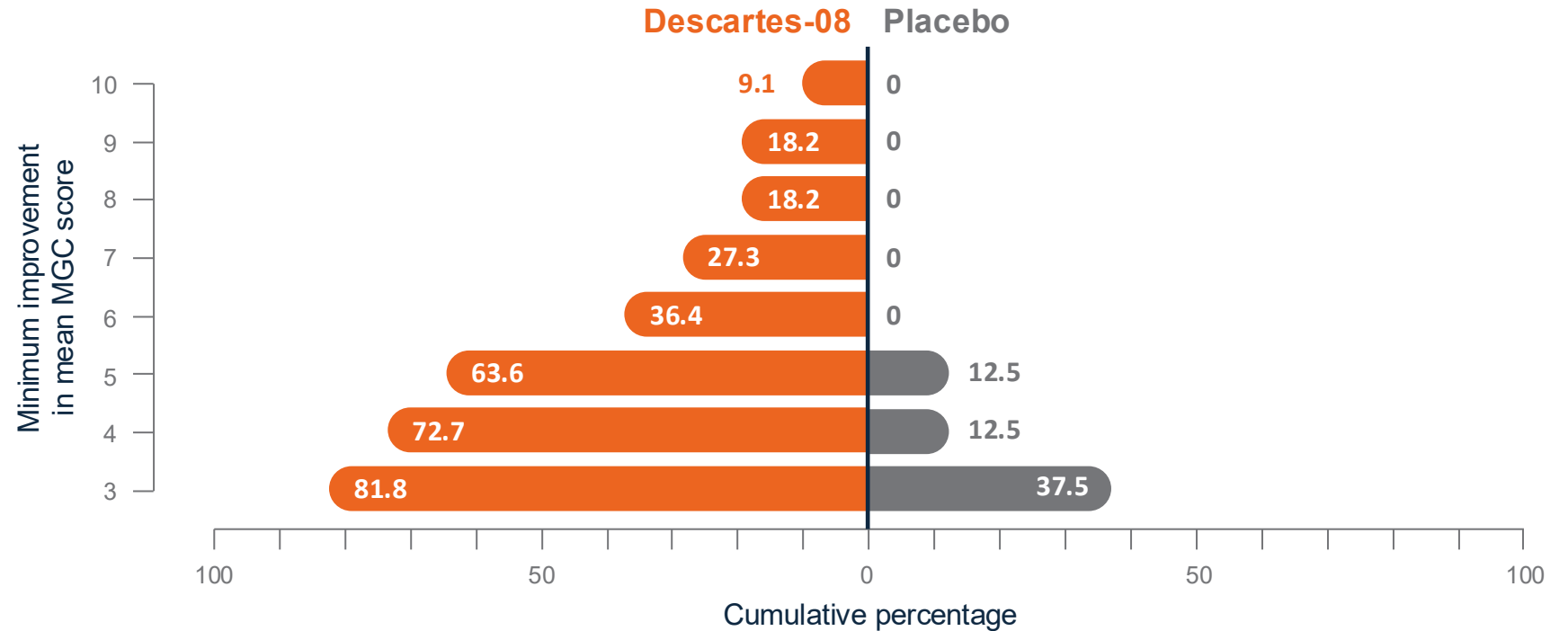
Primary endpoint: ≥ 5 -point improvement in the MGC score at Month 3



A higher proportion of AChR+ gMG patients treated with Descartes-08 achieved meaningful improvements in MGC scores at Month 3 versus placebo

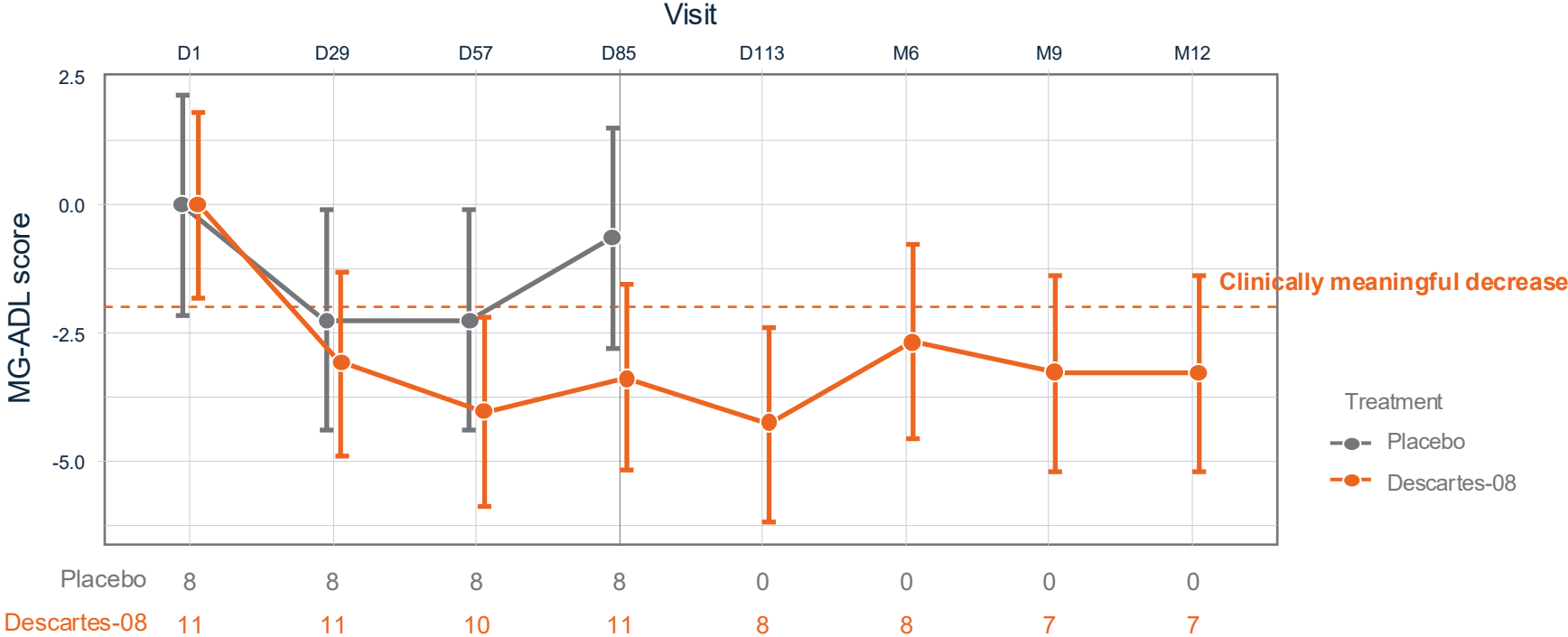
A greater proportion of **AChR+ patients** randomized to **Descartes-08** achieved a ≥ 3 -point improvement in MGC vs placebo: **81.8%** vs **37.5%**

Minimum point improvement (≥ 3 -point) in mean MGC at Month 3



Meaningful reductions in MG-ADL at Month 3 were sustained through Month 12 with Descartes-08 in AChR+ gMG

Mean change from baseline in MG-ADL score²



There was a **significant** and **clinically meaningful reduction** in mean [SD] MG-ADL score at Month 3 for the Descartes-08 AChR+ group versus placebo (-3.4 [2.8] vs -0.6 [2.9], p=0.0409), which was **sustained through Month 12**¹

mITT (modified intention-to-treat) population. Data are presented as least-squares means with 95% CI, using a mixed model for repeated measures. Orange dashed line represents the threshold for a clinically meaningful reduction in MG-ADL score. AChR+, positive for autoantibodies against the acetylcholine receptor; CI, confidence interval; D, day; gMG, generalized myasthenia gravis; M, month; MG-ADL, Myasthenia Gravis Activities of Daily Living; SD, standard deviation. 1. Vu T, et al. Nat Med. 2026. doi: 10.1038/s41591-025-04171-y [online ahead of print]; 2. Cartesian Therapeutics. Data on file.

Descartes-08 was well tolerated with no reported cases of CRS, neurotoxicity, immunosuppression, or clinically significant cytopenias in AChR+ gMG

Treatment-emergent AEs from Day 1 to Month 3 for Descartes-08 and placebo

	Descartes-08 (n=16), n (%)	Placebo (n=12), n (%)
Any AE	16 (100)	12 (100)
Serious AE	4 (25.0)	2 (16.7)
Any AE leading to discontinuation of study drug	1 (6.3)	1 (8.3)
Any infusion-related reaction	13 (81.3)	7 (58.3)
Headache	7 (43.8)	5 (41.7)
Nausea	7 (43.8)	3 (25.0)
Fever	8 (50.0)	1 (8.3)
Chills	10 (62.5)	0
Myalgia	5 (31.3)	0
Any infection	6 (37.5)	5 (41.7)
Upper respiratory infection	2 (12.5)	3 (25.0)
Herpes simplex reactivation	2 (12.5)	0
Other common AEs		
Fatigue	6 (37.5)	1 (8.3)
Limb swelling	1 (6.3)	1 (8.3)
Diarrhea	2 (12.5)	0

The **most common AEs** through Month 3 for the Descartes-08 treatment cohort were **chills, headache, fever, and nausea**, which typically resolved 24 hours post infusion



No reports of cytokine release syndrome (CRS) or immune effector cell-associated neurotoxicity syndrome



No increased risk of infections, no immunosuppression or cytopenias with Descartes-08 versus placebo



No AEs reported after Month 3 post infusion

Conclusions



Descartes-08 is a **chemotherapy-free mRNA CAR T-cell** therapy that directly targets BCMA-expressing cells implicated in MG pathogenesis



A single, outpatient treatment course of six once-weekly infusions of **Descartes-08** demonstrated **clinically meaningful** and **sustained improvements** in MG symptoms through Month 12 in patients with AChR+ gMG



Descartes-08 was **well tolerated**, with no reported cases of CRS, neurotoxicity, immune suppression, or clinically significant cytopenias



The pivotal **Phase 3 AURORA trial (NCT06799247)** is **currently enrolling** adult AChR+ gMG patients

For further information on the data presented, **scan the QR code**

