Efficacy of long-term zilucoplan treatment in subgroups of patients with generalised myasthenia gravis: A 120-week analysis of RAISE-XT

EAN 2026, Geneva, Switzerland; 27–30 June 2026

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Introduction

- Zilucoplan, a macrocyclic peptide, is a potent complement C5 inhibitor approved for the treatment of adults with anti-AChR Ab+ gMG[1]
- RAISE-XT (NCT04225871), a Phase 3, multicentre, OLE study,[2] showed clinically meaningful and sustained improvements in MG-specific outcomes in patients with gMG during treatment with zilucoplan up to Week 120[3]
- **In this analysis, we evaluated the efficacy of zilucoplan in patients with gMG, in subgroups defined by patient and disease characteristics**

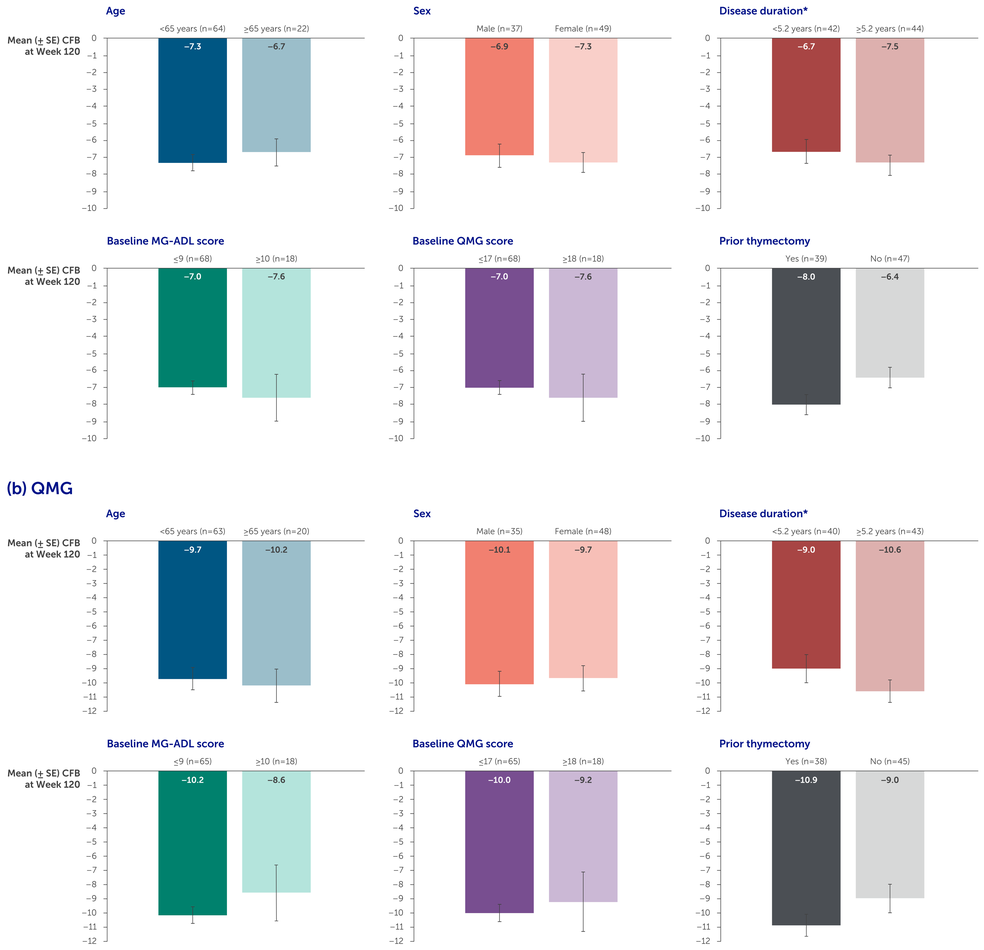
Methods

- Adults with anti-AChR Ab+ gMG who completed the Phase 2 (NCT03315130) or Phase 3 (NCT04115293; RAISE) study could enter RAISE-XT and self-administer daily subcutaneous zilucoplan 0.3 mg/kg injections[2]
- In this analysis, we report CFB to Week 120 in MG-ADL, QMG, MG-QoL15r and Neuro-QoL Short Form Fatigue scores for the following pre-specified subgroups:
  - Age (<65 years and ≥65 years)
  - Sex (male and female)
  - Disease duration from diagnosis (<median and ≥median)
  - Baseline MG-ADL score (≤9 and ≥10)
  - Baseline QMG score (≤17 and ≥18)
  - Prior thymectomy (yes and no)
- The primary outcome of RAISE-XT was incidence of TEAEs
- The interim data cut-off date was 11 November 2023

Results

- In total, 200 patients were enrolled in RAISE-XT
- Improvements from baseline were observed in MG-ADL and QMG scores through to Week 120 in all subgroups (**Figure 1**)
  - Similar improvements were observed for MG-QoL15r and Neuro-QoL Short Form Fatigue scores (**Table 1**)
- TEAEs occurred in 97.0% (n=194/200) of patients
  - Most TEAEs were mild or moderate in intensity and were not related to the study drug

Figure 1 At Week 120, improvements from baseline were observed in (a) MG-ADL and (b) QMG scores across pre-specified subgroups



mITT population. *Disease duration from diagnosis categorised as < or ≥ the median (5.2 years).

Table 1 Improvements from baseline were observed in MG-QoL15r and Neuro-QoL Short Form Fatigue scores across pre-specified subgroups at Week 120

		MG-QoL15r score, mean (SE) CFB	Neuro-QoL Short Form Fatigue score, mean (SE) CFB
Age	<65 years	**−11.6** (1.2) n=61	**−11.5** (1.4) n=45
	≥65 years	**−7.7** (2.1) n=19	**−8.6** (3.0) n=16
Sex	Male	**−9.8** (1.6) n=34	**−11.0** (2.2) n=25
	Female	**−11.3** (1.3) n=46	**−10.5** (1.6) n=36
Disease duration*	<5.2 years	**−8.9** (1.4) n=38	**−8.6** (1.8) n=30
	≥5.2 years	**−12.2** (1.5) n=42	**−12.8** (1.9) n=31
Baseline MG-ADL score	≤9	**−10.4** (1.1) n=62	**−10.4** (1.5) n=46
	≥10	**−11.7** (2.3) n=18	**−11.7** (2.8) n=15
Baseline QMG score	≤17	**−10.2** (1.1) n=63	**−10.2** (1.4) n=47
	≥18	**−12.3** (2.7) n=17	**−12.4** (3.2) n=14
Prior thymectomy	Yes	**−12.5** (1.6) n=36	**−12.7** (1.9) n=29
	No	**−9.1** (1.2) n=44	**−8.9** (1.7) n=32

mITT population. *Disease duration from diagnosis categorised as < or ≥ the median (5.2 years).

Summary and conclusions

In this analysis, we assessed the long-term effect of zilucoplan, as defined by patient and disease characteristics in patients with anti-AChR Ab+ gMG in RAISE-XT

Long-term zilucoplan treatment demonstrated consistent improvements in MG-specific outcomes up to Week 120 across pre-specified subgroups

These data highlight that zilucoplan demonstrated long-term efficacy, irrespective of patient and disease characteristics, including age, disease duration and disease severity

Abbreviations: Ab+, antibody positive; AChR, acetylcholine receptor; C5, component 5; CFB, change from baseline; gMG, generalised myasthenia gravis; MG, myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; MG-QoL15r, Myasthenia Gravis Quality of Life 15-item revised; mITT, modified intention to treat; Neuro-QoL, Quality of Life in Neurological Disorders; OLE, open-label extension; QMG, Quantitative Myasthenia Gravis; SE, standard error; TEAE, treatment-emergent adverse event.

Acknowledgements: This study was funded by UCB. The authors acknowledge Bea Perks, PhD, for Ogilvy Health, London, UK, for editorial assistance, which was funded by UCB. The authors thank Veronica Porkess, PhD, of UCB for publication and editorial support. The authors thank the patients and their caregivers, in addition to the investigators and their teams who contributed to this study.

Author disclosures: M. Isabel Leite is funded by the NHS (Myasthenia and Related Disorders Service and National Specialised Commissioning Group for Neuromyelitis Optica, UK) and by the University of Oxford, UK. She has been awarded research grants from UK associations for patients with myasthenia and with muscular disorders (Myaware and Muscular Dystrophy UK, respectively) and the University of Oxford. She has received speaker honoraria or travel grants from Biogen, the Guthy-Jackson Charitable Foundation, Novartis and UCB. She serves on scientific or educational advisory boards for argenx, Horizon Therapeutics (now Amgen) and UCB. Miriam Freimer has served as a paid Consultant for Arcelix, argenx and UCB. She receives research support from Abcuro, Alnylam Pharmaceuticals, argenx, Avidity Biosciences, COUR Pharmaceuticals, Dianthus Therapeutics, Fulcrum Therapeutics, Johnson & Johnson Innovative Medicine, the NIH, ReMeGen Biosciences and UCB. Channa Hewamadduma is Principal Investigator/NCI in clinical trials in various neuromuscular disorders sponsored by Alexion Pharmaceuticals, argenx, Avidity, Cartesian, Dyne, Janssen Pharmaceuticals (now Johnson & Johnson Innovative Medicine), Novartis, PegGen, Regeneron Pharmaceuticals and UCB. He has received funding for consultancy on scientific or educational advisory boards for argenx, Biogen, Lupin, Roche and UCB, and has received an investigator-led research grant from UCB. His study activities were supported by a Sheffield NIHR BRC UK centre grant. He is a trustee of the myasthenia gravis patient organisation Myaware. Angelina Maniaol has received payment for travel, meeting attendance, consulting honoraria or advisory board participation from Alexion Pharmaceuticals, argenx, Biogen, Johnson & Johnson and UCB. Kimiaki Utsugisawa has served as a paid Consultant for argenx, Chugai Pharmaceutical, HanAll Biopharma, Janssen Pharmaceuticals (now Johnson & Johnson Innovative Medicine), Merck, Mitsubishi Tanabe Pharma, UCB and Viela Bio (now Amgen); he has received speaker honoraria from Alexion Pharmaceuticals, argenx, the Japan Blood Products Organization and UCB. Michael D. Weiss has received honoraria for serving on scientific advisory boards for Alexion Pharmaceuticals, Amgen, Amylyx Pharmaceuticals, argenx, Biogen, Immunovant, Mitsubishi Tanabe Pharma and Ra Pharmaceuticals (now UCB), consulting honoraria from CSL Behring and Cytokinetics, and speaker honoraria from Amgen, Soleo Health, and UCB. He has been a paid Medical Monitor for a NeuroNEXT study. Babak Boroojerdi, Paul Mahoney and Natasa Savic are employees and shareholders of UCB. James F. Howard Jr. has received research funding (paid to his institution) from Ad Scientiam, Alexion/AstraZeneca Rare Disease, argenx, Cartesian Therapeutics, Centers for Disease Control and Prevention, Merck EMD Serono, MGFA, Muscular Dystrophy Association, NIH, NMD Pharma and UCB; honoraria/consulting fees from AcademicCME, Alexion/AstraZeneca Rare Disease, Amgen, argenx, Biohaven Ltd, Cartesian Therapeutics, CheckRare CME, CorEvitas, CurieBio, H. Lundbeck A/S, Japan Tobacco Company, Kyverna Therapeutics, Merck EMD Serono, NMD Pharma, Novartis, PeerView CME, Physicians Education Resource (PER, MJH) CME, Regeneron Pharmaceuticals, Seismic Therapeutics, Tolerazia AB, UCB and Vor Biopharma; non-financial support from Alexion/AstraZeneca Rare Disease, argenx, Biohaven Ltd, Cartesian Therapeutics, Tolerazia AB, UCB and Vor Biopharma.

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