UCB in gMG: Investor and Analyst Briefing Call

13:00 - 14:00 CEST

Tuesday, 10th of May 2022

Inspired by **patients.**
Driven by **science.**

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This presentation contains forward-looking statements, including, without limitation, statements containing the words "believes", "anticipates", "expects", "intends", "plans", "seeks", "estimates", "may", "will", "continue" and similar expressions. These forward-looking statements are based on current plans, estimates and beliefs of management. All statements, other than statements of historical facts, are statements that could be deemed forward-looking statements, including estimates of revenues, operating margins, capital expenditures, cash, other financial information, expected legal, arbitration, political, regulatory or clinical results or practices and other such estimates and results. By their nature, such forward-looking statements are not guarantees of future performance and are subject to known and unknown risks, uncertainties, and assumptions which might cause the actual results, financial condition, performance or achievements of UCB, or industry results, to be materially different from any future results, performance, or achievements expressed or implied by such forward-looking statements contained in this presentation.

Important factors that could result in such differences include but are not limited to: the global spread and impact of COVID-19, changes in general economic, business and competitive conditions, the inability to obtain necessary regulatory approvals or to obtain them on acceptable terms or within expected timing, costs associated with research and development, changes in the prospects for products in the pipeline or under development by UCB, effects of future judicial decisions or governmental investigations, safety, quality, data integrity or manufacturing issues; potential or actual data security and data privacy breaches, or disruptions of our information technology systems, product liability claims, challenges to patent protection for products or product candidates, competition from other products including biosimilars, changes in laws or regulations, exchange rate fluctuations, changes or uncertainties in tax laws or the administration of such laws, and hiring and retention of its employees. There is no guarantee that new product candidates will be discovered or identified in the pipeline, or that new indications for existing products will be developed and approved. Movement from concept to commercial product is uncertain; preclinical results do not guarantee safety and efficacy of product candidates in humans. So far, the complexity of the human body cannot be reproduced in computer models, cell culture systems or animal models. The length of the timing to complete clinical trials and to get regulatory approval for product marketing has varied in the past and UCB expects similar unpredictability going forward. Products or potential products which are the subject of partnerships, joint ventures or licensing collaborations may be subject to disputes between the partners or may prove to be not as safe, effective or commercially successful as UCB may have believed at the start of such partnership. UCB's efforts to acquire other products or companies and to integrate the operations of such acquired companies may not be as successful as UCB may have believed at the moment of acquisition. Also, UCB or others could discover safety, side effects or manufacturing problems with its products and/or devices after they are marketed. The discovery of significant problems with a product similar to one of UCB's products that implicate an entire class of products may have a material adverse effect on sales of the entire class of affected products. Moreover, sales may be impacted by international and domestic trends toward managed care and health care cost containment, including pricing pressure, political and public scrutiny, customer and prescriber patterns or practices, and the reimbursement policies imposed by third-party payers as well as legislation affecting biopharmaceutical pricing and reimbursement activities and outcomes. Finally, a breakdown, cyberattack or information security breach could compromise the confidentiality, integrity and availability of UCB's data and systems.

Given these uncertainties, the public is cautioned not to place any undue reliance on such forward-looking statements. These forward-looking statements are made only as of the date of this presentation, and do not reflect any potential impacts from the evolving COVID-19 pandemic, unless indicated otherwise. The company continues to follow the development diligently to assess the financial significance of this pandemic to UCB.

UCB expressly disclaims any obligation to update any forward-looking statements in this presentation, either to confirm the actual results or to report or reflect any change in its forward-looking statements with regard thereto or any change in events, conditions or circumstances on which any such statement is based, unless such statement is required pursuant to applicable laws and regulations.

In the event of any differences between this presentation and the Annual or Half-Year Report, the information included in the Report shall prevail.

Disclaimer & Safe Harbor

Rozanolixizumab is an investigational product currently in clinical development by UCB and has not been approved by any health authorities worldwide for the treatment of any conditions.

Zilucoplan is an investigational product currently in clinical development by UCB and has not been approved by any health authorities worldwide for the treatment of any conditions.

This document therefore discusses unlicensed products and/or indications. It is not intended to advocate any indication, dosage, or other claim, or to draw any conclusions regarding the safety and efficacy of rozanolixizumab or zilucoplan.

Agenda

Antje Witte, Head of Investor Relations, UCB	Housekeeping & Introductions
Charl van Zyl, Executive Vice-President Neurology Solutions, UCB	Welcome Remarks; UCB's Ambitions in Generalized Myasthenia Gravis
Iris Loew-Friedrich, Chief Medical Officer, UCB	Delivering transformational outcomes in gMG: Learning from the community to tailor our approach
James F. Howard, MD, Distinguished Professor of Neuromuscular Disease, Professor of Neurology, Medicine and Allied Health, The University of North Carolina at Chapel Hill School of Medicine and lead investigator in the RAISE trial	Zilucoplan - RAISE Study Results
Vera Bril, MD, FRCPC, Professor of medicine & neurology, University of Toronto; and director, Neuromuscular Section, division of neurology, University of Toronto and University Health Network, Toronto, lead investigator in the MycarinG trial	Rozanolixizumab - MycarinG Study results
Antje Witte, Head of Investor Relations, UCB	Q&A

Housekeeping & Introductions

This call is intended for financial analysts and investors. If you are a media representative, please disconnect now.

We encourage you to turn on your webcam for the duration of the meeting.

You are automatically muted. If you want to ask a question, please use the “Raise hand” function or write an e-mail to **antje.witte@ucb.com**

Welcome Remarks

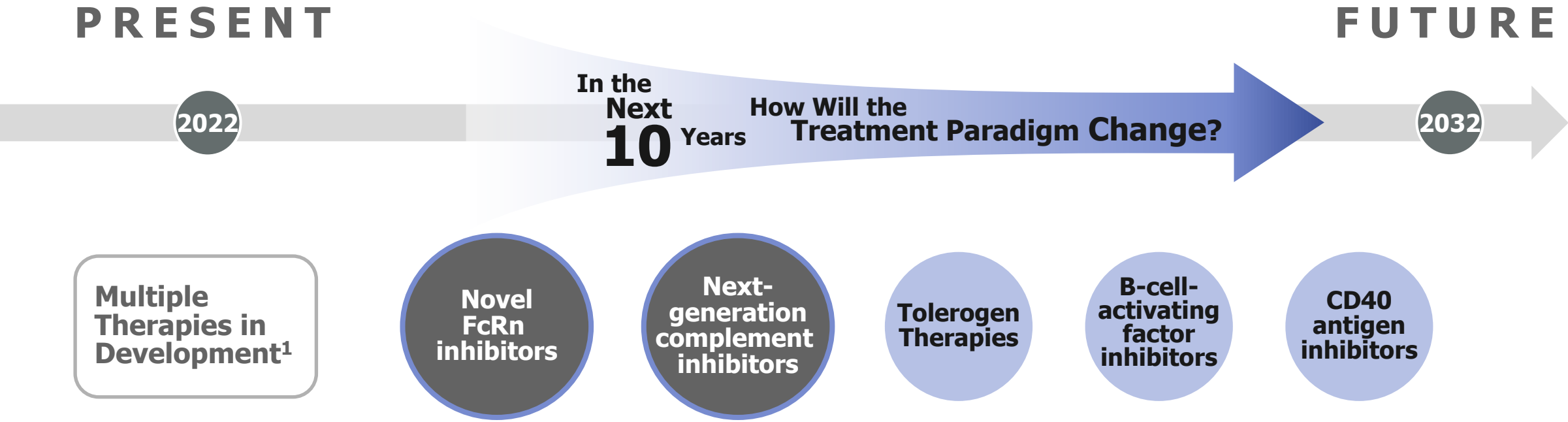
UCB's Ambitions in Generalized Myasthenia Gravis

Charl van Zyl, Executive Vice-President,
Neurology Solutions, UCB

Evolution in Myasthenia Gravis…

1. Myasthenia Gravis News. Accessed November 11, 2019. https://myastheniagravisnews.com/mestinon-pyridostigmine/. **2.** Myasthenia Gravis Foundation of America. Accessed November 18, 2019. https://myasthenia.org/Resources-Community/MG-Materials-Webinars/ Learn-More-About-MG. **3.** Kjær M. *Acta Neurol Scand*. 1971;47:464-474. **4.** Matell G, et al. *Ann NY Acad Sci*. 1976;274:659-676. **5.** Newsom-Davis J, et al. *Lancet*. 1979;1:464-468. **6.** Gajdos P, et al. *Cochrane Database Syst Rev*. 2012;12:CD002277. **7.** SOLIRIS® (eculizumab). Prescribing information. Alexion Pharmaceuticals, Inc.; 2019.

…to the next revolution in Myasthenia Gravis



1. Adis Insight. Accessed November 19, 2019. https://adisinsight.springer.com/search

Inspired by Patients, Driven by Science

Who we are: creating value for people living with severe diseases in immunology and neurology

Ambition

To transform the lives of people living with these severe diseases by putting patients at the center of our world and advancing scientific solutions to address their needs.

Unmet need in gMG: many people with gMG continue to experience the unpredictable burden of the disease impacting their ability to perform activities of daily living

We are committed to supporting the patient experience to help improve outcomes.

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Aim

To push scientific boundaries and lead efforts to develop interventions where current treatments offer little hope

- minimize its clinical manifestations
- change the treatment paradigm for gMG patients
- shape the future of gMG management

We are committed to deliver innovative treatments that support gMG patients and potentially enable them to live their best lives.

Unique portfolio comprising two mechanisms of action poised to transform the Myasthenia Gravis landscape

gMG Portfolio

Zilucoplan & Rozanolixizumab
Two **unique** targeted medicines enabled by digital innovation and support services

Current treatment options

- Many patients not well-controlled
- High level of disease and treatment burden

Dual mechanisms of action approach to address individual needs of patients

AChR+ patients		AChR+ / MuSK+ patients
Zilucoplan Complement 5 inhibitor to address complement activation	(AChR+) patient or physician preference	**Rozanolixizumab** Anti-FcRn antibody to address pathogenic auto-antibodies
Maintenance therapy	occasional acute exacerbations	Add-on treatment for exacerbations

Treatment goals:
- Fewer people experience exacerbations
- More symptom free days
- Fewer people relying on non-targeted therapies

AChR+, acetylcholinesterase receptor positive; FcRn, neonatal Fc receptor; gMG, generalized myasthenia gravis; MOA, mechanism of action, MuSK+, muscle specific kinase positive; zilucoplan and rozanolixizumab are investigational products and are not approved for any indication by any regulatory authority in the world. Zilucoplan and rozanolixizumab require additional studies before any conclusions for safety and efficacy can be made.

Delivering Transformational Outcomes in gMG

Learning from the Community to Tailor Our Approach

Iris Loew-Friedrich, Chief Medical Officer,
UCB

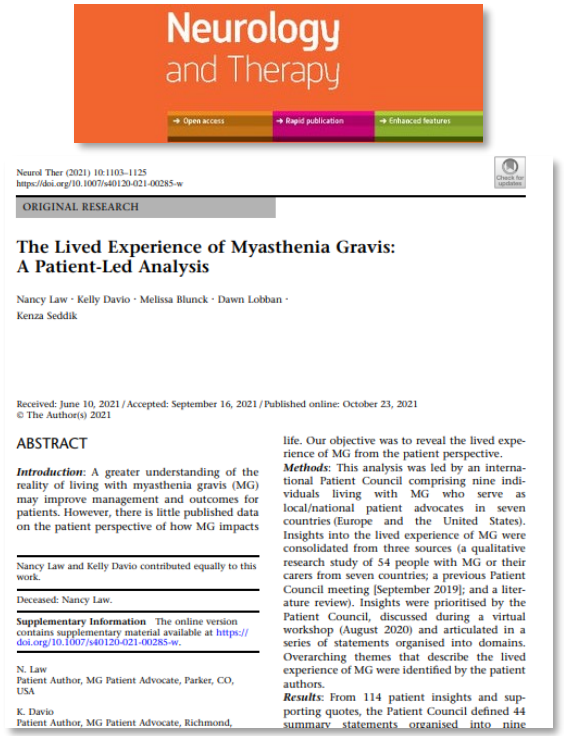
UCB: Learning from the (g)MG community

RDCN: Cross-functional medical education omnichannel initiative

Rare Disease Connect in Neurology

Launched in 2021, continuously evolving platform

The Lived Experience of Myasthenia Gravis: A Patient-Led Analysis



Published in 2021

Collaborating for the Myasthenia Gravis Community

Launched February 2022

gMG: an Uncontrolled and Debilitating Disease

70-90%

of patients **never reach complete stable remission**[1,2]

20%

of patients will experience a **potentially life-threatening myasthenic crisis**[3,4]

69%

of patients who experienced a myasthenic crisis **had no identifiable precipitating factor**[4]

~36-64%

of patients receiving treatments still had **difficulty controlling gMG symptoms** over the course of a 3-year study[2]

21-43%

of patients experienced **ER visits or hospitalizations** over the course of 6 months[3]

References on file.

gMG: an Uncontrolled and Debilitating Disease

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Physical

70% of patients with gMG have **difficulties walking**[5]
40% of patients have **difficulty chewing and swallowing**[5]

Personal

42% of patients with gMG experience **depression**[6] (vs. 16% of the general population)

Professional

27% of patients experience **unemployment**[7]
36% of patients experience **decrease in income**[7]

References on file.

New Therapeutic Targets and Treatments are Needed

UNMET NEED: treatment options to **reduce symptom worsening** and **improve the quality of life**[1]

Minimise the impact of the disease[1]

Need for **more-effective** and **targeted** treatments[1]

Reduce the burden of treatment and the burden of care[1]

Need for **minimally invasive, convenient, rapid-acting,** and **well-tolerated** treatments[1]

Each patient is unique, and a one-size-fits-all approach is not appropriate.

To reduce the burden of gMG, HCPs will require options that they can tailor to their specific patients' needs, as well as patient support services and technologies to monitor responses and treatment outcomes.

IgG levels and complement damage are not correlated.[2]

1. Guptill JT, et al. Neurotherapeutics. 2016;13:118-131.
2. Fitchner ML, et al. PLoS ONE 17(3): e0264489.

Lead investigators for our 2 pivotal gMG studies

Professor James F. Howard

James F. Howard, MD, Distinguished Professor of Neuromuscular Disease, Professor of Neurology, Medicine and Allied Health, The University of North Carolina at Chapel Hill School of Medicine and **lead investigator in the RAISE trial**

Professor Vera Bril

MD, FRCPC, Professor of medicine & neurology, University of Toronto; and director, Neuromuscular Section, division of neurology, University of Toronto and University Health Network, Toronto**, lead investigator in the MycarinG trial**

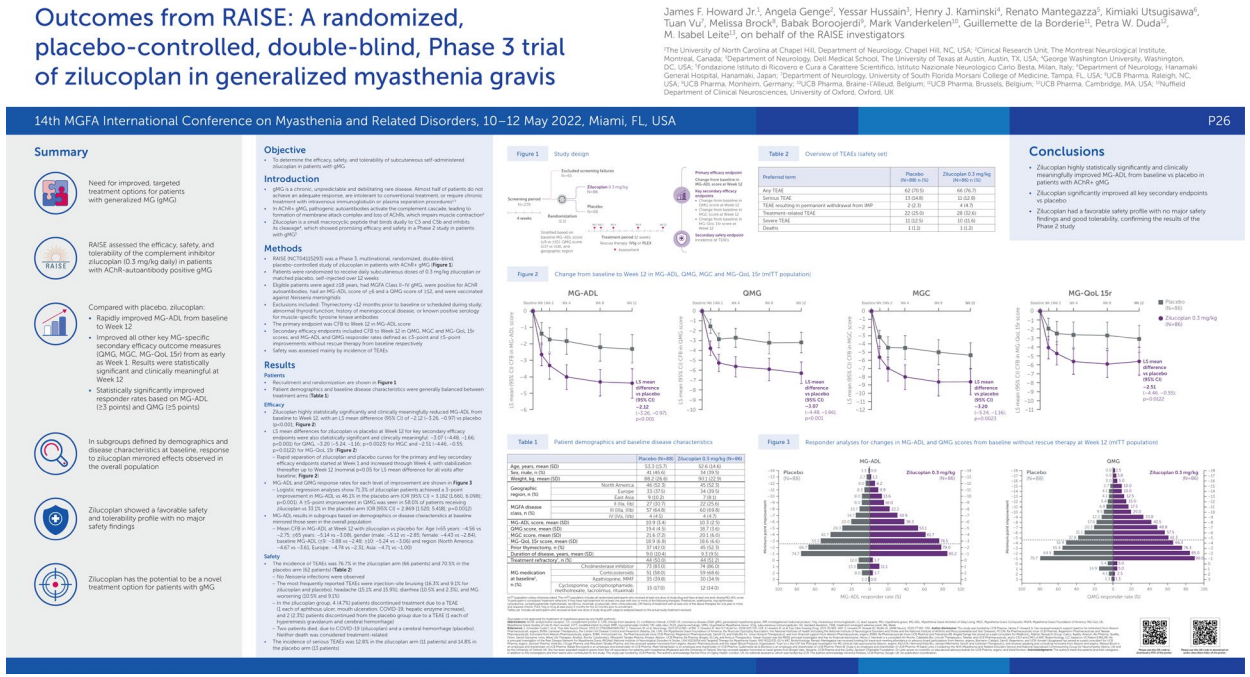
Efficacy and safety of zilucoplan in patients with myasthenia gravis: RAISE Phase 3 study results

Professor James F. Howard Jr

Lead investigator

MGFA Presentation

Howard et al. Outcomes from RAISE: A randomized, placebo-controlled, double-blind, Phase 3 trial of zilucoplan in generalized myasthenia gravis. Poster 26 presented at 14th MGFA International Conference On Myasthenia And Related Disorders



Outcomes from RAISE: A randomized, placebo-controlled, double-blind, Phase 3 trial of zilucoplan in generalized myasthenia gravis

James F. Howard Jr.[1], Angela Genge[2], Yessar Hussain[3], Henry J. Kaminski[4], Renato Mantegazza[5], Kimiaki Utsugisawa[6], Tuan Vu[7], Melissa Brock[8], Babak Borojerdi[9], Mark Vanderkelen[10], Guillemette de la Borderie[11], Petra W. Duda[12], M. Isabel Leite[13], on behalf of the RAISE investigators

[1]The University of North Carolina at Chapel Hill, Department of Neurology, Chapel Hill, NC, USA; [2]Clinical Research Unit, The Montreal Neurological Institute, Montreal, Canada; [3]Department of Neurology, Dell Medical School, The University of Texas at Austin, Austin, TX, USA; [4]George Washington University, Washington, DC, USA; [5]Fondazione Istituto di Ricovero e Cura a Carattere Scientifico, Istituto Nazionale Neurologico Carlo Besta, Milan, Italy; [6]Department of Neurology, Hanamaki General Hospital, Hanamaki, Japan; [7]Department of Neurology, University of South Florida Morsani College of Medicine, Tampa, FL, USA; [8]UCB Pharma, Raleigh, NC, USA; [9]UCB Pharma, Monheim, Germany; [10]UCB Pharma, Braine-l'Alleud, Belgium; [11]UCB Pharma, Brussels, Belgium; [12]UCB Pharma, Cambridge, MA, USA; [13]Nuffield Department of Clinical Neurosciences, University of Oxford, Oxford, UK

14th MGFA International Conference on Myasthenia and Related Disorders, 10–12 May 2022, Miami, FL, USA P26

Summary

Need for improved, targeted treatment options for patients with generalized MG (gMG)

RAISE assessed the efficacy, safety, and tolerability of the complement inhibitor zilucoplan (0.3 mg/kg daily) in patients with AChR-autoantibody positive gMG

Compared with placebo, zilucoplan:
- Rapidly improved MG-ADL from baseline to Week 12
- Improved all other key MG-specific secondary efficacy outcome measures (QMG, MGC, MG–QoL 15r) from as early as Week 1. Results were statistically significant and clinically meaningful at Week 12
- Statistically significantly improved responder rates based on MG-ADL (≥3 points) and QMG (≥5 points)

In subgroups defined by demographics and disease characteristics at baseline, response to zilucoplan mirrored effects observed in the overall population

Zilucoplan showed a favorable safety and tolerability profile with no major safety findings

Zilucoplan has the potential to be a novel treatment option for patients with gMG

Objective
- To determine the efficacy, safety, and tolerability of subcutaneous self-administered zilucoplan in patients with gMG

Introduction
- gMG is a chronic, unpredictable and debilitating rare disease. Almost half of patients do not achieve an adequate response, are intolerant to conventional treatment, or require chronic treatment with intravenous immunoglobulin or plasma separation procedures[1]
- In AChR+ gMG, pathogenic autoantibodies activate the complement cascade, leading to formation of membrane attack complex and loss of AChRs, which impairs muscle contraction[2]
- Zilucoplan is a small macrocyclic peptide that binds dually to C5 and C5b and inhibits terminal C5b-9, which showed promising efficacy and safety in a Phase 2 study in patients with gMG[3]

Methods
- RAISE (NCT04115293) was a Phase 3, multinational, randomized, double-blind, placebo-controlled study of zilucoplan in patients with AChR+ gMG (**Figure 1**)
- Patients were randomized to receive daily subcutaneous doses of 0.3 mg/kg zilucoplan or matched placebo, self-injected over 12 weeks
- Key eligibility criteria were age ≥18 years, had MGFA Class II–IV gMG, were positive for AChR autoantibodies, had an MG-ADL score of ≥6 and a QMG score of ≥12, and were vaccinated against Neisseria meningitidis
- Exclusions included: Thymectomy <12 months prior to baseline or scheduled during study; abnormal thyroid function; history of meningococcal disease; or known positive serology for muscle-specific tyrosine kinase antibodies
- The primary endpoint was CFB to Week 12 in MG-ADL score
- Secondary efficacy endpoints included CFB to Week 12 in QMG, MGC and MG-QoL 15r scores, and MG-ADL and QMG responder rates defined as ≥3-point and ≥5-point improvements without rescue therapy from baseline respectively
- Safety was assessed mainly by incidence of TEAEs

Results

Patients
- Recruitment and randomization are shown in **Figure 1**
- Patient demographics and baseline disease characteristics were generally balanced between treatment arms (**Table 1**)

Efficacy
- Zilucoplan highly statistically significantly and clinically meaningfully reduced MG-ADL from baseline to Week 12, with an LS mean difference (95% CI) of −2.12 (−3.26, −0.97) vs placebo (p=0.0003; **Figure 2**)
- LS mean differences for zilucoplan vs placebo at Week 12 for key secondary efficacy endpoints were also statistically significant and clinically meaningful: −3.07 (−4.48, −1.66; p<0.0001) for QMG, −3.20 (−5.24, −1.16; p=0.0023) for MGC and −2.51 (−4.46, −0.55; p=0.0122) for MG–QoL 15r (**Figure 2**)
  - Rapid separation of zilucoplan and placebo curves for the primary and key secondary efficacy endpoints started at Week 1 and increased through Week 4, with stabilization thereafter up to Week 12 (nominal p<0.05 for LS mean difference for all visits after baseline; **Figure 2**)
- MG-ADL and QMG response rates for each level of improvement are shown in **Figure 3**
  - Logistic regression analyses show 71.1% of zilucoplan patients achieved a 3-point improvement in MG-ADL vs 46.1% in the placebo arm (OR [95% CI] = 3.02 [1.640, 6.098]; p<0.001). A ≥5-point improvement in QMG was seen in 58.0% of patients receiving zilucoplan vs 34.9% in the placebo arm (OR [95% CI] = 2.969 [1.520, 5.438]; p=0.0012)
- MG-ADL results in subgroups based on demographics or disease characteristics at baseline mirrored those seen in the overall population
  - Mean CFB in MG-ADL at Week 12 with zilucoplan vs placebo for: Age (<65 years: −4.56 vs −2.75; ≥65 years: −5.14 vs −3.08); gender (male: −5.12 vs −2.85; female: −4.43 vs −2.84); baseline MG-ADL (≤9: −3.88 vs −2.48; ≥10: −5.24 vs −3.00) and region (North America: −4.67 vs −3.61; Europe: −4.74 vs −2.31; Asia: −4.71 vs −1.00)

Safety
- The incidence of TEAEs was 76.7% in the zilucoplan arm (66 patients) and 70.5% in the placebo arm (62 patients) (**Table 2**)
  - No Neisseria infections were observed
  - The most frequently reported TEAEs were injection-site bruising (16.3% and 9.1% for zilucoplan and placebo), headache (15.1% and 15.9%), diarrhea (10.5% and 2.3%), and MG worsening (10.5% and 9.1%)
  - In the zilucoplan group, 4 (4.7%) patients discontinued treatment due to a TEAE (1 each of aphthous ulcer, mouth ulceration, COVID-19, hepatic enzyme increased, and 2 (2.3%) patients discontinued from the placebo group due to a TEAE (1 each of hypertensive granulomas and cerebral hemorrhage)
  - Two patients died, due to COVID-19 (zilucoplan) and a cerebral hemorrhage (placebo). Neither death was considered treatment-related
- The incidence of serious TEAEs was 12.8% in the zilucoplan arm (11 patients) and 14.8% in the placebo arm (13 patients)

Conclusions
- Zilucoplan highly statistically significantly and clinically meaningfully improved MG-ADL from baseline vs placebo in patients with AChR+ gMG
- Zilucoplan significantly improved all key secondary endpoints vs placebo
- Zilucoplan had a favorable safety profile with no major safety findings and good tolerability, confirming the results of the Phase 2 study

Inspired by patients. Driven by science.

Zilucoplan is an investigational new product and has not been approved by any authority.

RAISE background and study objective

- gMG is a chronic, unpredictable and debilitating rare disease. Almost half of patients do not achieve an adequate response, are intolerant to conventional treatment, or require chronic treatment with intravenous immunoglobulin or plasma separation procedures[1,2]
- In AChR+ gMG, pathogenic autoantibodies activate the complement cascade, leading to formation of membrane attack complex and loss of AChRs, which impair muscle contraction[3]
- Zilucoplan is a small macrocyclic peptide that binds dually to C5 and C5b and inhibits its cleavage[4], which showed promising efficacy and safety in a Phase 2 study in patients with gMG[5]

Objective

To determine the efficacy, safety, and tolerability of subcutaneous self-administered zilucoplan in patients with gMG

Howard et al. MGFA 2022, Poster 26.
AChR, acetylcholine receptor; gMG, generalized myasthenia gravis.
1. Schneider-Gold C, et al. Ther Adv Neurol Disord. 2019;12:1756286419832242. 2. Petersson M, et al. Neurology. 2021;97:e1382–e1391. 3. Howard JF. Ann N Y Acad Sci. 2018;1412:113–128.
4. Howard JF, et al. Exp Opin Investig Drugs. 2021;30:483–493. 5. Howard JF, et al. JAMA Neurol. 2020;77:582–592.

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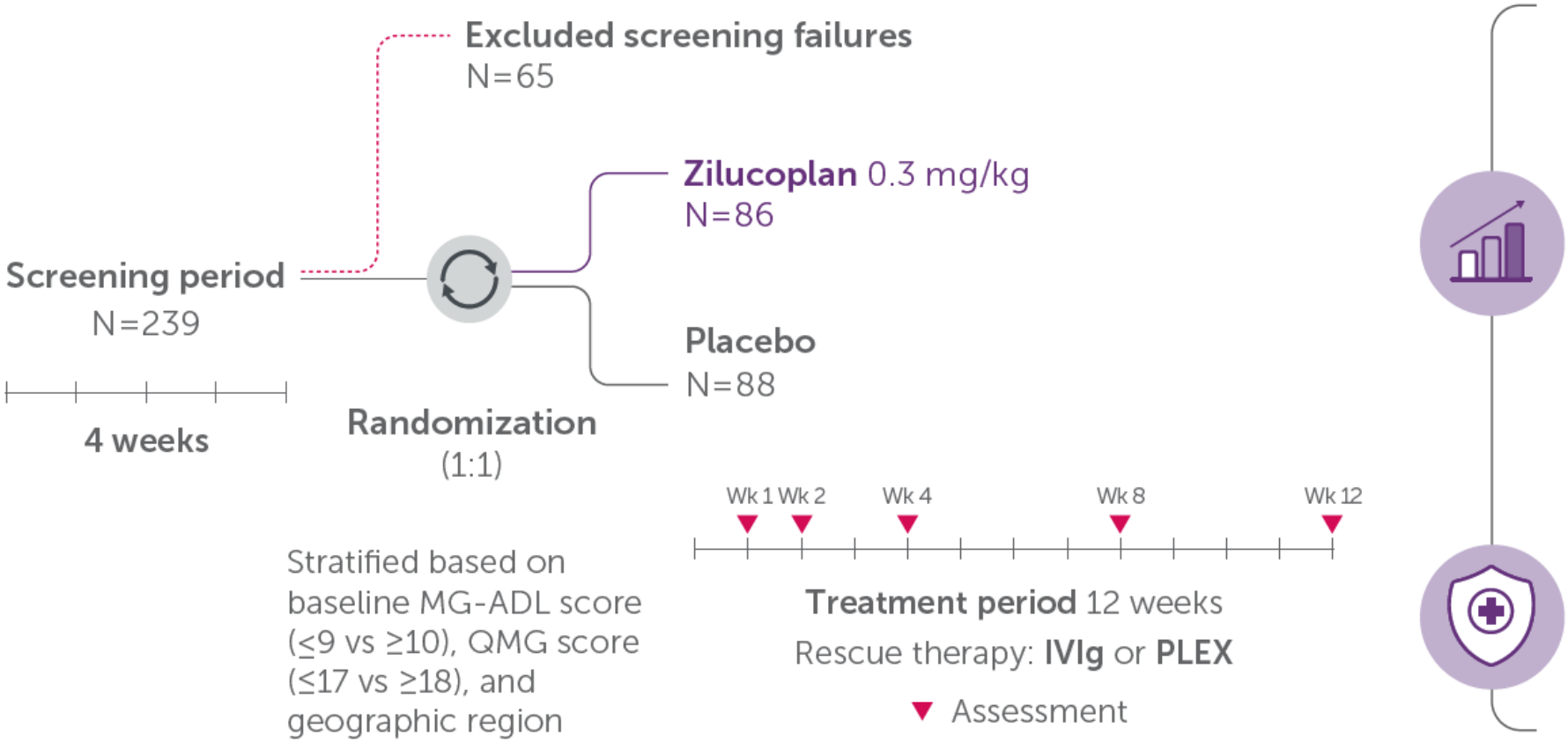
RAISE study design and endpoints



Primary efficacy endpoint

Change from baseline in MG-ADL score at Week 12

Key secondary efficacy endpoints

- Change from baseline in QMG score at Week 12
- Change from baseline in MGC score at Week 12
- Change from baseline in MG-QoL 15r score at Week 12

Secondary safety endpoint

Incidence of TEAEs

Howard et al. MGFA 2022, Poster 26.
AE, adverse event; IVIg, intravenous immunoglobulin; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGC, Myasthenia Gravis Composite; MG-QoL 15r, Revised 15-item Myasthenia Gravis Quality of Life score; PLEX, plasma exchange; QMG, Quantitative Myasthenia Gravis.

Zilucoplan is an investigational new product and has not been approved by any authority.

Key inclusion and exclusion criteria

Inclusion criteria

- ≥18 years
- MGFA Class II–IV gMG
- Positive for AChR autoantibodies
- MG-ADL score of ≥6
- QMG score of ≥12
- Vaccination against *Neisseria meningitidis*

Exclusion criteria

- Thymectomy <12 months prior to baseline or scheduled during study
- Abnormal thyroid function
- History of meningococcal disease
- Known positive serology for muscle-specific tyrosine kinase antibodies

Adapted from Howard et al. MGFA 2022, Poster 26.
AChR, acetylcholine receptor; gMG, generalized myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGFA, Myasthenia Gravis Foundation of America; QMG, Quantitative Myasthenia Gravis.

Patient demographics (mITT population)

Patient demographics and baseline demographics were generally balanced between treatment arms

		Placebo (n=88)	Zilucoplan 0.3 mg/kg (n=86)
	Age, years, mean (SD)	53.3 (15.7)	52.6 (14.6)
	Sex, male, n (%)	41 (46.6)	34 (39.5)
	Weight, kg, mean (SD)	88.2 (26.6)	90.1 (22.9)
Geographic region, n (%)	North America	46 (52.3)	45 (52.3)
	Europe	33 (37.5)	34 (39.5)
	East Asia	9 (10.2)	7 (8.1)
MGFA disease class, n (%)	II (IIa, IIb)	27 (30.7)	22 (25.6)
	III (IIIa, IIIb)	57 (64.8)	60 (69.8)
	IV (IVa, IVb)	4 (4.5)	4 (4.7)

Adapted from Howard et al. MGFA 2022, Poster 26.
mITT population includes all randomized participants who received at least one dose of study drug and have at least one post-dosing MG-ADL score.
mITT, modified intent-to-treat; MG, myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGFA, Myasthenia Gravis Foundation of America; SD, standard deviation.

Patient demographics (mITT population)

Patient demographics and baseline demographics were generally balanced between treatment arms

		Placebo (n=88)	Zilucoplan 0.3 mg/kg (n=86)
MG-ADL, mean (SD)		10.9 (3.4)	10.3 (2.5)
QMG score, mean (SD)		19.4 (4.5)	18.7 (3.6)
MGC score, mean (SD)		21.6 (7.2)	20.1 (6.0)
MG-QoL 15r score, mean (SD)		18.9 (6.8)	18.6 (6.6)
Prior thymectomy, n (%)		37 (42.0)	45 (52.3)
Duration of disease, years, mean (SD)		9.0 (10.4)	9.3 (9.5)
Treatment refractory*, n (%)		44 (50.0)	44 (51.2)
MG medication at baseline†, n (%)	Cholinesterase inhibitor	73 (83.0)	74 (86.0)
	Corticosteroids	51 (58.0)	59 (68.6)
	Azathioprine, MMF	35 (39.8)	30 (34.9)
	Cyclosporine, cyclophosphamide, methotrexate, tacrolimus, rituximab	15 (17.0)	12 (14.0)

Adapted from Howard et al. MGFA 2022, Poster 26.
mITT population unless otherwise stated. mITT population includes all randomized participants who received at least one dose of study drug and have at least one post-dosing MG-ADL score.
*A participant is considered 'treatment refractory' if they have had treatment for at least one year with two or more of the following therapies: Prednisone, azathioprine, mycophenolate, cyclosporine, cyclophosphamide, methotrexate, tacrolimus, rituximab, eculizumab, other corticosteroids; OR history of treatment with at least one of the above therapies for one year or more and required chronic PLEX, IVIg or SCIg at least every 3 months for the 12 months prior to enrolment. †Safety set. Includes all participants who received at least one dose of study drug with participants analysed based on the actual study treatment received.
IVIg, intravenous immunoglobulin; mITT, modified intent-to-treat; MG, myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGC, Myasthenia Gravis Composite; MG-QoL 15r, Revised 15-item Myasthenia Gravis Quality of Life score; MMF, mycophenolate mofetil; PLEX, plasma exchange; QMG, Quantitative Myasthenia Gravis; SCIg, subcutaneous immunoglobulin; SD, standard deviation.

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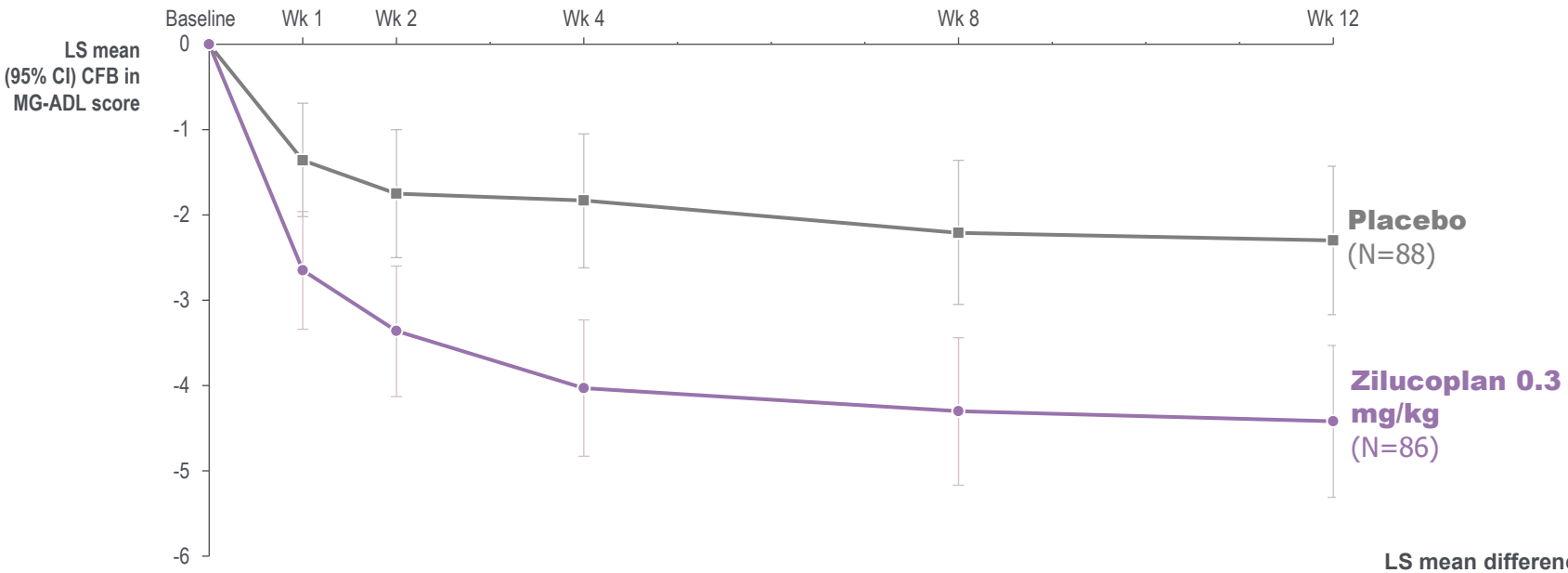
MG-ADL CFB to Week 12 (mITT population)

- Zilucoplan highly statistically significantly and clinically meaningfully reduced MG-ADL from baseline to Week 12 (p<0.001)
- Rapid separation of zilucoplan and placebo curves started at Week 1 and increased through Week 4, with stabilization thereafter up to Week 12 (nominal p<0.05 for LS mean difference for all visits after baseline)

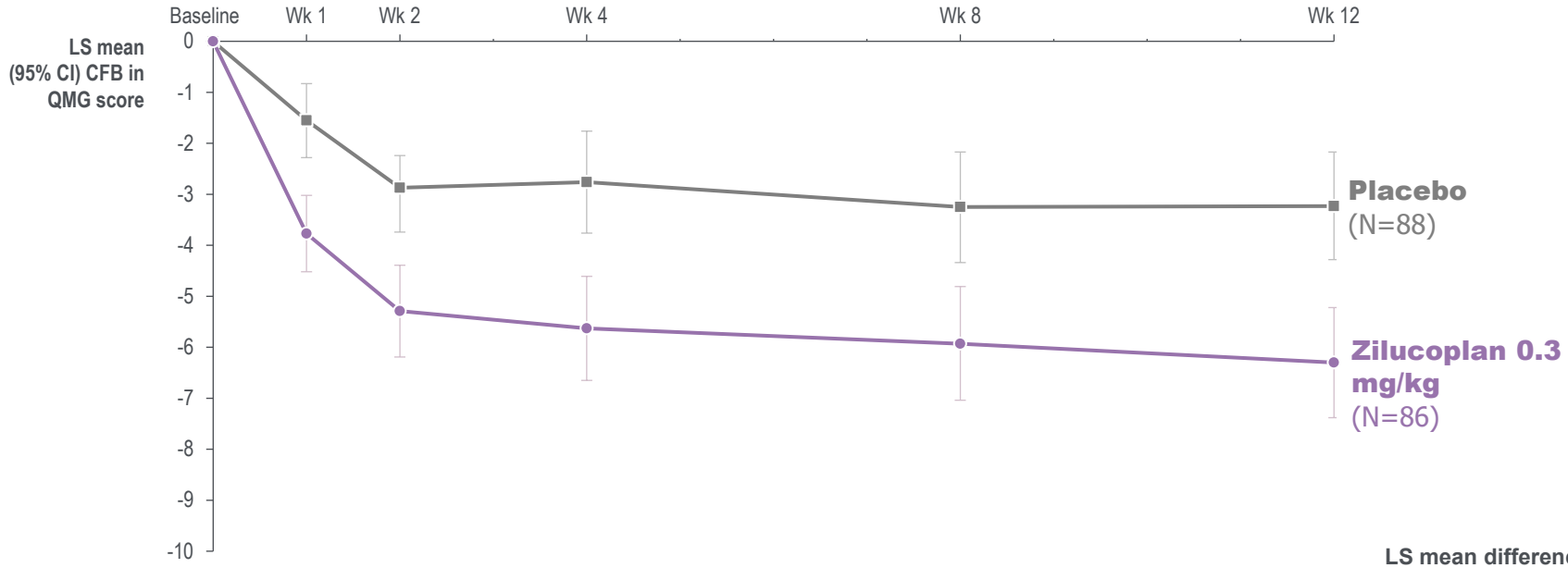


LS mean difference vs placebo (95% CI)	
Zilucoplan 0.3 mg/kg	**−2.12** (−3.26, −0.97)

Adapted from Howard et al. MGFA 2022, Poster 26.
mITT population includes all randomized participants who received at least one dose of study drug and have at least one post-dosing MG-ADL score. A 2-point change in MG-ADL score is considered clinically meaningful.[1]
CFB, change from baseline; CI, confidence interval; LS, least squares; MG-ADL, Myasthenia Gravis Activities of Daily Living; mITT, modified intent-to-treat.
1. Muppidi S, et al. Muscle Nerve. 2011;44:727–731.

QMG CFB to Week 12 (mITT population)

- LS mean difference for zilucoplan vs placebo at Week 12 for QMG was also highly statistically significant and clinically meaningful ($p<0.001$)
- Rapid separation of zilucoplan and placebo curves started at Week 1 and increased through Week 4, with stabilization thereafter up to Week 12 (nominal $p<0.05$ for LS mean difference for all visits after baseline)



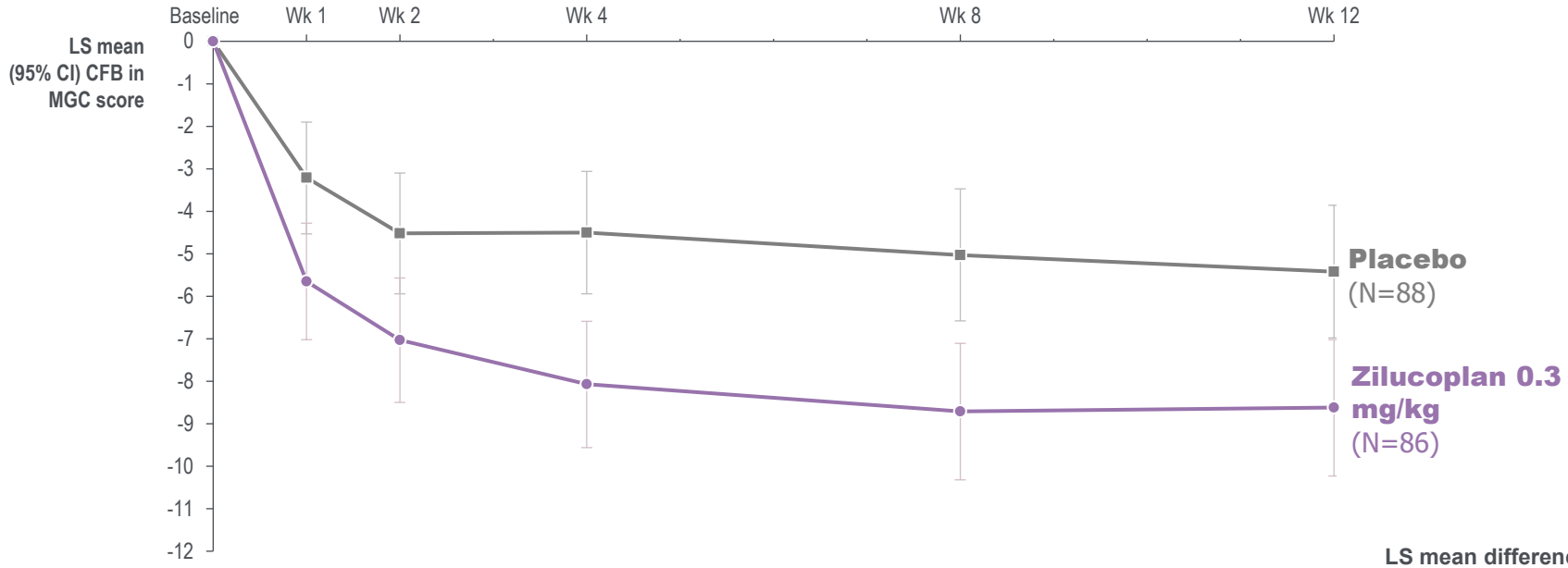
LS mean difference vs placebo (95% CI)	
Zilucoplan 0.3 mg/kg	**−3.07** (−4.48, −1.66)

Adapted from Howard et al. MGFA 2022, Poster 26.
mITT population includes all randomized participants who received at least one dose of study drug and have at least one post-dosing MG-ADL score. A 3-point change in QMG is considered clinically meaningful.[1]
CFB, change from baseline; CI, confidence interval; LS, least squares; MG-ADL, Myasthenia Gravis Activities of Daily Living; mITT, modified intent-to-treat; QMG, quantitative myasthenia gravis.

1. Katzberg HD, et al. Muscle Nerve. 2014;49:661–665.

MGC CFB to Week 12 (mITT population)

- LS mean difference for zilucoplan vs placebo at Week 12 for MGC was statistically significant and clinically meaningful (p=0.0023)
- Rapid separation of zilucoplan and placebo curves started at Week 1 and increased through Week 4, with stabilization thereafter up to Week 12 (nominal p<0.05 for LS mean difference for all visits after baseline)

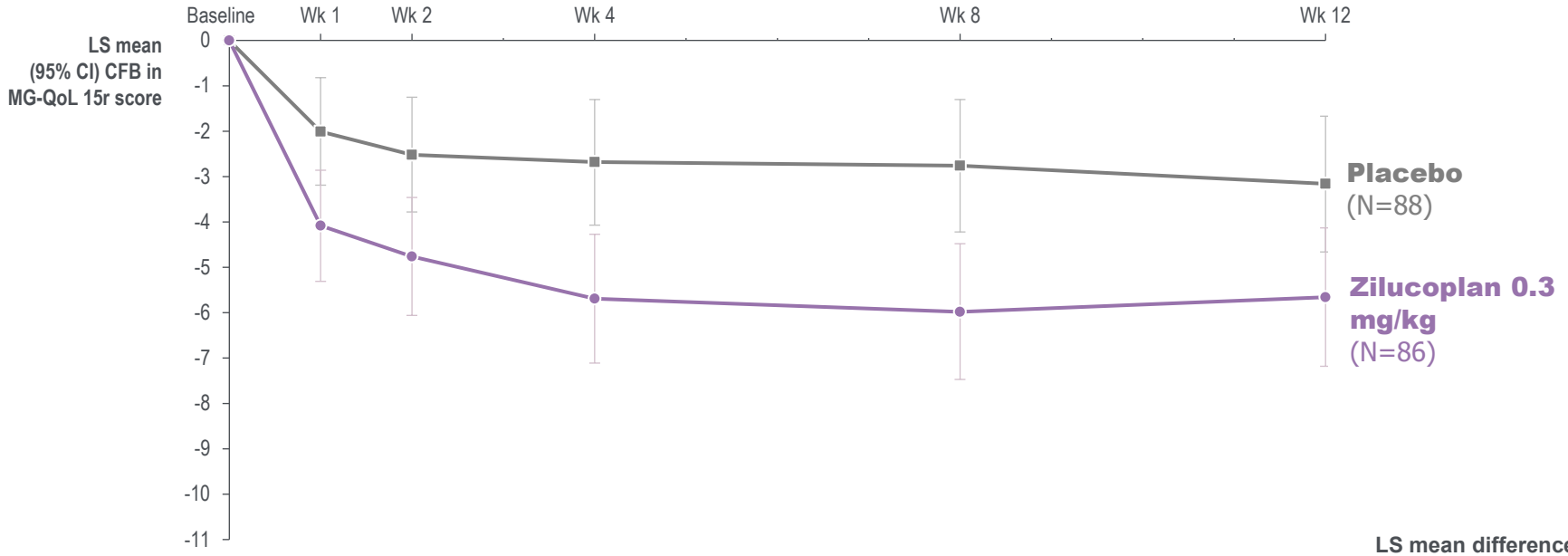


LS mean difference vs placebo (95% CI)	
Zilucoplan 0.3 mg/kg	**−3.20** (–5.24, −1.16)

Adapted from Howard et al. MGFA 2022, Poster 26.
mITT population includes all randomized participants who received at least one dose of study drug and have at least one post-dosing MG-ADL score. A 3-point change in MGC is considered clinically meaningful.[1]
CFB, change from baseline; CI, confidence interval; LS, least squares; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGC, Myasthenia Gravis Composite; mITT, modified intent-to-treat.
1. Benetar M, et al. Muscle Nerve. 2012;45:909–917.

MG-QoL 15r CFB to Week 12 (mITT population)

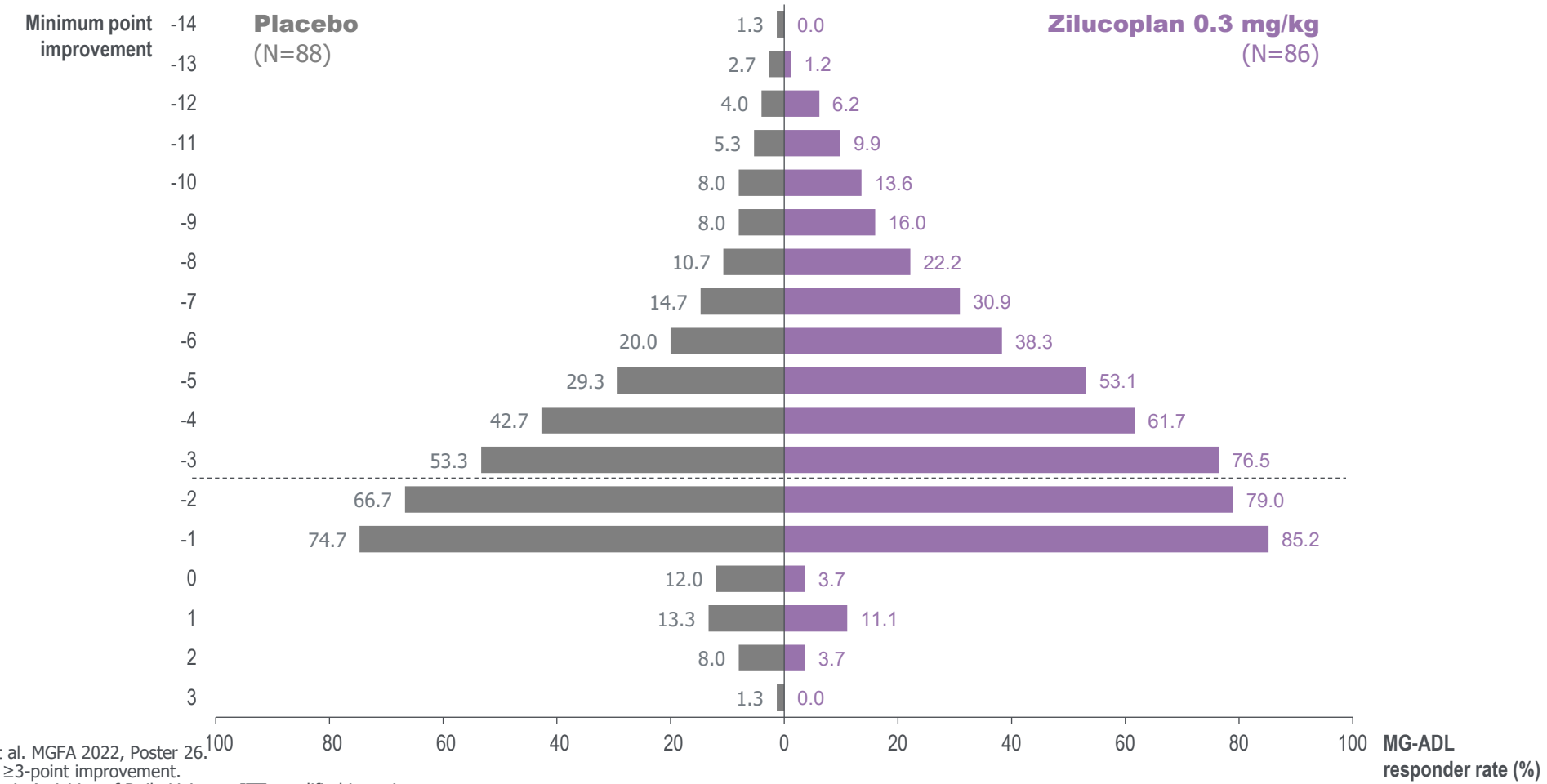
- LS mean difference for zilucoplan vs placebo at Week 12 for MG-QoL 15r was statistically significant and clinically meaningful (p=0.0122)
- Rapid separation of zilucoplan and placebo curves started at Week 1 and increased through Week 4, with stabilization thereafter up to Week 12 (nominal p<0.05 for LS mean difference for all visits after baseline)



LS mean difference vs placebo (95% CI)	
Zilucoplan 0.3 mg/kg	**−2.51** (–4.46, –0.55)

Adapted from Howard et al. MGFA 2022, Poster 26.
mITT population includes all randomized participants who received at least one dose of study drug and have at least one post-dosing MG-ADL score.
CFB, change from baseline; CI, confidence interval; LS, least squares; MG-ADL, Myasthenia Gravis Activities of Daily Living; MG-QoL 15r, Myasthenia Gravis Quality of Life 15-item revised scale; mITT, modified intent-to-treat.

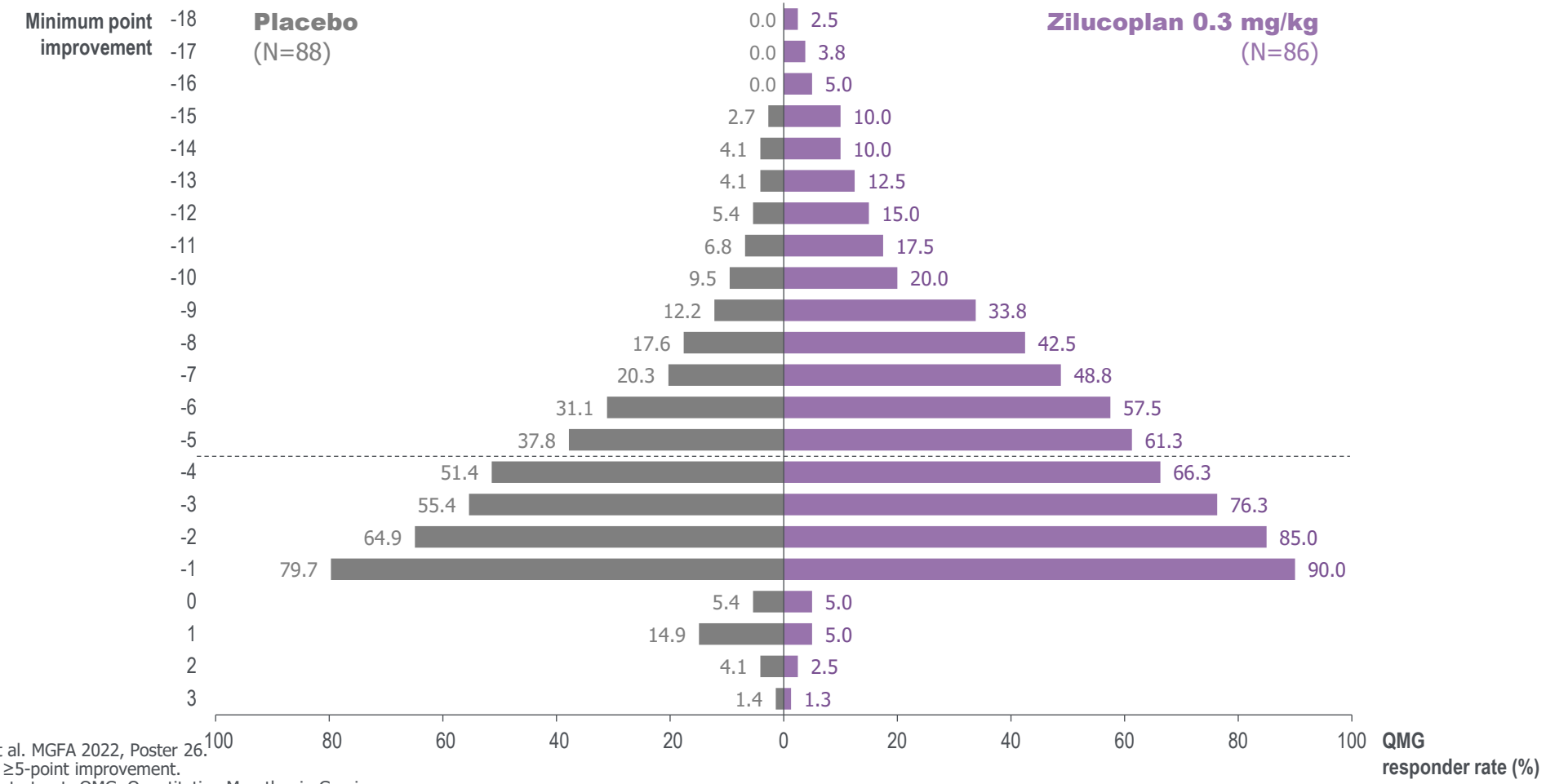
Responder analyses for changes in MG-ADL scores from baseline without rescue therapy (mITT population)



Adapted from Howard et al. MGFA 2022, Poster 26.
Responder is defined as ≥3-point improvement.
MG-ADL, Myasthenia Gravis Activities of Daily Living; mITT, modified intention-to-treat.

Zilucoplan is an investigational new product and has not been approved by any authority.

Responder analyses for changes in QMG scores from baseline without rescue therapy (mITT population)

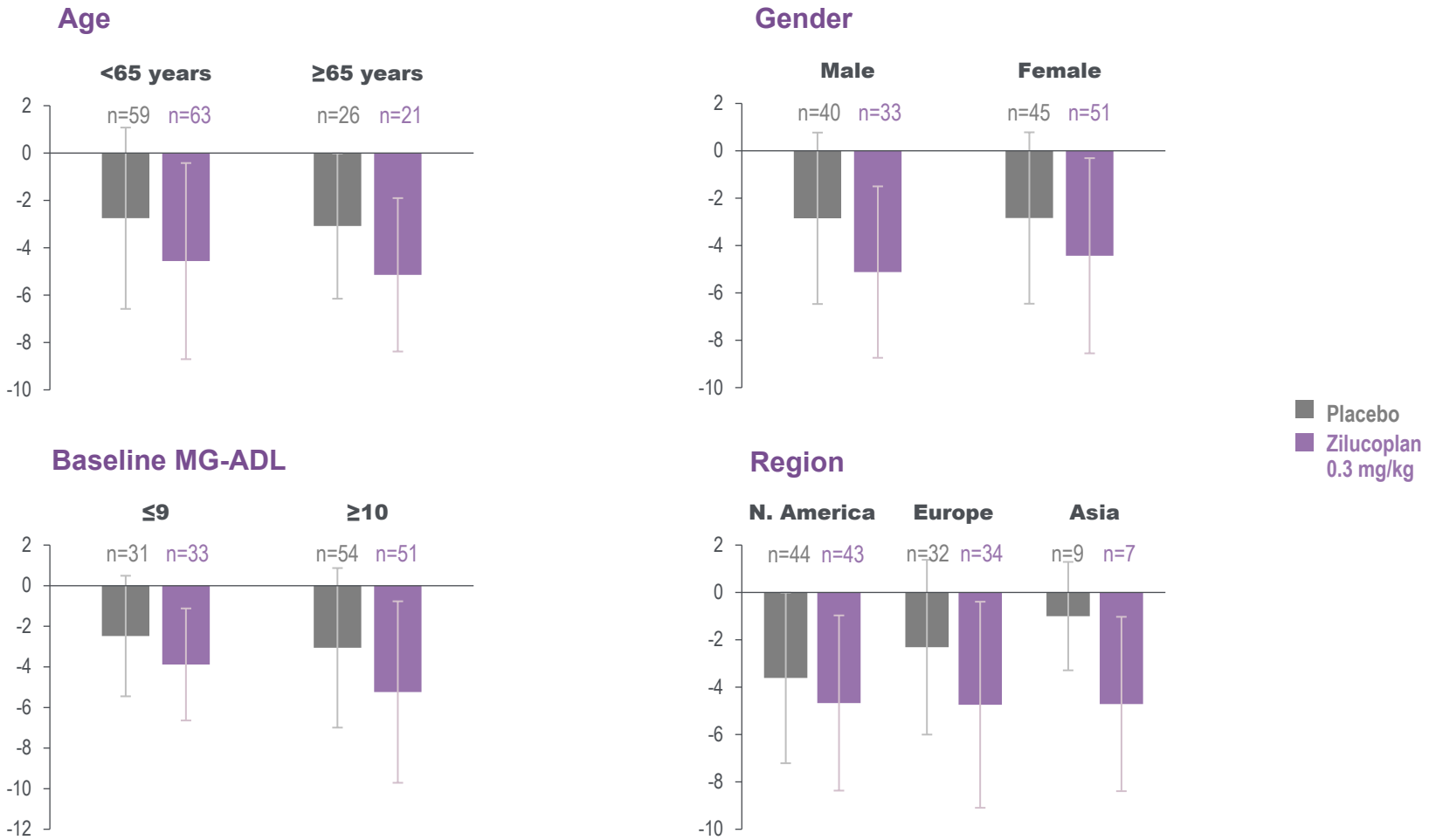


Adapted from Howard et al. MGFA 2022, Poster 26.
Responder is defined as ≥5-point improvement.
mITT, modified intention-to-treat; QMG, Quantitative Myasthenia Gravis.

Zilucoplan is an investigational new product and has not been approved by any authority.

Mean change from baseline in MG-ADL score at Week 12 by baseline subgroup

- MG-ADL results in subgroups based on demographics or disease characteristics at baseline mirrored those seen in the overall population



Adapted from Howard et al. MGFA 2022, Poster 26. UCB Data on File. 2022 MG0010 Table 14.2.1.4 p51–52.
Error bars represent standard deviation.
MG-ADL, Myasthenia Gravis Activities of Daily Living; MGFA, Myasthenia Gravis Foundation of America

Zilucoplan is an investigational new product and has not been approved by any authority.

Overview of TEAEs

Preferred term	**Placebo** (N=88) % (n)	**Zilucoplan 0.3 mg/kg** (N=86) % (n)
Any TEAE	**70.5** (62)	**76.7** (66)
Serious TEAE	**14.8** (13)	**12.8** (11)
TEAE resulting in permanent withdrawal from IMP	**2.3** (2)	**4.7** (4)
Treatment-related TEAE	**25.0** (22)	**32.6** (28)
Severe TEAE	**12.5** (11)	**11.6** (10)
Death	**1.1** (1)	**1.2** (1)

- The most common TEAEs were:
  - Injection-site bruising (16.3% for zilucoplan, 9.1% for placebo)
  - Headache (15.1% for zilucoplan, 15.9% for placebo)
  - Diarrhea (10.5% for zilucoplan, 2.3% for placebo)
  - MG worsening (10.5% for zilucoplan, 9.1% placebo)

Adapted from Howard et al. MGFA 2022, Poster 26.
Safety set includes all participants who received at least one dose of study drug with participants analyzed based on the actual study treatment received.
IMP, investigational medicinal product; TEAE, treatment-emergent adverse event.

Conclusions

- Zilucoplan **highly statistically significantly and clinically meaningfully improved MG-ADL from baseline** vs placebo in patients with AChR+ gMG
- Zilucoplan **significantly improved all key secondary endpoints** vs placebo
- Zilucoplan had a **favorable safety profile** with **no major safety findings and good tolerability,** confirming the results of the Phase 2 study
    - These data suggest that zilucoplan has the potential to be a **new option** for the management of gMG

Howard et al. MGFA 2022, Poster 26.
Zilucoplan is an investigational new product and has not been approved by any authority.
AChR, acetylcholine receptor; gMG, generalized myasthenia gravis.

Zilucoplan is an investigational new product and has not been approved by any authority.

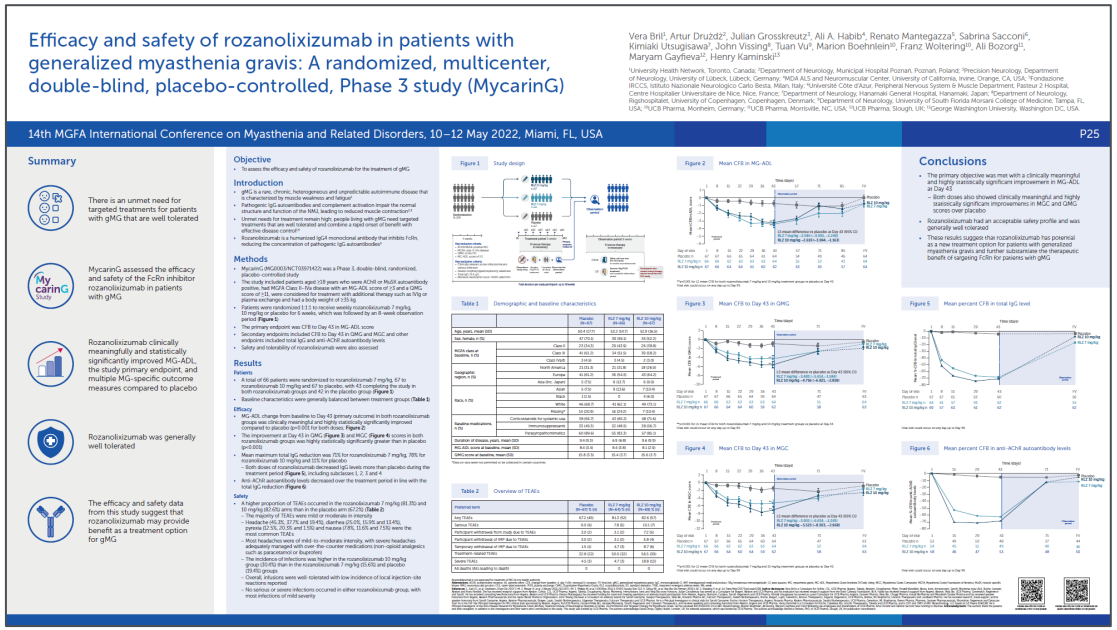
Efficacy and safety of rozanolixizumab in patients with myasthenia gravis: MycarinG Phase 3 study results

Professor Vera Bril

Lead Investigator

MGFA Presentations

1. Bril et al. Efficacy and safety of rozanolixizumab in patients with generalized myasthenia gravis: A randomized, multicenter, double-blind, placebo-controlled, Phase 3 study (MycarinG). Poster 25



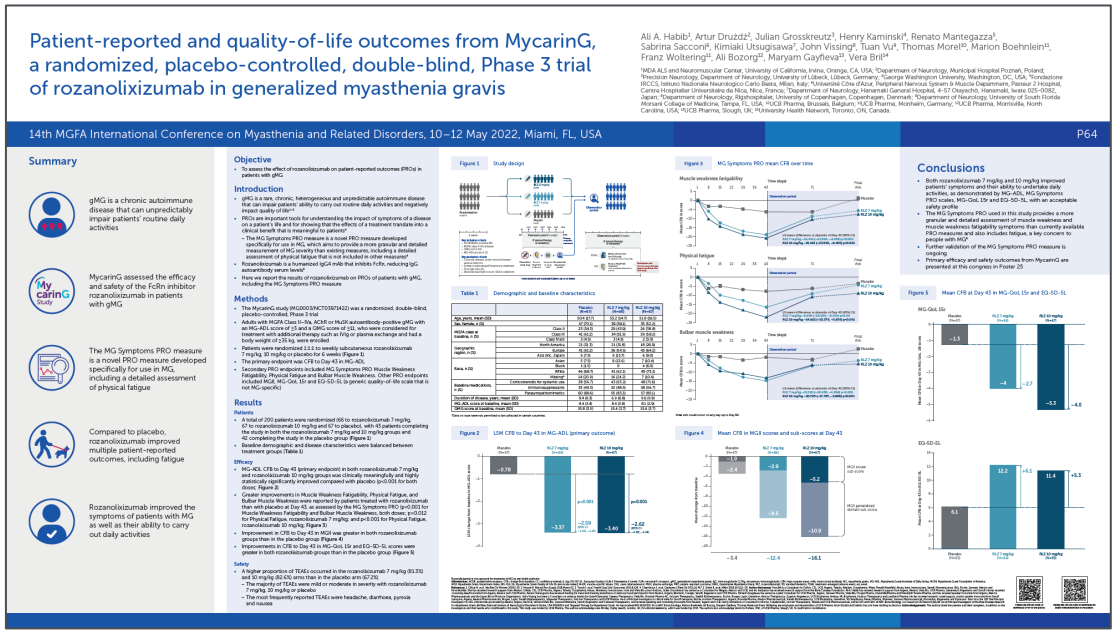
Efficacy and safety of rozanolixizumab in patients with generalized myasthenia gravis: A randomized, multicenter, double-blind, placebo-controlled, Phase 3 study (MycarinG)

Vera Bril[1], Artur Druzdz[2], Julian Grosskreutz[3], Ali A. Habib[4], Renato Mantegazza[5], Sabrina Sacconi[6], Kimiaki Utsugisawa[7], John Vissing[8], Tuan Vu[9], Marion Boehnlein[10], Franz Woltering[10], Ali Bozorg[11], Maryam Gayfieva[12], Henry Kaminski[13]

[1]University Health Network, Toronto, Canada; [2]Department of Neurology, Municipal Hospital Poznań, Poznań, Poland; [3]Precision Neurology, Department of Neurology, University of Lübeck, Lübeck, Germany; [4]MDA ALS and Neuromuscular Center, University of California, Irvine, Orange, CA, USA; [5]Fondazione IRCCS, Istituto Nazionale Neurologico Carlo Besta, Milan, Italy; [6]Université Côte d'Azur, Peripheral Nervous System & Muscle Department, Pasteur 2 Hospital, Centre Hospitalier Universitaire de Nice, Nice, France; [7]Department of Neurology, Hanamaki General Hospital, Hanamaki, Japan; [8]Department of Neurology, Rigshospitalet, University of Copenhagen, Copenhagen, Denmark; [9]Department of Neurology, University of South Florida Morsani College of Medicine, Tampa, FL, USA; [10]UCB Pharma, Monheim, Germany; [11]UCB Pharma, Morrisville, NC, USA; [12]UCB Pharma, Slough, UK; [13]George Washington University, Washington DC, USA

14th MGFA International Conference on Myasthenia and Related Disorders, 10–12 May 2022, Miami, FL, USA — P25

2. Habib et al. Patient-reported and quality-of-life outcomes from MycarinG, a randomized, placebo-controlled, double-blind, Phase 3 trial of rozanolixizumab in generalized myasthenia gravis. Poster 64



Patient-reported and quality-of-life outcomes from MycarinG, a randomized, placebo-controlled, double-blind, Phase 3 trial of rozanolixizumab in generalized myasthenia gravis

Ali A. Habib[1], Artur Druzdz[2], Julian Grosskreutz[3], Henry Kaminski[4], Renato Mantegazza[5], Sabrina Sacconi[6], Kimiaki Utsugisawa[7], John Vissing[8], Tuan Vu[9], Thomas Morel[10], Marion Boehnlein[11], Franz Woltering[11], Ali Bozorg[12], Maryam Gayfieva[12], Vera Bril[13]

[1]MDA ALS and Neuromuscular Center, University of California, Irvine, Orange, CA, USA; [2]Department of Neurology, Municipal Hospital Poznań, Poland; [3]Precision Neurology, Department of Neurology, University of Lübeck, Lübeck, Germany; [4]George Washington University, Washington, DC, USA; [5]Fondazione IRCCS, Istituto Nazionale Neurologico Carlo Besta, Milan, Italy; [6]Université Côte d'Azur, Peripheral Nervous System & Muscle Department, Pasteur 2 Hospital, Centre Hospitalier Universitaire de Nice, Nice, France; [7]Department of Neurology, Hanamaki General Hospital, 4-57 Otsuyachi, Hanamaki, Iwate 025-0082, Japan; [8]Department of Neurology, Rigshospitalet, University of Copenhagen, Copenhagen, Denmark; [9]Department of Neurology, University of South Florida Morsani College of Medicine, Tampa, FL, USA; [10]UCB Pharma, Brussels, Belgium; [11]UCB Pharma, Monheim, Germany; [12]UCB Pharma, Morrisville, North Carolina, USA; [13]UCB Pharma, Slough, UK; [14]University Health Network, Toronto, ON, Canada.

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MycarinG: background and study objective

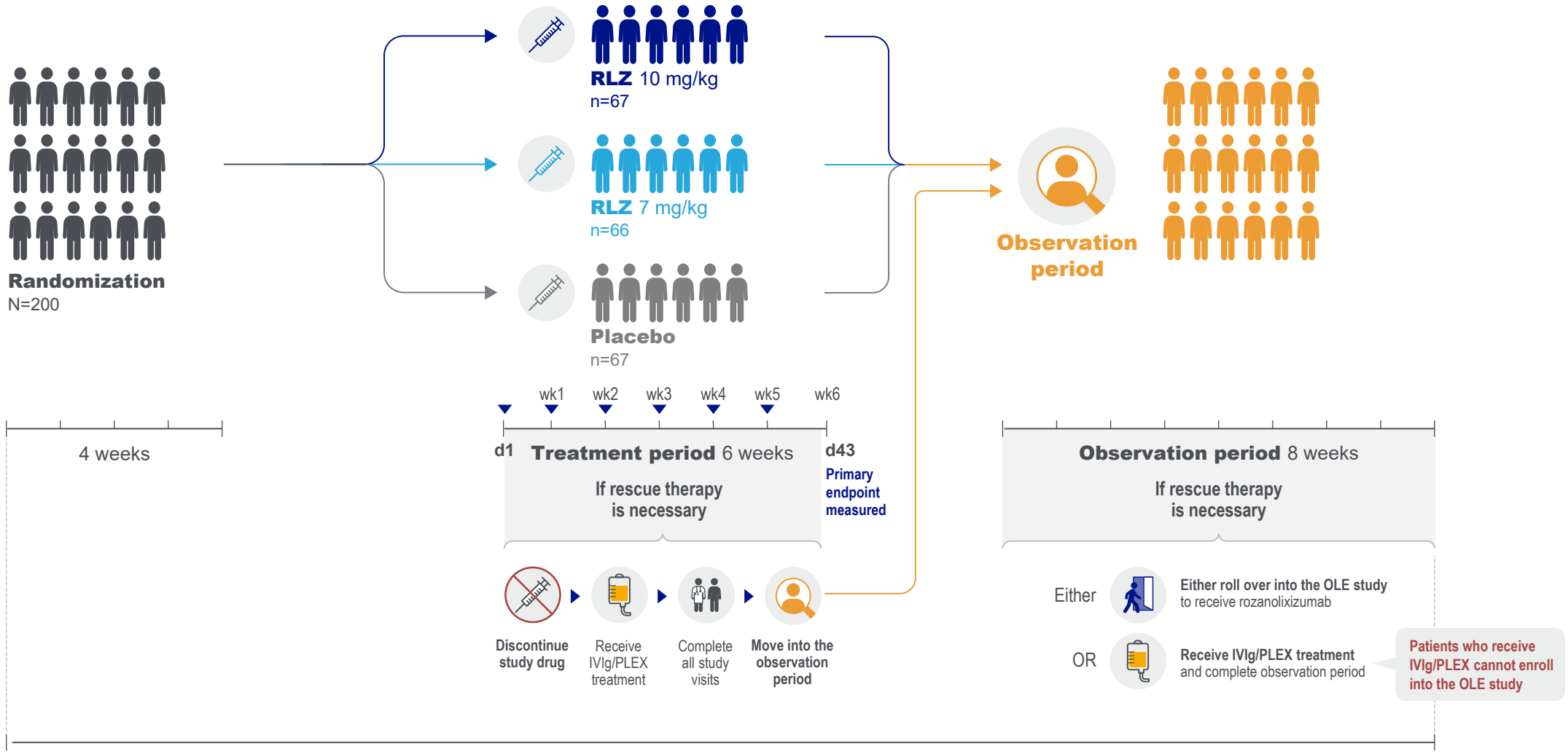
- gMG is a chronic, unpredictable and debilitating rare disease characterized by chronic muscle weakness and fatigue[1]
- gMG can impair patients' ability to carry out routine daily activities and negatively impact quality of life[1–3]
- Pathogenic IgG autoantibodies and complement impair the normal structure and function of the NMJ, leading to reduced muscle contraction[4]
- Rozanolixizumab is a fully humanized IgG4 monoclonal antibody that targets the IgG-binding region of FcRn, inhibiting IgG salvage and recycling, and reducing serum levels of pathogenic autoantibodies[5–7]
- MycarinG[8,9]
  - Randomized, multicenter, double-blind, placebo-controlled, Phase 3 study (MG0003/NCT03971422)
  - Assessed the efficacy and safety of rozanolixizumab in adult patients with AChR or MuSK autoantibody-positive gMG

AChR, acetylcholine receptor; FcRn, neonatal Fc receptor; gMG, generalized myasthenia gravis; IgG, immunoglobulin G; MuSK, muscle-specific kinase; NMJ, neuromuscular junction.

1. Gilhus N, et al. Nat Rev Dis Primers 2019;5:30. 2. Nowak R. Neurol Rev (Suppl) 2018;March:S1. 3. Twork S, et al. Health Qual Life Outcomes. 2010;8:129; 4. Phillips W, et al. F1000Res. 2016;5:F1000 Faculty Rev-513; 5. Roopenian D and Akliesh S. Nat Rev Immunol. 2007;7:715–725; 6. Smith B et al. MAbs. 2018;10:1111–1130; 7. Kiessling P, et al. Sci Trans Med. 2017;9:pii:eaan1208. 8. Bril et al. MGFA 2022, Poster 25. 9. Habib et al. MGFA 2022, Poster 64.

Rozanolixizumab is an investigational new product and has not been approved by any authority.

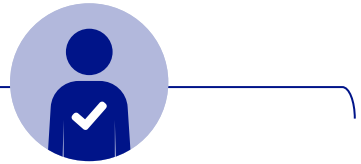
MycarinG study design



Adapted from Bril et al. MGFA 2022, Poster 25; Habib et al. MGFA 2022, Poster 64.
IVIg, intravenous immunoglobulin; OLE, open-label extension; PLEX, plasma exchange; RLZ, rozanolixizumab.

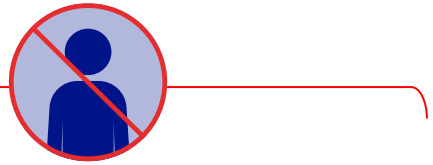
Rozanolixizumab is an investigational new product and has not been approved by any authority.

Inclusions, exclusions, and endpoints



Key inclusion criteria

- **≥18 years** of age
- **Diagnosis of gMG** at screening/baseline
- **Autoantibodies against AChR or MuSK** at screening/baseline
- **MGFA Class II to IVa** at screening/baseline
- **MG-ADL score of** ≥**3** and a **QMG score** ≥**11** at screening/baseline
- **Considered for additional treatment** such as IVIg or PLEX by the investigator
- **Body weight of ≥35 kg**



Key exclusion criteria

- **Clinically relevant active infection** or recent serious infection
- **Severe oropharyngeal or respiratory weakness**
- **Serum total IgG level ≤5.5 g/L**
- **Absolute neutrophil count <1500 cells/mm^3**

Selected endpoints

- The **primary endpoint** was CFB to Day 43 in **MG-ADL** score
- Secondary endpoints included CFB to Day 43 in **MGC**, **QMG** and **MG Symptoms PRO**
- Other endpoints included **MGII**, **MG-QoL 15r**, **EQ-5D-5L**, **total IgG** and **anti-AChR** autoantibody levels
- **Safety and tolerability** of rozanolixizumab were also assessed

Bril et al. MGFA 2022, Poster 25; Habib et al. MGFA 2022, Poster 64.
AChR, acetylcholine receptor; CFB, change from baseline; EQ-5D-5L, EuroQol- 5 Dimension questionnaire; FcRn, neonatal Fc receptor; gMG, generalized myasthenia gravis; IgG, immunoglobulin G; IVIg, intravenous immunoglobulin; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGC, Myasthenia Gravis Composite; MGFA, Myasthenia Gravis Foundation of America; MGII, Myasthenia Gravis Impairment Index; MG-QoL 15r, Revised 15-item Myasthenia Gravis Quality of Life score; MuSK, muscle-specific kinase; PLEX, plasma exchange; PRO, patient-reported outcome; QMG, Quantitative Myasthenia Gravis; RLZ, rozanolixizumab; TB, tuberculosis.

Patient demographics and baseline characteristics

Baseline demographic and disease characteristics were balanced between treatment groups

		Placebo (n=67)	RLZ 7 mg/kg (n=66)	RLZ 10 mg/kg (n=67)
Age, years, mean (SD)		50.4 (17.7)	53.2 (14.7)	51.9 (16.5)
Sex, female, n (%)		47 (70.1)	39 (59.1)	35 (52.2)
MGFA disease class at baseline, n (%)	Class II	23 (34.3)	29 (43.9)	26 (38.8)
	Class III	41 (61.2)	34 (51.5)	39 (58.2)
	Class IVa/b	3 (4.5)	3 (4.5)	2 (3.0)
Geographic region, n (%)	North America	21 (31.3)	21 (31.8)	18 (26.9)
	Europe	41 (61.2)	36 (54.5)	43 (64.2)
	Asia (inc. Japan)	5 (7.5)	9 (13.7)	6 (9.0)
Race, n (%)	Asian	5 (7.5)	9 (13.6)	7 (10.4)
	Black	1 (1.5)	0	4 (6.0)
	White	46 (68.7)	41 (62.1)	49 (73.1)
	Missing*	14 (20.9)	16 (24.2)	7 (10.4)

Adapted from Bril et al. MGFA 2022, Poster 25; Habib et al. MGFA 2022, Poster 64.
*Data on race were not permitted to be collected in certain countries.
MGFA, Myasthenia Gravis Foundation of America; RLZ, rozanolixizumab; SD, standard deviation.

Patient demographics and baseline characteristics

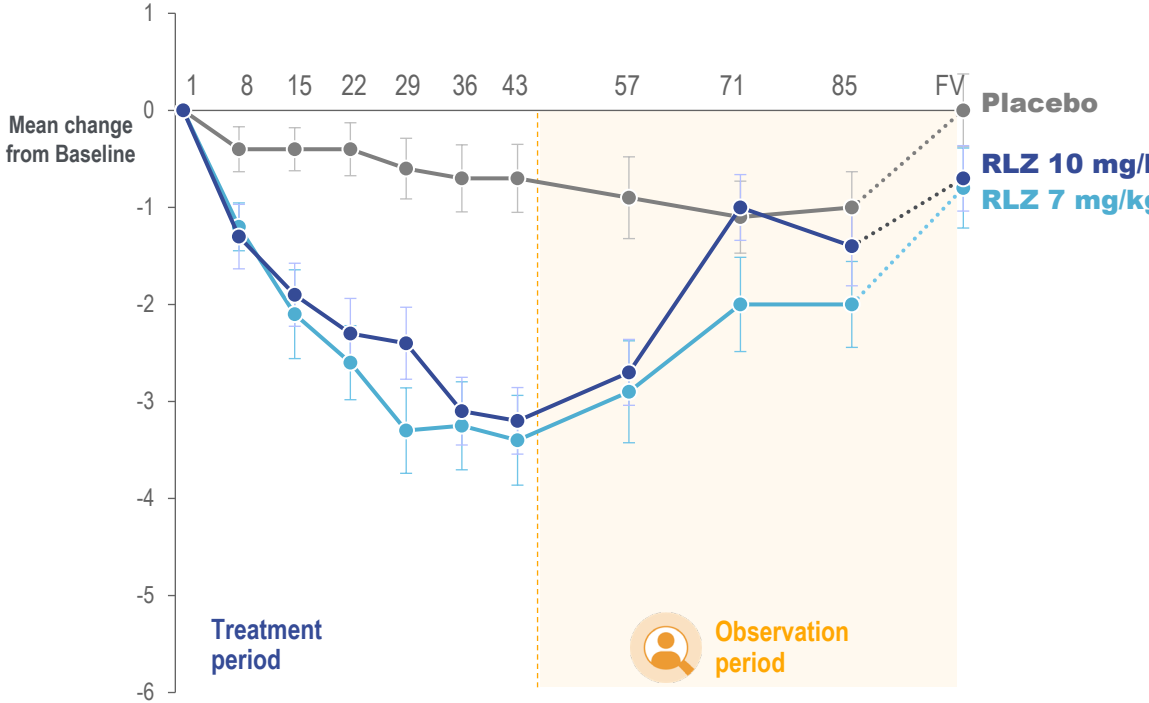
Baseline demographic and disease characteristics were balanced between treatment groups

		Placebo (n=67)	RLZ 7 mg/kg (n=66)	RLZ 10 mg/kg (n=67)
Baseline medications, n (%)	Corticosteroids for systemic use	38 (56.7)	43 (65.2)	48 (71.6)
	Immunosuppressants	33 (49.3)	32 (48.5)	38 (56.7)
	Parasympathomimetics	60 (89.6)	55 (83.3)	57 (85.1)
Duration of disease, years, mean (SD)		9.4 (9.3)	6.9 (6.8)	9.6 (9.9)
MG-ADL score at baseline, mean (SD)		8.4 (3.4)	8.4 (3.8)	8.1 (2.9)
QMG score at baseline, mean (SD)		15.8 (3.5)	15.4 (3.7)	15.6 (3.7)

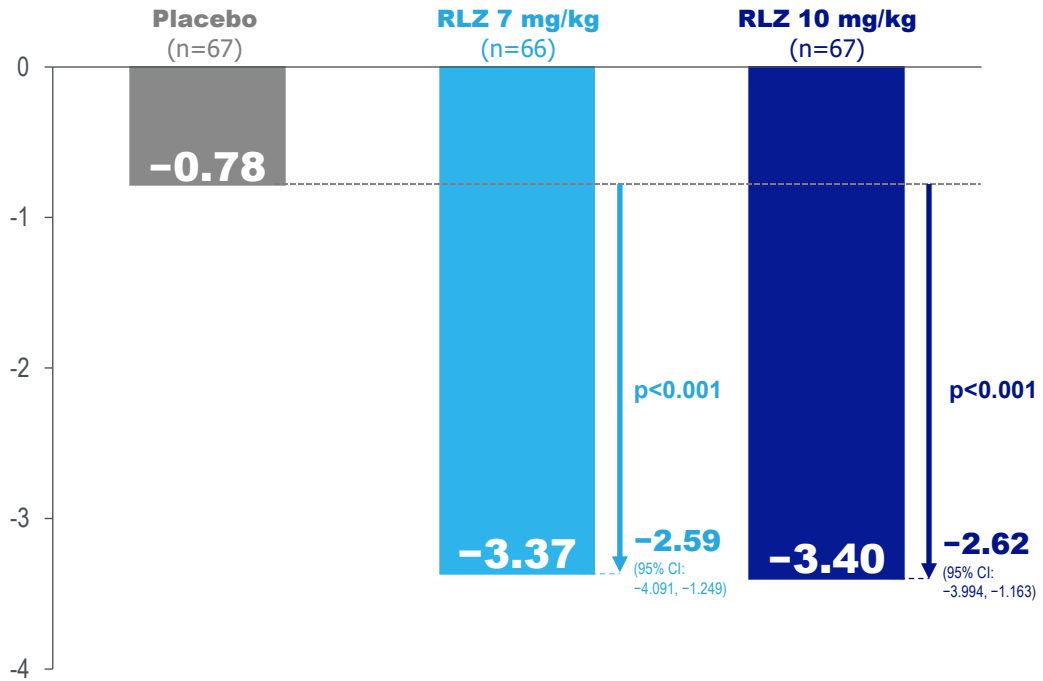
Adapted from Bril et al. MGFA 2022, Poster 25; Habib et al. MGFA 2022, Poster 64.
MG-ADL, myasthenia gravis activities of daily living; QMG, quantitative myasthenia gravis; RLZ, rozanolixizumab; SD, standard deviation.

Mean CFB in MG-ADL and LS mean MG-ADL CFB at Day 43

CFB with rozanolixizumab 7 mg/kg and 10 mg/kg were both clinically meaningfully and highly statistically significantly improved compared with placebo (p<0.001) for both doses



Day of visit	1	8	15	22	29	36	43	57	71	85	FV
Placebo, n	67	67	66	65	64	61	64	54	49	46	64
RLZ 7 mg/kg, n	66	66	62	62	63	63	64	55	52	43	64
RLZ 10 mg/kg, n	67	66	64	64	61	60	62	63	59	57	64



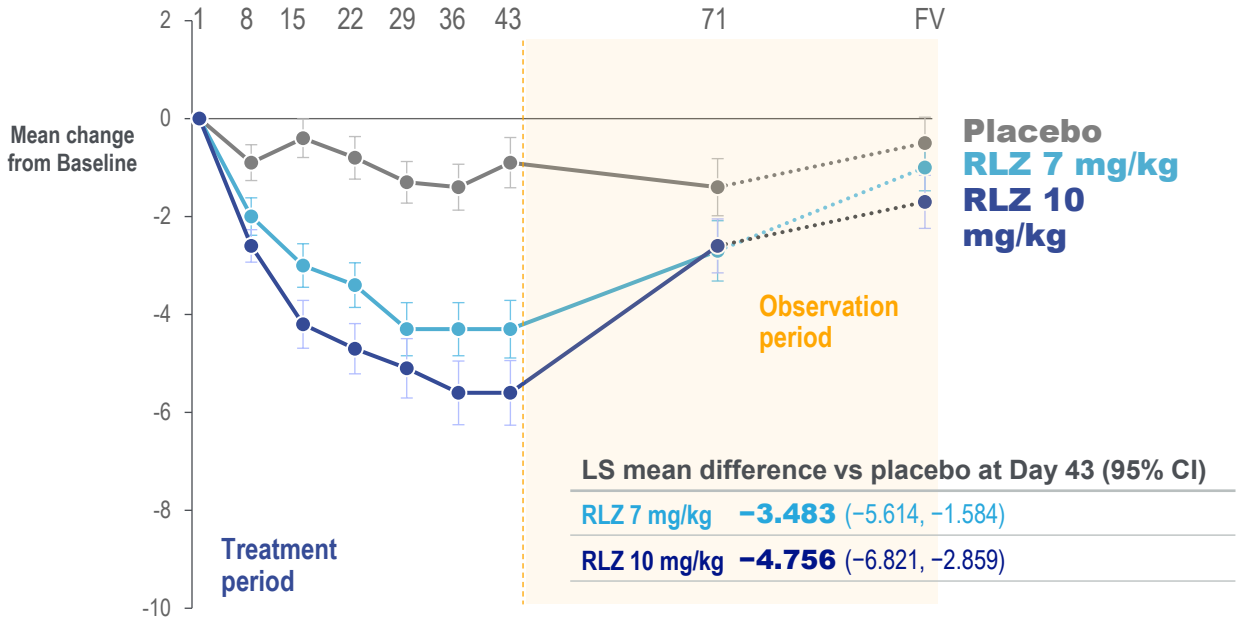
LS mean difference vs placebo (95% CI)		
RLZ 7 mg/kg	**−2.586**	(−4.091, −1.249)
RLZ 10 mg/kg	**−2.619**	(−3.994, −1.163)

Adapted from Bril et al. MGFA 2022, Poster 25; Habib et al. MGFA 2022, Poster 64.
CFB, change from baseline; CI, confidence interval; FV, final visit (could occur on any day up to Day 99); LS, least squares; MG-ADL, Myasthenia Gravis Activities of Daily Living; RLZ, rozanolixizumab.

Mean CFB in QMG and MGC scores

CFB to Day 43 was highly statistically significantly greater in QMG and MGC in both rozanolixizumab groups compared to placebo (p<0.001)

QMG (p<0.001 vs placebo)

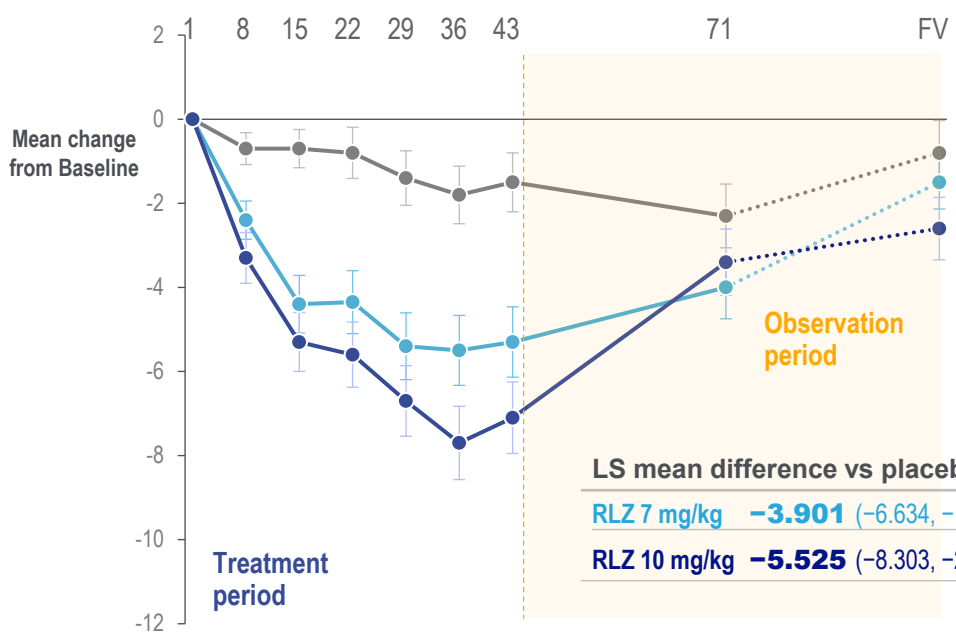


LS mean difference vs placebo at Day 43 (95% CI)

RLZ 7 mg/kg	**−3.483** (−5.614, −1.584)
RLZ 10 mg/kg	**−4.756** (−6.821, −2.859)

Day of visit	1	8	15	22	29	36	43	71	FV
Placebo, n	67	67	66	65	64	59	64	47	63
RLZ 7 mg/kg, n	66	66	62	62	63	63	64	51	64
RLZ 10 mg/kg, n	67	66	64	64	60	59	62	58	63

MGC (p<0.001 vs placebo)



LS mean difference vs placebo at Day 43 (95% CI)

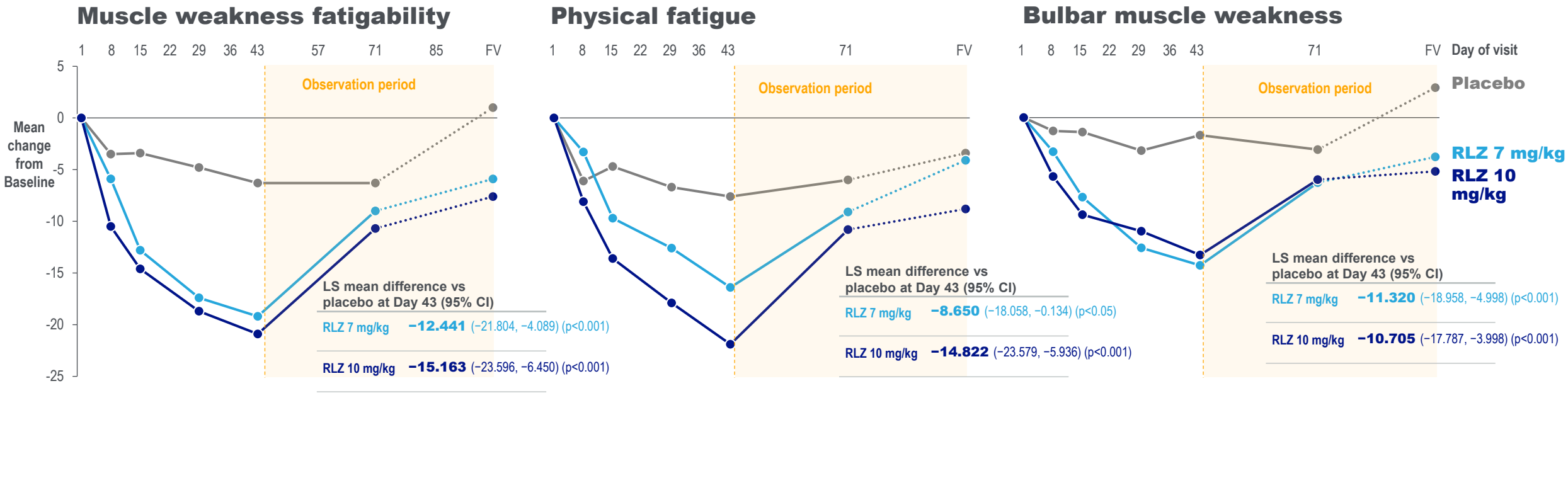
RLZ 7 mg/kg	**−3.901** (−6.634, −1.245)
RLZ 10 mg/kg	**−5.525** (−8.303, −2.968)

	1	8	15	22	29	36	43	71	FV
Placebo	67	67	66	65	64	60	64	47	63
RLZ 7 mg/kg	66	66	62	62	63	63	64	52	64
RLZ 10 mg/kg	67	66	64	60	64	59	62	58	63

Adapted from Bril et al. MGFA 2022, Poster 25.
CFB, change from baseline; FV, final visit (could occur up to Day 99); MGC, Myasthenia Gravis Composite; QMG, Quantitative Myasthenia Gravis; RLZ, rozanolixizumab CI, confidence interval. LS, least-squares

Mean CFB in key MG Symptoms PRO scores

Improvements in key MG symptoms PRO dimensions over time were observed for both RLZ dose groups versus placebo at Day 43

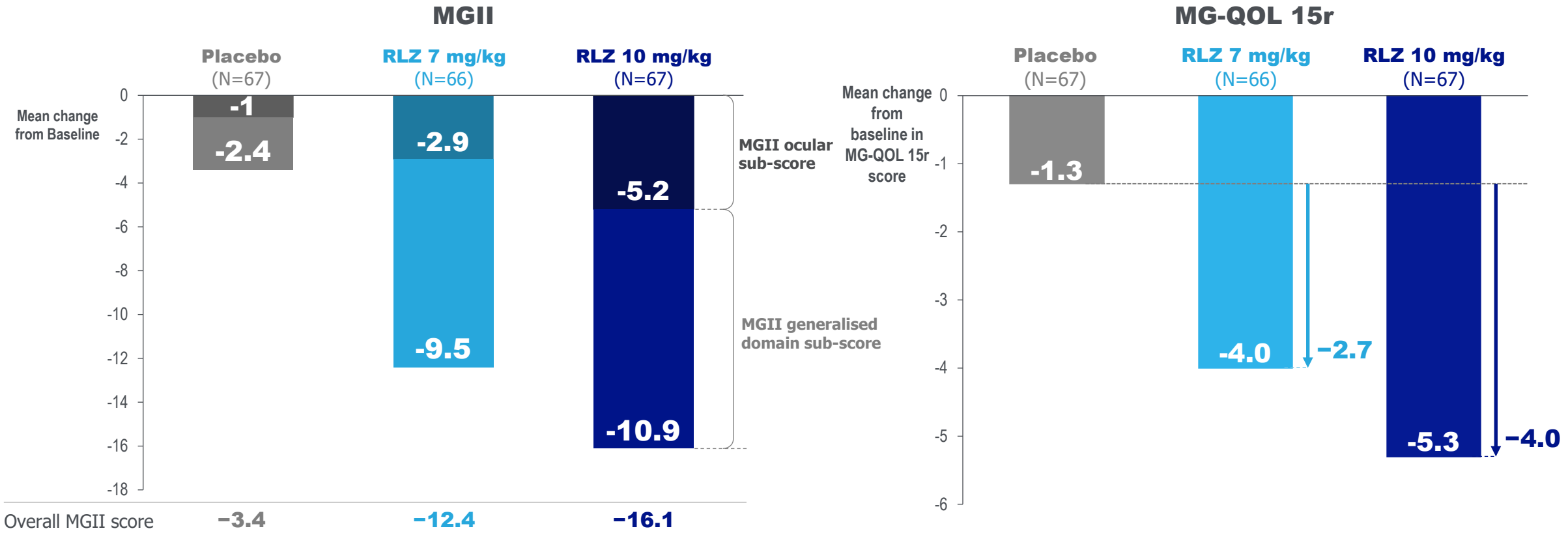


Adapted from Habib et al. MGFA 2022, Poster 64.
CFB, change from Baseline; FV, final visit (could occur up to Day 99); MG, myasthenia gravis; PRO, patient-reported outcome; RLZ, rozanolixizumab. CI, confidence interval. LS, least-squares

Rozanolixizumab is an investigational new product and has not been approved by any authority.

Mean CFB in MGII scores and sub-scores and MG-QoL 15r at Day 43

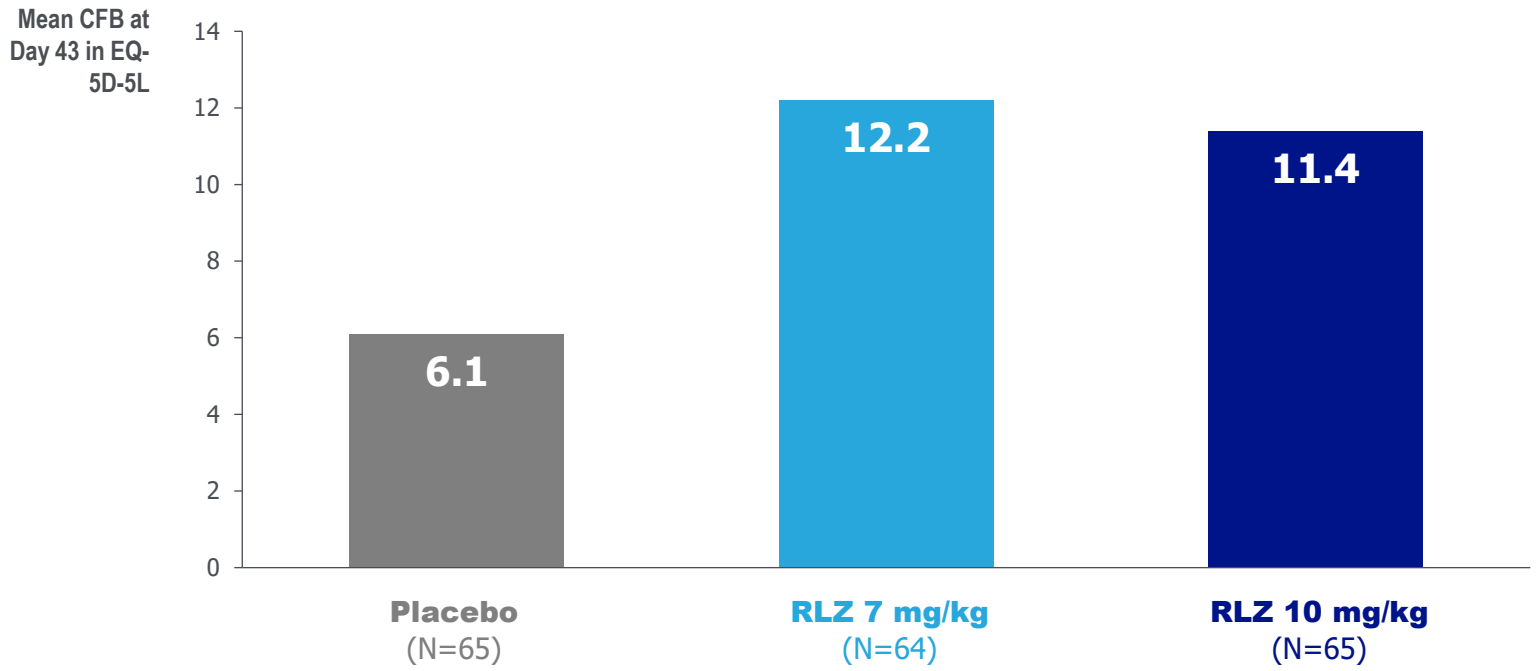
Improvement in MGII score, MGII ocular sub-score and MGII generalised domain sub-score at Day 43 were greater for both RLZ dose groups versus placebo, and improvement in health-related quality of life (MG-QOL15r) was greater for both RLZ dose groups versus placebo



Adapted from Habib et al. MGFA 2022, Poster 64.
CFB, change from Baseline; MGQoL15r, Myasthenia Gravis Quality of Life 15-item scale revised; MGII, myasthenia gravis impairment index; RLZ, rozanolixizumab.

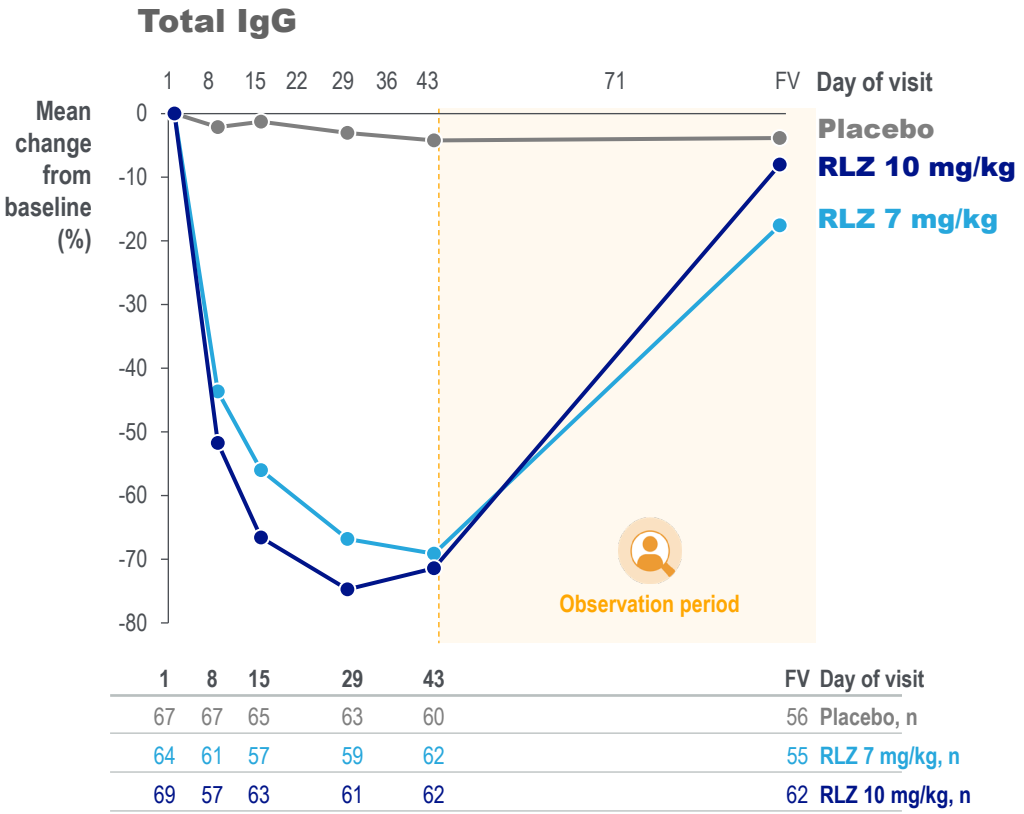
CFB to Day 43 in EQ-5D-5L at Day 43

Improvement in patient-reported health (EQ-5D-5L) was greater for both RLZ dose groups versus placebo



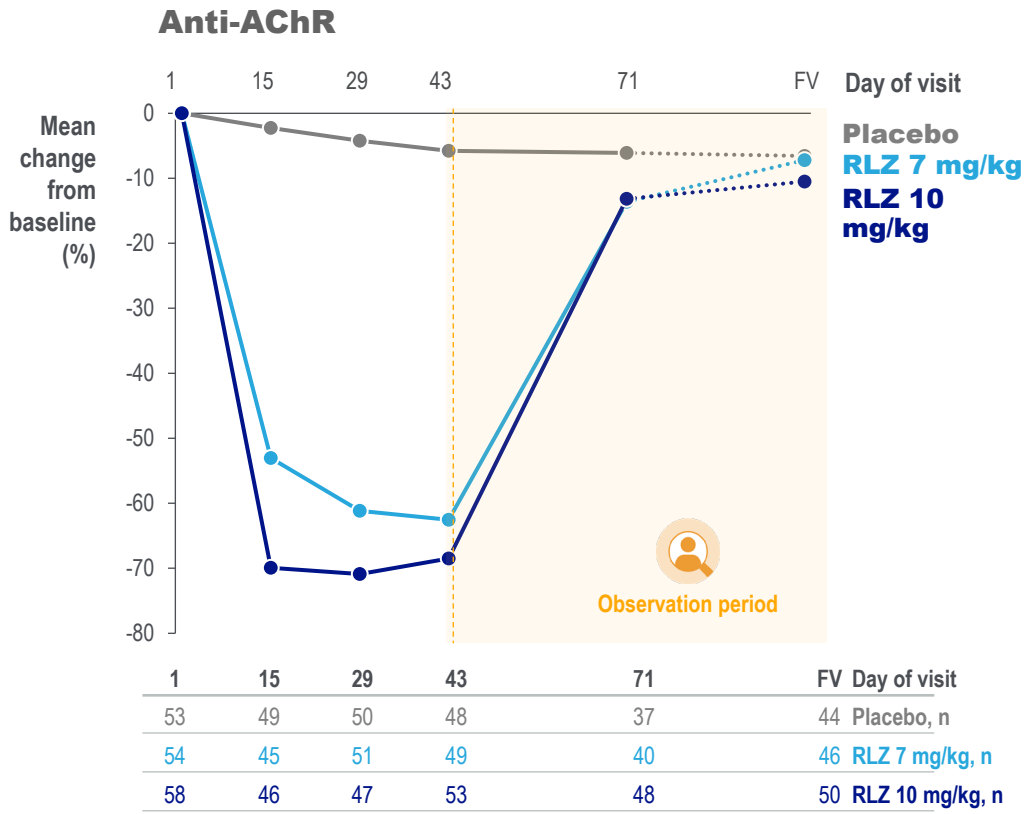
Adapted from Habib et al. MGFA 2022, Poster 64.
CFB, change from Baseline; EQ-5D-5L, EuroQol- 5 Dimension questionnaire; RLZ, rozanolixizumab.

CFB in total IgG and MG-specific autoantibodies



1	8	15	29	43	FV	Day of visit
67	67	65	63	60	56	Placebo, n
64	61	57	59	62	55	RLZ 7 mg/kg, n
69	57	63	61	62	62	RLZ 10 mg/kg, n

Mean maximum IgG reduction was 71% for RLZ 7 mg/kg and 78% for RLZ 10 mg/kg and included subclasses 1-4



1	15	29	43	71	FV	Day of visit
53	49	50	48	37	44	Placebo, n
54	45	51	49	40	46	RLZ 7 mg/kg, n
58	46	47	53	48	50	RLZ 10 mg/kg, n

Anti-AChR autoantibody levels decreased over the treatment period in line with the total IgG reduction

Adapted from Bril et al. MGFA 2022, Poster 25.
AChR, acetylcholine receptor; CFB, change from baseline; FV, final visit (could occur up to Day 99); IgG, immunoglobulin class G; MG, myasthenia gravis; MuSK, muscle-specific kinase; RLZ, rozanolixizumab.

Overview of TEAEs

- A higher proportion of TEAEs occurred in the rozanolixizumab 7 mg/kg (81.3%) and 10 mg/kg (82.6%) arms than in the placebo arm (67.2%)

Preferred term	**Placebo** (n=67) % (n)	**RLZ 7 mg/kg** (n=64) % (n)	**RLZ 10 mg/kg** (n=69) % (n)
Any TEAE	**67.2** (45)	**81.3** (52)	**82.6** (57)
Serious TEAEs	**9.0** (6)	**7.8** (5)	**10.1** (7)
Participant withdrawal from study due to TEAEs	**3.0** (2)	**3.1** (2)	**7.2** (5)
Participant withdrawal of IMP due to TEAEs	**3.0** (2)	**3.1** (2)	**5.8** (4)
Temporary withdrawal of IMP due to TEAEs	**1.5** (1)	**4.7** (3)	**8.7** (6)
Treatment-related TEAEs	**32.8** (22)	**50.0** (32)	**56.5** (39)
Severe TEAEs	**4.5** (3)	**4.7** (3)	**18.8** (13)
All deaths (AEs leading to death)	**0**	**0**	**0**

Adapted from Bril et al. MGFA 2022, Poster 25.
*Treatment-related TEAEs as assessed by investigators.
AE, adverse event; IMP, investigational medicinal product; RLZ, rozanolixizumab; TEAE, treatment-emergent adverse event.

Overview of TEAEs

- The majority of TEAEs were mild or moderate in intensity
- The most common TEAEs were
  - Headache (45.3%, 37.7% and 19.4%)
  - Diarrhea (25.0%, 15.9% and 13.4%)
  - Pyrexia (12.5%, 20.3% and 1.5%)
  - Nausea (7.8%, 11.6% and 7.5%)
- Most headaches were of mild-to-moderate intensity, with severe headaches adequately managed with over-the-counter medications (non-opioid analgesics such as paracetamol or ibuprofen)
- The incidence of infections was higher in the rozanolixizumab 10 mg/kg group (30.4%) than in the rozanolixizumab 7 mg/kg (15.6%) and placebo (19.4%) groups
- Overall, infusions were well-tolerated with low incidence of local injection-site reactions reported
- No serious or severe infections occurred in either rozanolixizumab group, with most infections of mild severity

Bril et al. MGFA 2022, Poster 25
TEAE, treatment-emergent adverse event.

Conclusions

- The primary objective was met with a **clinically meaningful and highly statistically significant improvement** in MG-ADL at Day 43
  - Both doses also **clinically meaningfully and highly statistically significantly improved** MGC and QMG scores over placebo
- Both rozanolixizumab 7 mg/kg and 10 mg/kg improved patients' symptoms and their **ability to undertake daily activities**, as demonstrated by MG-ADL, MG Symptoms PRO scales, MG-QoL 15r and EQ-5D-5L, with an acceptable safety profile
  - The MG Symptoms PRO used in this study provides a more granular and detailed assessment of muscle weakness and muscle weakness fatigability symptoms than currently available PRO measures and also includes fatigue, a key concern to people with MG
  - Further validation of the MG Symptoms PRO measure is ongoing
- Rozanolixizumab had an **acceptable safety profile and was generally well tolerated**
- These results suggest that rozanolixizumab has **potential as a new treatment option** for patients with gMG and further substantiate the therapeutic benefit of targeting FcRn for patients with gMG

Bril et al. MGFA 2022, Poster 25; Habib et al. MGFA 2022, Poster 64.
CFB, change from baseline; FcRn, neonatal Fc receptor; gMG, generalized myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGC, Myasthenia Gravis Composite; PRO, patient-reported outcome; QMG, Quantitative Myasthenia Gravis; TEAE, treatment-emergent adverse event.

Q&A

Antje Witte, Head of Investor Relations, UCB