Improvement of ocular subdomain scores with zilucoplan in patients with generalized myasthenia gravis in RAISE and RAISE-XT studies

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Introduction

- Ocular symptoms are burdensome for people with gMG and can negatively impact their quality of life[1]
- In the pivotal Phase 3 RAISE study (NCT04115293), zilucoplan, a complement C5 inhibitor, demonstrated clinically meaningful and statistically significant improvements in MG-ADL and QMG total scores in patients with anti-AChR Ab+ gMG[2]
- The objective of this analysis was to evaluate the effect of zilucoplan on ocular symptoms in patients with gMG in RAISE and RAISE-XT (NCT04225871), an ongoing, Phase 3, open-label extension study

Methods

- In RAISE, adults with anti-AChR Ab+ gMG were randomized to self-administer either subcutaneous zilucoplan 0.3 mg/kg or placebo once daily for 12 weeks
- Patients who completed RAISE or a Phase 2 study (NCT03315130) entered RAISE-XT to receive zilucoplan 0.3 mg/kg
  - The primary outcome of RAISE-XT was incidence of TEAEs
- We assessed (*post hoc*) CFB in MG-ADL, QMG and MG-QoL 15r ocular subdomain scores at Week 12 of RAISE and Week 120 of RAISE-XT in patients with baseline scores ≥1 in that subdomain, as well as in individual ocular items for each scale at Week 120 (**Table 1**)
- The interim data cut-off date for RAISE-XT was 11 November 2023

Results

- Overall, 174 patients enrolled in RAISE (zilucoplan, n=86; placebo, n=88)
- At Week 12 of RAISE, mean CFB in ocular subdomain scores across MG-ADL, QMG and MG-QoL 15r was greater for zilucoplan than placebo (**Figure 1**)
- In total, 200 patients enrolled in RAISE-XT
- The improvements observed in the ocular subdomain scores of these assessments were sustained through to Week 120 in RAISE-XT (**Figure 2**)
  - Similar improvements were also observed in patients' scores on individual ocular items for these assessments (**Figure 3**)
- In RAISE-XT, TEAEs occurred in 97.0% (194/200) of patients
  - Overall, 40.5% (81/200) of patients experienced a serious TEAE, of whom 2.5% (5/200) experienced a serious treatment-related TEAE

Summary and conclusions

This *post hoc* analysis evaluated the effect of zilucoplan on ocular symptoms in patients with gMG in RAISE and RAISE-XT

Treatment with zilucoplan led to improvements in MG-ADL, QMG and MG-QoL 15r ocular subdomain scores versus placebo in the RAISE study

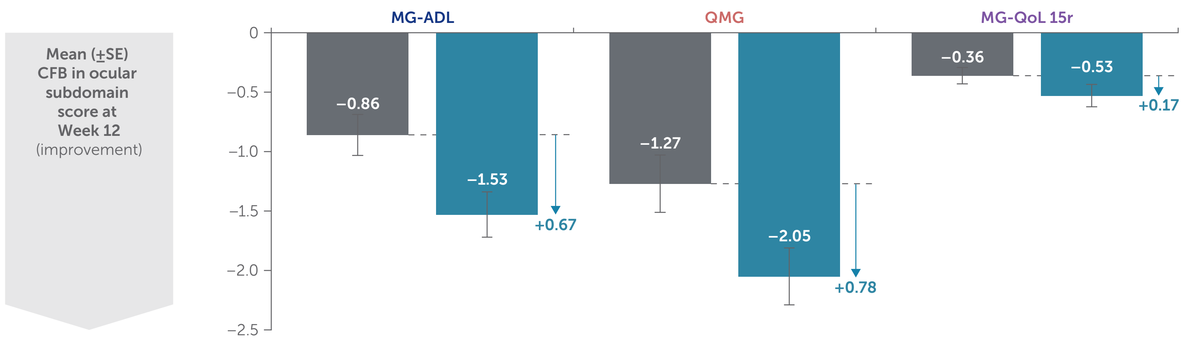
The improvements in ocular subdomain scores were sustained with long-term use of zilucoplan, through to Week 120

These data further support zilucoplan as a treatment option for patients with gMG, including those with ocular symptoms

Table 1 Ocular items in MG-ADL, QMG and MG-QoL 15r assessments

	Ptosis	Diplopia	Facial muscles	Trouble with eyes	Total ocular score
MG-ADL	Eyelid droop (score 0–3)	Double vision (score 0–3)	–	–	6
QMG	Ptosis (upward gaze)(score 0–3)	Double vision on lateral gaze (score 0–3)	Facial muscles (score 0–3)	–	9
MG-QoL 15r	–	–	–	I have trouble with my eyes because of my MG (e.g. double vision) (score 0–2)	2

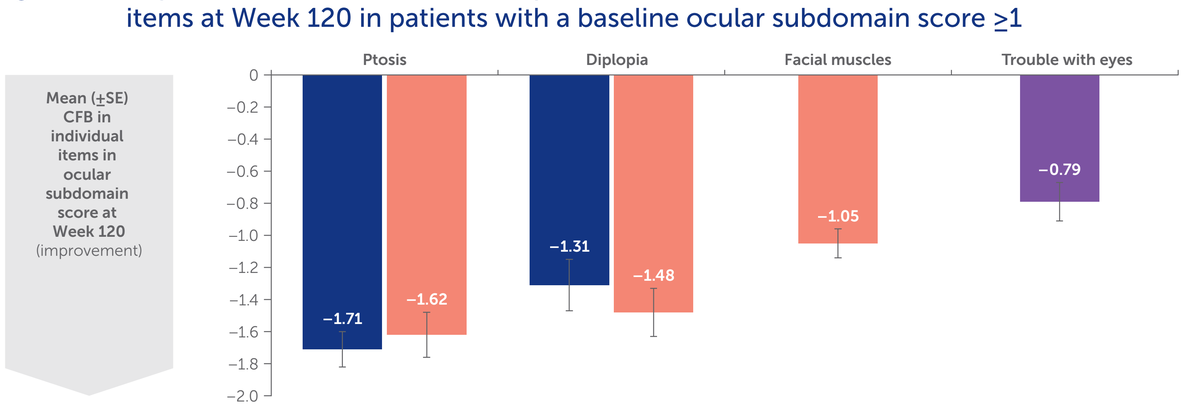
Figure 1 In patients who were experiencing ocular symptoms at baseline, improvement in ocular subdomain scores at Week 12 was greater with zilucoplan than placebo



Subdomain score at baseline		MG-ADL	QMG	MG-QoL 15r
Placebo	n	**83**	**83**	**78**
	Mean (SE)	**3.66** (0.17)	**5.11** (0.22)	**1.56** (0.06)
Zilucoplan 0.3 mg/kg	n	**79**	**81**	**76**
	Mean (SE)	**3.39** (0.16)	**4.88** (0.22)	**1.53** (0.06)

mITT population, which included all patients who received at least one dose of zilucoplan and had at least one post-dosing MG-ADL score. CFB assessed in patients with ocular subdomain scores ≥1.

Figure 3 Improvements were observed in patients' scores on all individual ocular subdomain items at Week 120 in patients with a baseline ocular subdomain score ≥1

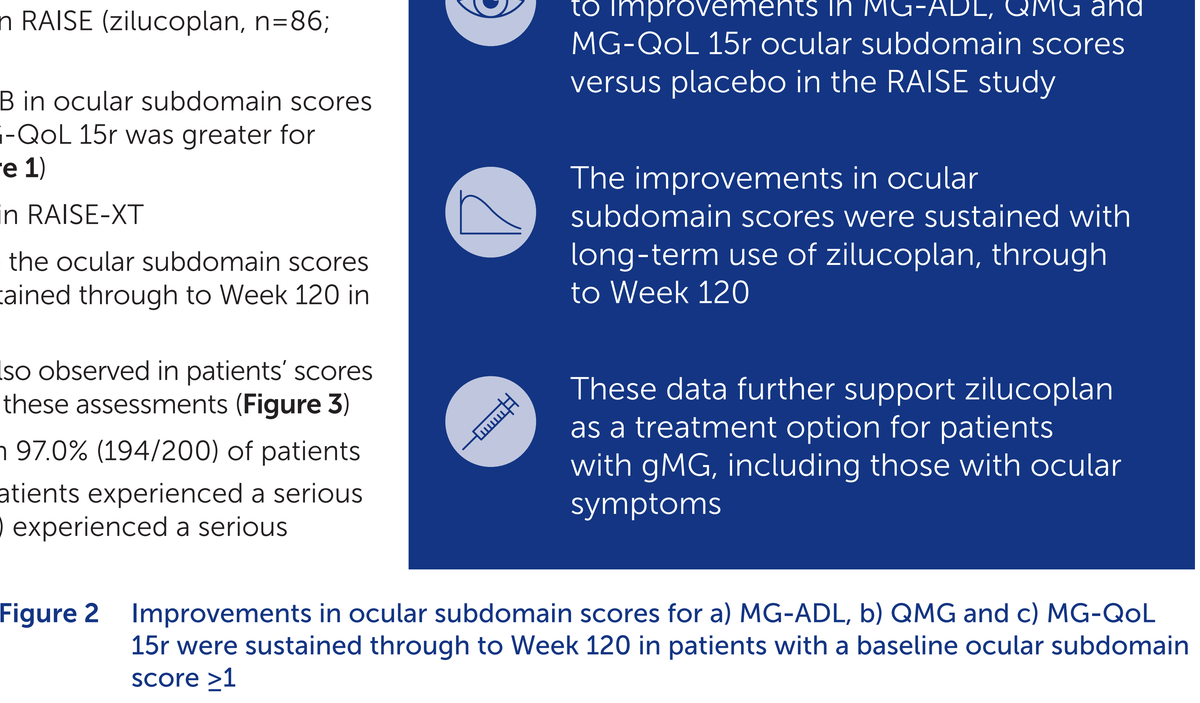


Subdomain score at baseline		Ptosis	Diplopia	Facial muscles	Trouble with eyes
MG-ADL	n	**164**	**148**		
	Mean (SE)	**2.09** (0.06)	**1.95** (0.06)		
QMG	n	**151**	**161**	**155**	
	Mean (SE)	**1.99** (0.07)	**2.40** (0.06)	**1.44** (0.05)	
MG-QoL 15r	n				**160**
	Mean (SE)				**1.51** (0.04)

mITT population. Scoring scales range from 0 to 3 for each individual item for MG-ADL and QMG, and 0 to 2 for MG-QoL 15r.

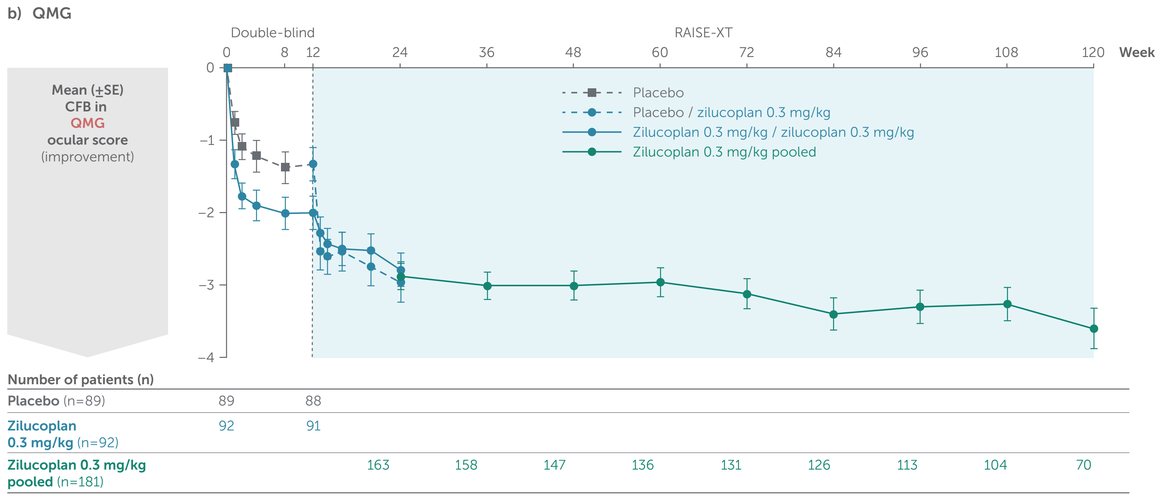
Figure 2 Improvements in ocular subdomain scores for a) MG-ADL, b) QMG and c) MG-QoL 15r were sustained through to Week 120 in patients with a baseline ocular subdomain score ≥1

a) MG-ADL



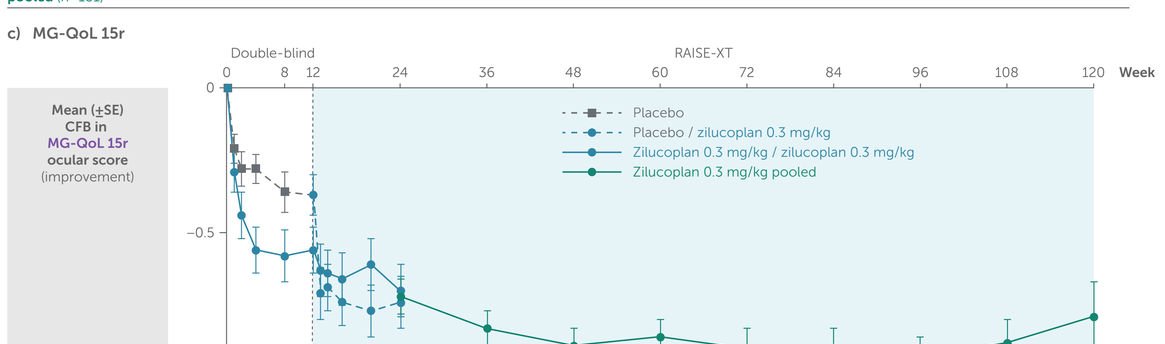
Number of patients (n)											
Placebo (n=89)	89	89									
Zilucoplan 0.3 mg/kg (n=90)	90	90									
Zilucoplan 0.3 mg/kg pooled (n=179)			164	157	146	137	129	127	121	105	71

b) QMG



Number of patients (n)											
Placebo (n=89)	89	88									
Zilucoplan 0.3 mg/kg (n=92)	92	91									
Zilucoplan 0.3 mg/kg pooled (n=181)			163	158	147	136	131	126	113	104	70

c) MG-QoL 15r



Number of patients (n)											
Placebo (n=79)	79	79									
Zilucoplan 0.3 mg/kg (n=81)	81	79									
Zilucoplan 0.3 mg/kg pooled (n=160)			141	138	128	119	115	111	97	91	56

mITT population.

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

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