Sustained minimal symptom expression in generalized myasthenia gravis: A 120-week *post hoc* analysis of RAISE-XT

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Introduction

- Zilucoplan is a small (15-amino acid) macrocyclic peptide complement C5 inhibitor approved for the treatment of adults with anti-AChR Ab+ gMG[1,2]
- The efficacy and safety of zilucoplan were assessed in two randomized, placebo-controlled, double-blind studies (Phase 2 [NCT03315130]; Phase 3 [RAISE, NCT04115293]), and are being further explored in RAISE-XT (NCT04225871), an ongoing, Phase 3 OLE study[2,3]
- Achievement of MSE is being increasingly recognized as a treatment goal for patients with gMG[4]
- This *post hoc* analysis of the Phase 2, RAISE and RAISE-XT studies assessed the durability of MSE response in patients treated with zilucoplan for up to 120 weeks

Methods

- In RAISE, adults with MGFA Disease Class II–IV anti-AChR Ab+ gMG were randomized 1:1 to once-daily subcutaneous zilucoplan 0.3 mg/kg or placebo for 12 weeks[2]
- Patients completing RAISE, or the qualifying Phase 2 study, could enter RAISE-XT to self-administer once-daily subcutaneous injections of zilucoplan 0.3 mg/kg[3]
  - The primary outcome of RAISE-XT was incidence of TEAEs[3]
- The following outcomes were assessed *post hoc* (RAISE-XT data cutoff: November 11, 2023) from the start of zilucoplan treatment:
  - The cumulative proportion of patients who achieved MSE (MG-ADL score of 0 or 1, without rescue therapy) for the first time at any time during zilucoplan treatment up to Week 120, calculated using Kaplan–Meier analysis
  - The cumulative proportion of patients who achieved both MG-ADL response (≥3-point improvement without rescue therapy) and MSE at any time during zilucoplan treatment up to Week 120, calculated using Kaplan–Meier analysis
  - The proportion of time spent in MSE up to Week 120

Results

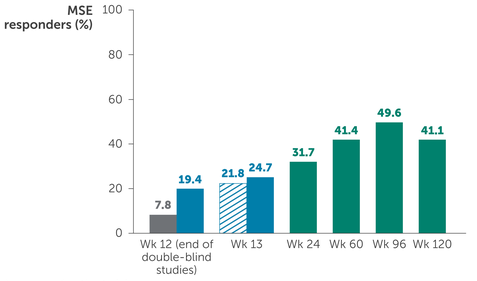
- Of 200 patients enrolled in RAISE-XT, 183 received zilucoplan 0.3 mg/kg or placebo in the double-blind studies (**Table 1**)
- MSE responder rates at Week 12 increased through to Week 24 and were sustained through to Week 120 (**Figure 1**)
- The cumulative proportion of patients achieving MSE at any time from the start of zilucoplan treatment up to Week 120 was 63% in the pooled zilucoplan 0.3 mg/kg group (N=183; **Figure 2**)
  - In the zilucoplan 0.3 mg/kg / zilucoplan 0.3 mg/kg (n=93) and placebo / zilucoplan 0.3 mg/kg (n=90) groups, the cumulative proportion of patients who had achieved MSE by Week 120 was 61% and 64%, respectively
- The cumulative proportion of patients achieving MG-ADL response at any time up to Week 120 was 89%; of these responders, 62% achieved MSE at any time up to Week 120
- Some patients achieved MSE after 1 week of zilucoplan treatment (**Figure 2**)
  - Median time to achievement of MSE from the start of zilucoplan treatment was 36.7 (95% CI: 24–63) weeks in the pooled zilucoplan 0.3 mg/kg group (N=183)
- After achievement of MSE during zilucoplan treatment, patients (n=102) maintained their MSE response for a median of 80.8% (range: 0.8–100.0%) of time up to Week 120 (**Figure 3**)
- Over a median of 2.2 (range: 0.1–5.6) years' exposure in RAISE-XT, TEAEs were experienced by 97.0% (n/N=194/200) of patients; most were mild or moderate

Table 1 Baseline demographics and disease characteristics were indicative of a broad population of patients with mild-to-severe gMG

		Zilucoplan 0.3 mg/kg pooled (N=183)
	Age, years, mean (SD)	**52.9** (15.0)
	Sex, male, n (%)	**83** (45.4)
MGFA Disease Class, n (%)	IIa/b	**54** (29.5)
	IIIa/b	**117** (63.9)
	IVa/b	**12** (6.6)
	MG-ADL score, mean (SD)	**10.3** (3.0)
	QMG score, mean (SD)	**19.0** (4.1)
	Prior thymectomy, n (%)	**88** (48.1)
	Prior MG crisis, n (%)	**59** (32.2)
	Duration of disease, years, mean (SD)*	**9.1** (9.9)
Baseline gMG-specific therapies, n (%)	Cholinesterase inhibitors	**150** (82.0)
	Corticosteroids	**120** (65.6)
	Immunosuppressants	**94** (51.4)

Subset of the mITT population, including all patients enrolled in RAISE-XT who received ≥1 dose of zilucoplan 0.3 mg/kg and had ≥1 post-dosing MG-ADL score.
Data obtained from double-blind baseline. *From disease diagnosis.

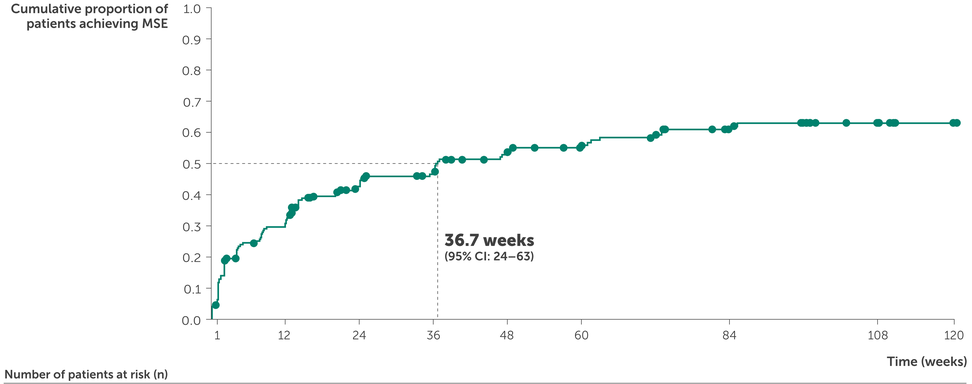
Figure 1 High MSE responder rates were sustained from Week 24 through to Week 120 in the pooled zilucoplan group



Number of patients (n)	Wk 12 (end of double-blind studies)	Wk 13	Wk 24	Wk 60	Wk 96	Wk 120
Placebo (n=90)	90	87	–	–	–	–
Zilucoplan 0.3 mg/kg (n=93)	93	89	–	–	–	–
Zilucoplan 0.3 mg/kg pooled (N=183)	–	–	167	140	123	73

Subset of the mITT population, which included all patients enrolled in RAISE-XT who received ≥1 dose of zilucoplan 0.3 mg/kg and had ≥1 post-dosing MG-ADL score. Dashed bar at Week 13 includes patients who received placebo in the double-blind studies and switched to zilucoplan 0.3 mg/kg upon entering RAISE-XT.

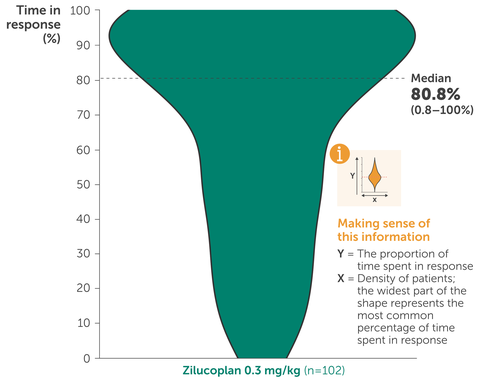
Figure 2 MSE was attained as rapidly as 1 week after starting zilucoplan treatment



Number of patients at risk (n)	1	12	24	36	48	60	84	108	120
Zilucoplan 0.3 mg/kg pooled (N=183)	174	125	89	79	64	56	40	25	20

Subset of the mITT population, which included all patients enrolled in RAISE-XT who received ≥1 dose of zilucoplan 0.3 mg/kg and had ≥1 post-dosing MG-ADL score. Time to MSE was defined as the time from the start of zilucoplan treatment (double-blind baseline for patients treated with zilucoplan in the double-blind studies, and RAISE-XT baseline for patients treated with placebo in the double-blind studies).

Figure 3 Once MSE was achieved, patients spent the majority of their time in response up to Week 120



Subset of the mITT population, further restricted to patients who achieved MSE during zilucoplan 0.3 mg/kg treatment.

Summary and conclusions

This *post hoc* analysis of pooled data from the Phase 2, RAISE and RAISE-XT zilucoplan studies assessed the durability of MSE response in patients with anti-AChR Ab+ gMG treated with zilucoplan for up to 120 weeks

In total, 63% of patients achieved MSE at any time during treatment with zilucoplan up to Week 120

Median time to achievement of MSE from the start of zilucoplan treatment was 36.7 weeks, with some patients achieving MSE at Week 1

Once MSE was achieved, patients maintained their MSE response for a median of 80.8% of time, up to 120 weeks of zilucoplan treatment

Patients receiving zilucoplan experienced sustained MSE over long-term treatment

Abbreviations: Ab+, antibody positive; AChR, acetylcholine receptor; C5, component 5; CI, confidence interval; gMG, generalized myasthenia gravis; MG, myasthenia gravis; MG-ADL, Myasthenia Gravis Activities of Daily Living; MGFA, Myasthenia Gravis Foundation of America; mITT, modified intent-to-treat; MSE, minimal symptom expression; OLE, open-label extension; QMG, Quantitative Myasthenia Gravis; SD, standard deviation; TEAE, treatment-emergent adverse event; Wk, Week.

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