One-Year Single-Center Experience with Efgartigimod in Anti-AChR Positive Generalized Myasthenia Gravis: From Clinical Response to Quality of Life

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Background and Objectives

This retrospective one-year follow-up study aimed to evaluate the efficacy and safety of Efgartigimod in patients with anti-acetylcholine receptor (AChR) antibody-positive generalized Myasthenia Gravis (gMG). The objectives included assessing sustained clinical improvement, reduction in hospitalization and rescue therapy, and enhancement of quality of life.

Materials and Methods

Five patients with confirmed anti-AChR-positive gMG received Efgartigimod intravenously or subcutaneously according to clinical indications. Clinical assessments included MG-ADL and MGC scores before and after each cycle, and FSS and MG-QoL15r at baseline, 6 and 12 months. A total of five patients were initially enrolled; however, only four were included in the final analysis because one patient discontinued treatment due to lack of efficacy (dropout).

Results

Patients demonstrated progressive improvement in MG-ADL and MGC scores over time, as shown in the figure below. The downward trend reflects a clinically meaningful reduction in disease burden across treatment cycles. FSS and QoL15r scores suggested stabilization or mild improvement in fatigue and quality of life.

Outcome	Timepoint	Median ± SD (p-value)
MG-ADL	Week 1 (Cycle 1)	-2.00 ± 2.94 (p=0.285)
	Week 2 (Cycle 1)	-5.00 ± 0.50 (p=0.125)
	Week 3 (Cycle 1)	-6.50 ± 1.41 (p=0.125)
	Week 4 (Cycle 1)	-6.50 ± 1.41 (p=0.125)
	After cycle 1	-5.00 ± 2.63 (p=0.125)
	After cycle 2	-6.00 ± 2.22 (p=0.125)
	After cycle 3	-3.50 ± 0.71 (p=0.500)
MCG	After cycle 1	-4.50 ± 0.96 (p=0.125)
	After cycle 2	-4.50 ± 0.96 (p=0.125)
	After cycle 3	-5.50 ± 0.71 (p=0.500)

MG-ADL and MCG scores (median ± SD) over time in efgartigimod-treated patients.

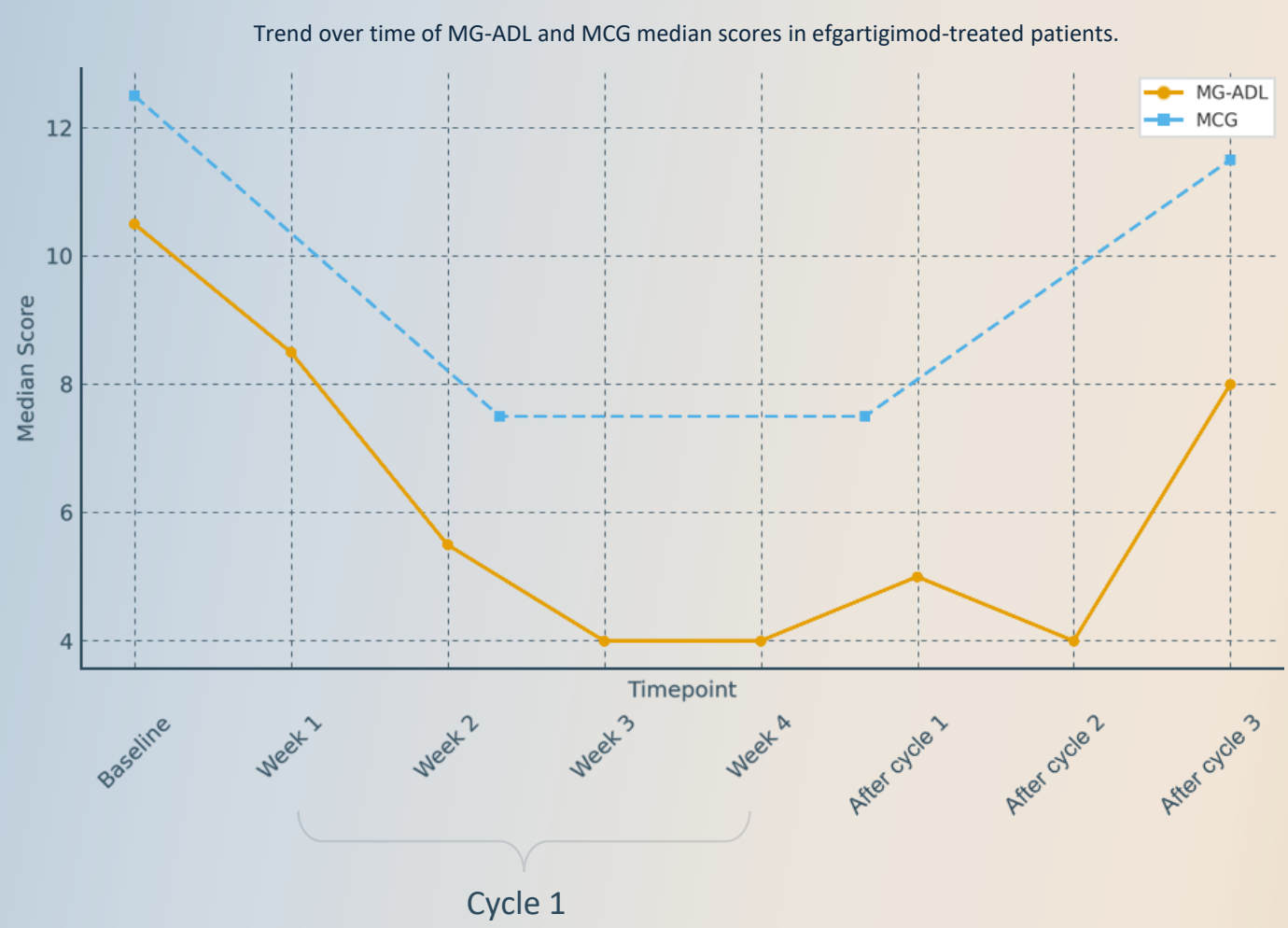
Discussion

Although statistical significance was not reached, this may be explained by the small sample size (n=4). Nevertheless, the consistent clinical improvement observed in MG-ADL and MGC supports the therapeutic benefit of Efgartigimod in generalized MG. The treatment was well tolerated and associated with fewer hospitalizations and reduced need for rescue therapies.

Conclusions

Efgartigimod proved effective and well tolerated, with no adverse effects reported. Despite the lack of statistical significance—likely related to the small sample size—the results confirm a clear trend toward clinical and patient-reported improvement, reinforcing its favorable efficacy and safety profile in anti-AChR-positive generalized MG.

Trend over time of MG-ADL and MCG median scores in efgartigimod-treated patients.



References
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