A Real-Life Experience with Zilucoplan in AChR-seropositive Generalized Myasthenia Gravis

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BACKGROUND AND OBJECTIVES

Generalized Myasthenia gravis (gMG) is a rare chronic autoimmune disease affecting the postsynaptic membrane of the neuromuscular junction. New treatment options, such as the C5 inhibitors, are growing with promising results. In this observational and retrospective study we investigated the efficacy and safety of Zilucoplan in a real life clinical setting.

MATERIALS AND METHODS

Zilucoplan was administered by daily subcutaneous self-administered injection. Efficacy was assessed by MGFA-PIS, MG-ADL, MGC, QMG scales and MG-QOL-15 questionnaire before the start of the treatment and after 1, 4, 12, 24 and 48 weeks. Incidence of treatment-emergent adverse events and prednisone dosages were collected at each time point.

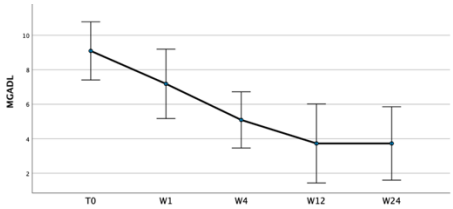
RESULTS

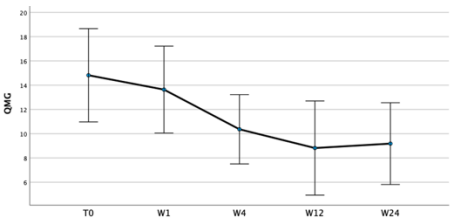
13 patients (11 females, mean age 54.4 y) affected by AChR-seropositive gMG received Zilucoplan with a mean 35.1 weeks of follow-up. A clinical reduction in MG-ADL, QMG, MGC and MG-QOL-15 scores was observed from W4 to W12 and sustained at W24 of follow-up. Compared to baseline, the mean MG-ADL score decreased by 1.9 points at Week 1 and 5.36 points at Week 24. Regarding QMG, an average reduction of 6 point was observed at week 12 and 5.64 point at week 24. An MG-ADL responder rate of 84.6% at W12 and 72.7% at W24 was reported. Minimal symptoms expression (MSE) was obtained in 30.8% at W12 and 18.9% at W24. Prednisone dosage showed a decreasing trend, with a mean reduction of 1.54 mg at W24, however this reduction did not reach statistical significance. Mild transitory adverse events were reported in 61.5% with discontinuation in 23% of patients.

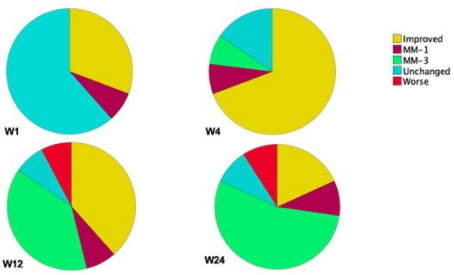
DISCUSSION AND CONCLUSIONS

A meaningful effect was demonstrated in the first 24 weeks of treatment on MG-ADL, MGC, MG-QOL-15 and QMG, showing rapidity in onset of action since the first week and sustained response after 24 weeks and obtaining MSE in more patients. This treatment was very easy to administer with a favourable safety profile. Zilucoplan was well-tolerated and effective in most patients in any subtypes of AChR-positive gMG. Further studies with a broad population of patients and more prolonged follow-up are needed to confirm these preliminary data from real-life experience.

Clinical variable	Total (N=13)
Age (years old)	54.4 (16.8)
Age at onset	44.9 (19.4)
Sex (female %)	11 (85%)
Body Weight (Kg)	69 (15.1)
Disease duration (years)	9.5 (7.1)
Previous thymectomy (n, %)	6 (46%)
Diagnosis of thymoma (n, %)	2 (15.4%)
Comorbidity (n, %)	13 (100%)
Number of comorbidities	3.1 (1.1)
Treatment refractory (n, %)	9 (69%)
Prednisone dose at baseline (mg/day)	12.6 (7.6)
MG-ADL at baseline	9.7 (2.7)
QMG at baseline	15.9 (5.9)
MGC at baseline	16.8 (7.0)
MG-QOL-15 at baseline	18.1 (7.3)
Hospitalizations previous year (n, %)	9 (69%)
Rescue therapy previous year (n, %)	9 (69%)







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