EFGARTIGIMOD IN IVIG REFRACTORY OR INTOLERANT IN ACHR-SEROPOSITIVE GENERALIZED MYASTHENIA GRAVIS

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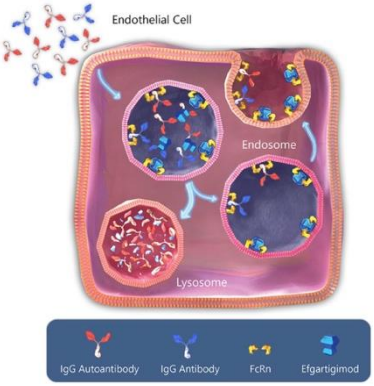

Background and objectives:

Generalized Myasthenia gravis (gMG) is a rare chronic autoimmune disease affecting the postsynaptic membrane of the neuromuscular junction. New treatment options, such as the FcRn inhibitors, are growing with promising results. In this study we investigated the real-world use of Efgartigimod (EFG) for AChR-positive gMG after IVIg failure due to inefficacy or intolerance.

Material and methods:

EFG was administered by 4 weekly intravenous infusions at 10 mg/kg. Efficacy was assessed by MG-ADL and QMG scales at baseline and after 4 weeks.

Results:

13 patients (7 males, mean age 52.9 y) received EFG after IVIg. All the patients included in the study had received at least one intravenous administration of immunoglobulin (IVIg) before Efgartigimod as treatment for gMG: in particular, 9 (70%) patients did not achieve a significant clinical response following IVIg, while AEs after IVIg were experienced by 4 (30%) patients (uncontrollable vomiting and headache, hypokalemia, hemolytic jaundice, severe hemolytic anemia). We observed a clinical meaningful and significant reduction of MG-ADL and QMG scores at W4 of follow-up. We observed a mean reduction in MG-ADL score of 4.0 (SD 3.5) and in QMG score of 3.5 (SD 1.9). The MG-ADL responder rate (MG-ADL reduction >2) was 84.6%, while 69.2% on QMG (QMG reduction >3). Minimal symptoms expression was obtained in one patient with a mean reduction of the daily prednisone dose of 6.9 mg. In terms of safety, 92.3% of patients treated with EFG reported no AE.

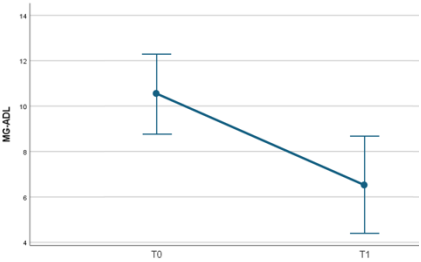

Figure 1. Mean reduction in MG-ADL score.

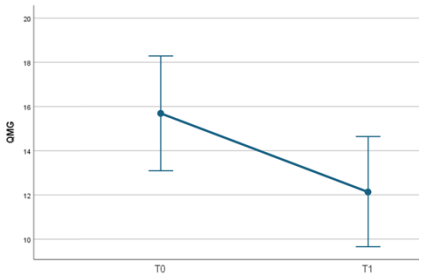

Figure 2. Mean reduction in QMG score.

Discussion and conclusion:

We found a clinical meaningful effect of EFG after IVIg on MG-ADL, and QMG scores with a favorable safety profile. This is an important result because the cohort studied presented high rate of comorbidity, long duration of the disease and they had already failed a rescue therapy. On this perspective, EFG appears to be a good candidate for gMG patients with high comorbidity burden, maintaining a good safety even in unfavourable settings. Further studies with a broader population are needed to confirm these preliminary data from real-life.

References: Saccà F, Pane C, Espinosa PE, Sormani MP, Signori A. Efficacy of innovative therapies in myasthenia gravis: A systematic review, meta-analysis and network meta-analysis. Eur J Neurol. 2023 Dec;30(12):3854-3867. Howard JF Jr, Bril V, Vu T, Karam C, Peric S, Margania T, Murai H, Bilinska M, Shakarishvili R, Smilowski M, Guglietta A, Ulrichts P, Vangeneugden T, Utsugisawa K, Verschuuren J, Mantegazza R; ADAPT Investigator Study Group. Safety, efficacy, and tolerability of efgartigimod in patients with generalised myasthenia gravis (ADAPT): a multicentre, randomised,, phase 3 trial. Lancet Neurol. 2021 Jul;20(7):526-536. placebo-controlled