Subcutaneous Immunoglobulin in Myasthenia Gravis: Results of a North American Open Label Study

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Objective:
Objectives: To evaluate the efficacy, safety and tolerability of subcutaneous immunoglobulin (SClg) in the treatment in myasthenia gravis (MG) patients who are on IVlg as part of routine clinicalcare.

Background:
Background: IVlg has been demonstrated to improve the Quantitative MG (QMG) score as compared to placebo and in another study to be efficacious in comparison to plasma exchange. In routine care, SClg might be easier to administer than IVlg.

Design/Methods:
Methods: This multi-center North American open label prospective investigator-initiated study had 2 components: IVlg Screening Phase (ISP; Weeks -10 to -1) followed by Experimental Treatment Phase (ETP; Weeks 0 to 12). We hypothesized that more than 65% of the patients entering the ETP would have stable QMG score (primary outcome) at Week 12. We recruited 23 patients in the ISP and 22 entered the ETP. 12(54.5%) were females and 18(78%) were white, with mean age 51.4 years±17. We had complete ETP QMG data on 19/22; one subject withdrew from ISP owing to worsened condition and two subjects withdrew before Week 4 (needle dislike). The per protocol primary statistical analysis was conducted for n=22 subjects using a one-sided z-test of proportions at the 5% significance level. Sensitivity analyses were conducted using a cohort of n=22 subjects using ‘worst-case’ imputation scenario as well as post hoc analysis.

Results:
Findings: On primary analysis, 19/22 (86.4%; 95% CI:0.72–1.00)were treatment “successes” (p=0.018). Sensitivity analysis using the ‘worst-case’ imputation resulted in 17/22 (77.3%;0.60–0.95) declared as treatment success (p=0.114).Post hoc analysis of the primary outcome confirmed treatment success in 17/20 (85%;0.69– 1.00) (p=0.0304). There was no difference in the secondary outcome measures though MG composite was better at Week 12. SClg was safe and well tolerated in this population.

Conclusions:
Conclusion: Most MG patients who were doing well on IVlg maintained disease stability for another 12 weeks once transitioned to SClg.