Retrospective Study of Efficacy and Safety of Rozanolixizumab-noli in Patients with Generalized Myasthenia Gravis in the Home-Infusion Setting

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Background

- Generalized Myasthenia Gravis (gMG) is a rare, chronic, autoimmune neuromuscular transmission disorder characterized by IgG antibody-mediated attacks on the neuromuscular junction components of acetylcholine receptors (AChR) or muscle-specific tyrosine kinase (MuSK) receptors.
- The disruption leads to diminished muscle contraction and significant impairment in daily function.
- Rozanolixizumab-noli is a humanized IgG4 monoclonal antibody that targets the neonatal Fc receptor (FcRn), resulting in the reduction of circulating IgG.
- It is indicated for the treatment of adult patients with anti-AChR or anti-MuSK antibody positive (Ab +) gMG and is the first targeted therapy for anti-MuSK AB + gMG.
- This home infusion company is currently one of only three distributors in the United States that dispenses Rozanolixizumab-noli.
- As the inaugural targeted therapy for anti-MuSK AB + gMG, evaluating its efficacy and safety is crucial in addressing the unmet needs of non-responsive patients.

Study Objective

- Evaluate the clinical efficacy and safety of rozanolixizumab-noli in patients with AChR Ab+ and MuSK Ab+ gMG in the home infusion setting.

Methods

- Retrospective multi-center analysis utilizing electronic medical records on all current and discharged patients from 5 pharmacy branches from this home infusion company that dispensed rozanolixizumab-noli from August 01,2023-January 31, 2024.
- **Primary endpoint:** Clinical meaningful experience based on Myasthenia Gravis-Activities of Daily Living (MG-ADL) of ≥2.
- **Secondary endpoints**: Average change in baseline MG-ADL score at end of treatment (1 dose weekly for 6 weeks).
- Safety data will be evaluated on treatment-emergent adverse events (TEAEs) and those that discontinued due to TEAEs.

Table 1: Patient Criteria

Inclusion Criteria	Exclusion Criteria
gMG aged ≥18 years	Patients who canceled service prior to administration of the first dose
AChR or MuSK Ab +	Those lacking assessments or records evaluating their response to therapy
Baseline MG-ADL score ≥3	Patients that missed their weekly dose by more than 4 days after the scheduled time point
Received at least 1 dose of Rozanolixizumab-noli	

Table 1: Patient Demographics

Demographics	
Total Patients, n	33
Age, years, mean (range	66.2 (13.5)
Sex, male, n (%)	18 (55)
MG-ADL score at baseline, mean (SD)	9.2 (4.1)

Table 2:TEAEs

TEAEs % (n=33)	
Any TEAEs	63.6 (21)
Mild/Moderate	42.4 (14)
Serious	12.1 (4)
Treatment withdrawal due to TEAEs	9.1 (3)

Figure 1: Most Common TEAEs

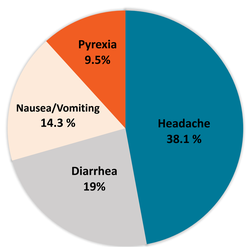
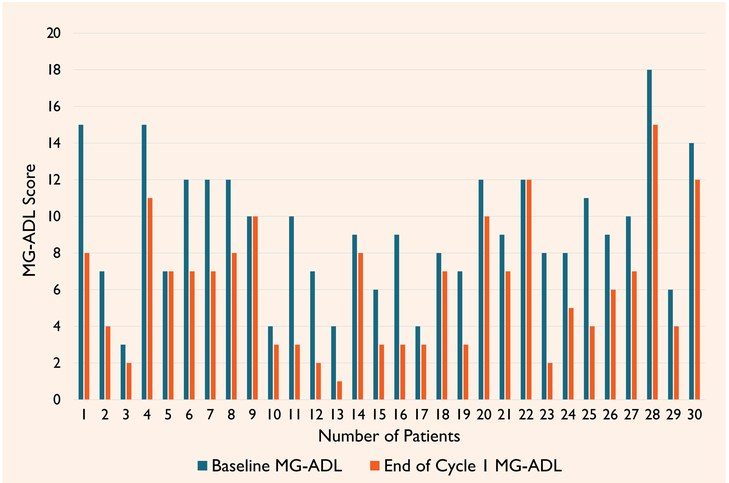


Figure 2: Comparison of MG-ADL Score at Baseline and End of Cycle



Results

Efficacy

- At the end of cycle 1, 70% (21/30) of patients had a clinical improvement in symptoms based on MG-ADL of ≥2.
- The average change from baseline to end of treatment was a reduction in MG-ADL of 3.03.

Safety

- The majority of patients did experience TEAEs at some point during treatment.
- The majority of TEAEs were mild/moderate in intensity.
- 12.1% patients that did endure serious TEAEs still completed 6 doses while 9.1% of patients discontinued treatment.

Discussion

- This data further substantiates the therapeutic benefit of targeting anti-MuSK AB + in the treatment of gMG.
- The observed positive response, and the overall well-tolerated nature of the treatment, not only provides valuable insights into the potential benefits for gMG patients but also signals a noteworthy qualitative and quantitative improvement in their conditions.
- Limitations include incomplete assessments.
- Further studies are imperative to establish a more thorough analysis to understand the long-term benefits.

Conclusions

- The findings from this study underscore the promising efficacy and safety profile of rozanolixizumab-noli as a viable treatment option for generalized Myasthenia Gravis (gMG).
- This research contributes to the growing body of knowledge in gMG therapeutics and sets the stage for continued investigation into the sustained effectiveness of rozanolixizumab-noli in improving lives.

References

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