

Vitaccess Real™ Myasthenia Gravis studies protocol: Registry-based mixed-methods studies on treatment outcomes in patients initiated on zilucoplan or rozanolixizumab in the real world

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Background

- Zilucoplan (complement C5 inhibitor) and rozanolixizumab (neonatal Fc receptor blocker) are targeted therapies that have demonstrated efficacy in phase 3 trials in gMG.¹⁻⁴
- However, real-world evidence remains limited and is needed to characterize treatment outcomes, utilization and patient experience in routine clinical practice.

Objective

To characterize real-world clinical outcomes and patient experience in adults with gMG initiating zilucoplan or rozanolixizumab.

Study design

The Vitaccess Real™ MG REALITY studies employ a registry-based **concurrent embedded mixed-methods** study design with quantitative and qualitative components (Figure 1).

Data are collected from multiple sources within the registry, including:



Qualitative interview data will complement quantitative outcomes, providing richer insight into patients' experiences, treatment satisfaction, and the real-world impacts of these therapies. See Table 1 for study outcomes.

Study population

Quantitative cohort

- Adults (≥18 years)
- With AChR or MuSK positive gMG
- Enrolled in the VRMG registry
- Residing in North America or Europe (Figure 2)
- Initiated zilucoplan or rozanolixizumab

Qualitative cohort

A subset of treated participants will be eligible for qualitative interviews.

Participants are recruited into the VRMG registry via:



Figure 2. Site locations currently recruiting for the VRMG registry used in the REALITY studies (subject to expansion)



Data analysis

Quantitative component

- Descriptive statistics for baseline characteristics.
- Longitudinal analyses from baseline over 12 months using mixed models for repeated measures of change, adjusted for key baseline covariates.
- Treatment utilization, medication use and HCRU summaries at 6 and 12 months.
- Subgroup and sensitivity analyses where feasible.

Qualitative component

- Thematic analysis using Braun and Clarke's framework.⁵

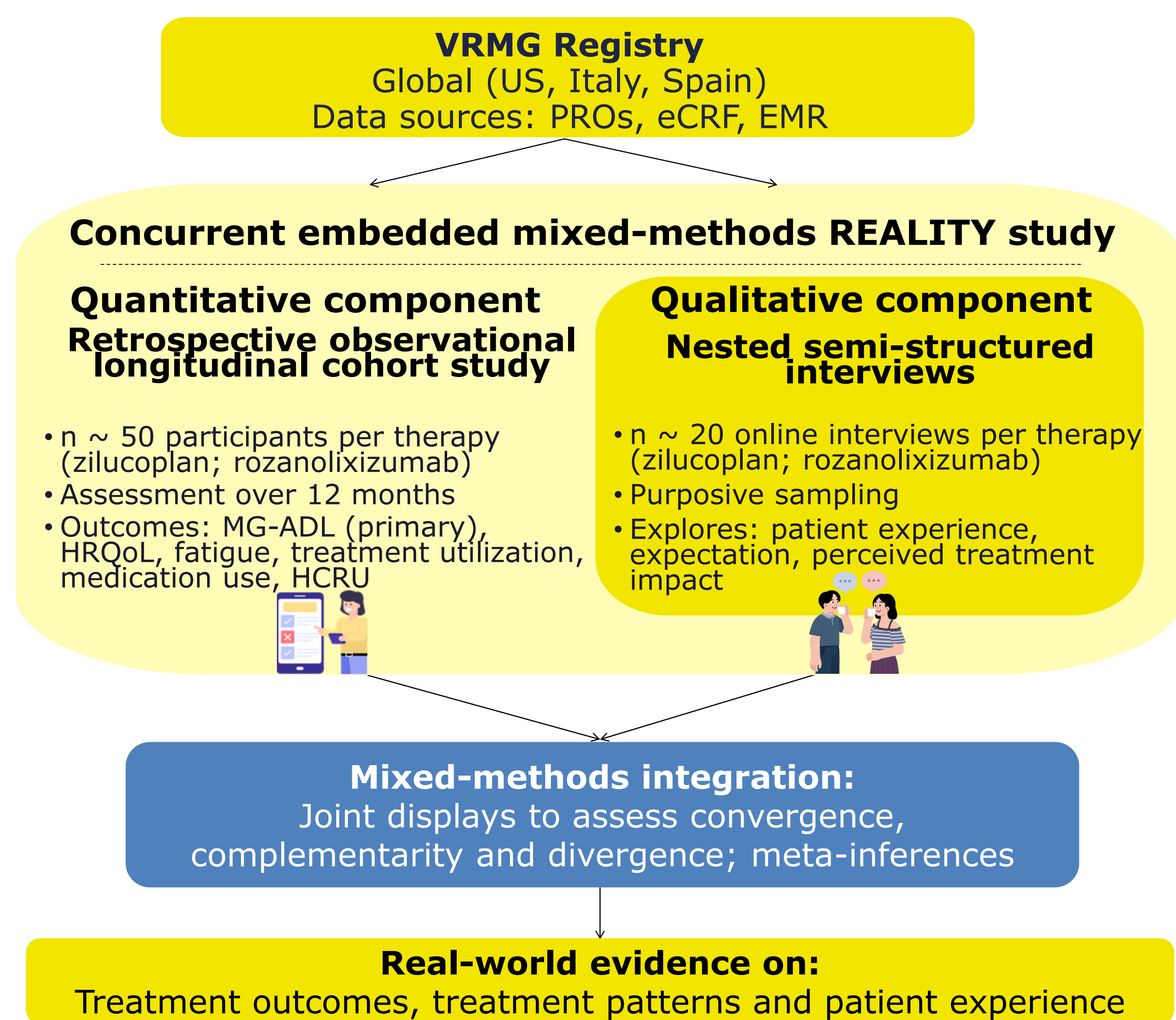
Integration

- Joint displays to assess convergence, complementarity and divergence.

Conclusion

- This registry-based mixed-methods design will provide a patient-centric understanding of real-world treatment outcomes in adults with gMG initiating zilucoplan or rozanolixizumab.
- By integrating longitudinal quantitative outcomes with in-depth qualitative patient experience data, the study will capture treatment utilization patterns, medication use, HCRU, PROs and patients lived experiences of, and perspectives on, the treatment.
- This approach is particularly valuable in rare diseases such as gMG, where real-world evidence can complement clinical trial data and support more informed, patient-centered care and decision-making.

Figure 1. Registry-based mixed-methods study design



Outcomes

Table 1. Registry-based mixed-methods study outcomes

Category	Outcomes	Assessment timing
Baseline characteristics	Demographics, diagnostic and clinical characteristics, medical history and prior treatment history	Baseline
Primary outcome	Change from baseline in MG-ADL total score	Baseline over 12 months
Other PROs	Change from baseline: • MG-QoL-15r total score • MG Symptoms PRO Physical Fatigue Scale total score • NeuroQoL Fatigue SF total score	Baseline over 12 months
Treatment utilization patterns	• Treatment utilization (duration, discontinuation) • Administration (method [rozanolixizumab only], frequency)	Baseline, 6 and 12 months
Medication use	• Oral steroids • Non-steroidal immunosuppressants • Rescue therapies	Baseline, 6 and 12 months
HCRU	Healthcare visits and hospitalizations by setting (clinic or outpatient; emergency visits; follow up and results; home visits; hospital inpatient; telemedicine appointments), including gMG exacerbation-related utilization, and duration and length of stay	Baseline, 6 and 12 months
Qualitative outcomes	Lived experience, perceived changes since treatment initiation, expectations, satisfaction and burden	June to November 2026

Results

- Recruitment began in August 2024. Registry progress (August 2026):
 - 11 clinical sites recruiting, 250 patients enrolled.
 - Currently 10 eligible participants either receiving zilucoplan or rozanolixizumab.
- **Quantitative component:** Interim analysis planned following achievement of approximately 50% of the target sample size.
- **Qualitative component:** Qualitative interviews are planned between June and November 2026, with findings expected to be integrated with the quantitative results in the final study reporting.

Abbreviations:

AChR, acetylcholine receptor; eCRFs, electronic case report forms; EMRs, electronic medical records; gMG, generalized myasthenia gravis; HCRU, healthcare resource utilization; HRQoL, health-related quality of life; MG-ADL, Myasthenia Gravis Activities of Daily Living; MG-QoL-15r, Myasthenia Gravis Quality of Life 15-item Revised; MG Symptoms PRO Fatigue Scale, Myasthenia Gravis Symptoms Patient-Reported Outcome Physical Fatigue Scale; MuSK, muscle-specific kinase; NeuroQoL Fatigue SF, Quality of Life in Neurological Disorders Short Form – Fatigue; NS-IST, non-steroidal immunosuppressive therapy; PRO, patient-reported outcome; VRMG, Vitaccess Real™ Myasthenia Gravis

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