Real-World Treatment Patterns, Healthcare Resource Utilization, and Unmet Needs in Generalized Myasthenia Gravis: A Retrospective Analysis of German Statutory Health Insurance Claims

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Background

- Generalized myasthenia gravis (gMG) is a rare, chronic autoimmune neuromuscular disorder causing fluctuating and potentially life-threatening muscle weakness.[1-4]
- Advanced therapies - neonatal Fc receptor (FcRn) blockers, complement C5 inhibitors and off-label anti-CD20 agents - have substantially expanded the treatment landscape in recent years.[5-8]
- Understanding treatment patterns and outcomes in routine German practice is critical to assess whether current therapies adequately address the long-term burden of gMG and to identify areas where unmet need persists.[4,5,9]

Objective:

To describe real-world treatment patterns, healthcare resource utilization (HCRU) and proxies of disease control among adults with gMG receiving advanced therapies in Germany.

Methods

- Retrospective cohort study using the InGef research database of anonymized German statutory health insurance claims (2016-2024).
- Adults with gMG diagnosed by a neurologist and treated with an advanced therapy were included (N=144).
- Baseline characteristics, comorbidities and prior treatments were assessed over the 12 months before index (start of the advanced therapy).
- Follow-up ran from initiation of the advanced therapy until database end, death or treatment switch.
- Time-to-first-event analyses assessed gMG exacerbations, rescue therapy (IVIg, plasma exchange or immunoadsorption), high-dose corticosteroid use and treatment switching.
- All-cause and MG-related HCRU (hospitalizations and outpatient visits) was summarized overall and by therapy class: FcRn blockers (efgartigimod, rozanolixizumab), C5 inhibitors (eculizumab, ravulizumab, zilucoplan) and anti-CD20 (rituximab).

Limitations:

- Claims data capture treatment dispensing and diagnoses but do not include clinical severity measures; therefore, treatment effectiveness could not be assessed directly.
- Analyses with fewer than 5 patients were not presented in accordance with data privacy requirements.
- Follow-up duration varied across treatment cohorts and should be considered when interpreting results

Results

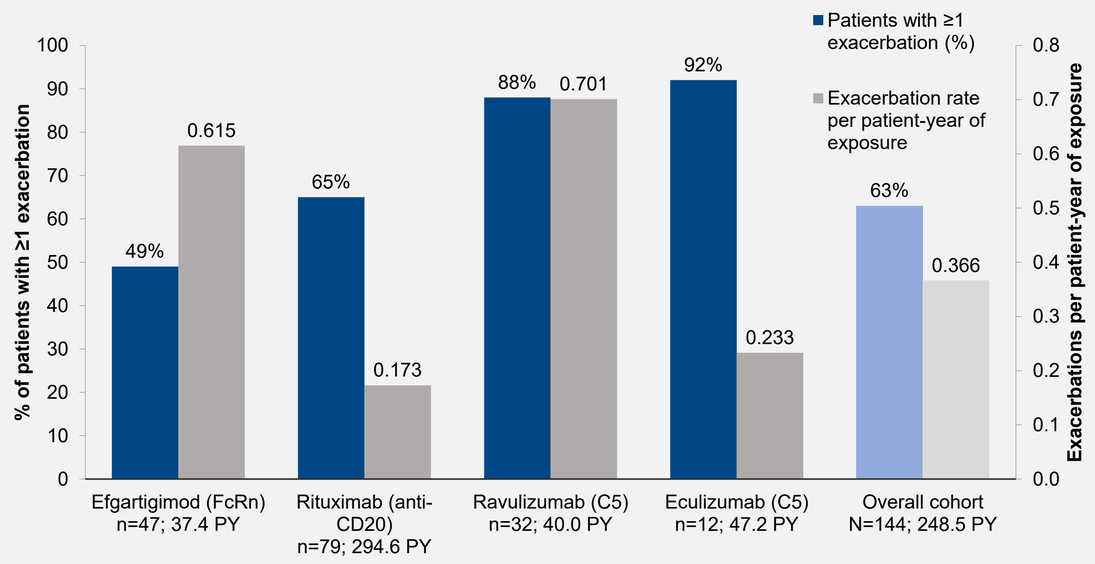
- Among 144 adults with gMG initiating an advanced therapy (mean age 56 years, 57% female), disease burden was already high before initiation: 91% had received pyridostigmine, 67% corticosteroids, 54% had experienced at least one exacerbation and 43% had required at least one rescue intervention in the prior year. Off-label rituximab was the most frequently used therapy (n=79), ahead of FcRn blockers (n=47) and C5 inhibitors (n=44). Despite treatment, exacerbations, rescue therapy and hospitalization remained frequent throughout follow-up (Table 1).

Table 1. Baseline Characteristics and Pre-Index Disease Burden (N=144)

Baseline characteristic (12 months pre-index)	n	%
Age, years - mean (SD)	55.9 (16.2)	-
Female sex	82	57%
Charlson Comorbidity Index - median (IQR)	2.5 (1-6)	-
Pyridostigmine	131	91%
Glucocorticoids	96	67%
At least one exacerbation	78	54%
At least one rescue treatment (IVIg/PLEX/IA)	62	43%

- Patients had already accumulated substantial disease burden before starting an advanced therapy: more than half had experienced an exacerbation and over 40% had required rescue treatment in the preceding year (Table 1).
- However, nearly two-thirds of patients experienced at least one gMG exacerbation during follow-up, highlighting persistent disease burden despite advanced therapies and suggesting that unmet need remains in achieving sustained disease control in gMG (Figure 1). Within the overall cohort, the median time to first exacerbation was 4.9 months.

Figure 1. Patients With At Least One gMG Exacerbation During Follow-Up, by Advanced Therapy



Rozanolixizumab and zilucoplan are not shown (n<5 per class). Percentages of patients with at least one exacerbation at any point during observed follow-up.

- Hospitalization was near-universal after starting treatment, and MG-related admissions and outpatient visits remained frequent across every therapy group (Table 2).

Table 2. Healthcare Resource Utilization During Follow-Up, Overall and by Therapy

Healthcare resource use	Overall (N=144)	Efgartigimod (n=47)	Eculizumab (n=12)	Rituximab (n=79)
≥1 all-cause hospitalization, n (%)	132 (92%)	36 (77%)	11 (92%)	77 (97%)
All-cause hospitalizations, median (IQR)	6 (2-12)	2 (1-5)	19 (12-24)	9 (4-15)
≥1 MG-related hospitalization, n (%)	104 (72%)	31 (66%)	11 (92%)	54 (68%)
MG-related hospitalizations, median (IQR)	4 (0-8)	1 (0-3)	17 (11-19)	5 (0-10)
≥1 outpatient visit, n (%)	120 (83%)	34 (72%)	11 (92%)	74 (94%)

HCRU is not adjusted for the differing follow-up duration between therapies (median 9.5 months for efgartigimod vs 44.7 months for rituximab).

- Exacerbations recurred within months of starting an advanced therapy, and roughly a quarter of patients still required rescue therapy or high-dose corticosteroids during follow-up (Table 3).

Table 3. Time-to-First-Event Outcomes From Start of Advanced Therapy

Outcome during follow-up	Patients with event, n (%)	Median time to first event, months (95% CI)
gMG exacerbation	91 (63%)	4.9 (4.2-6.5)
MG-related hospitalization	104 (72%)	0.5 (0.4-2.0)
High-dose corticosteroid use	37 (26%)	Not reached
Rescue therapy (IVIg/PLEX/IA)	34 (24%)	Not reached
Mechanical ventilation	9 (6%)	Not reached
Myasthenic crisis	0 (0%)	Not reached

Key takeaway

Advanced therapies were started late in the disease course, after exacerbations (54%) and rescue treatment (43%) in the prior year.

Despite treatment with advanced therapy, 63% patients had at least one exacerbation, with a median time to first event of about 5 months. In addition, 72% had at least one MG-related hospitalization and 24% still required rescue therapy during follow-up.

Outcomes differed by therapy. When factoring in the exposure time, exacerbations seem to be more frequent among ravulizumab (0.7 per patient-year) and efgartigimod (0.6 per patient-year) recipients.

Conclusions

In routine German care, adults with gMG treated with advanced therapies continued to experience frequent exacerbations, rescue therapy use, high-dose corticosteroid exposure and substantial hospitalization.

These findings underline a persistent unmet need for treatments that deliver sustained disease control and reduce the healthcare burden of gMG in real-world practice.

Abbreviations

anti-CD20, anti-cluster of differentiation 20; C5, complement component 5; CI, confidence interval; FcRn, neonatal Fc receptor; gMG, generalized myasthenia gravis; HCRU, healthcare resource utilization; IA, immunoadsorption; InGef, Institute for Applied Health Research Berlin; IQR, interquartile range; IVIg, intravenous immunoglobulin; PLEX, plasma exchange; SD, standard deviation.

Disclosures

This study used the InGef research database of anonymized German statutory health insurance claims.

All named authors met authorship criteria and approved the final content of this poster.

Wisam Karmous, Gonçalo Rodrigues, Wim Noel and Tom Denee are employees of Johnson & Johnson and may hold stock in Johnson & Johnson.

Moritz Lehne was an employee of Cytel Inc. at the time of the analysis. Anja Cengia is an employee of the Institute for Applied Health Research Berlin (InGef) GmbH.

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