

Poster Number	Poster Title	Authors	Abstracts
1	INVESTIGATING THE PREVALENCE OF MYASTHENIA GRAVIS AND PARKINSON DISEASE CO-OCCURRENCE IN THE UNITED STATES IN 2022	Sarah Abdul-Ghani, Saige Fong, J. Douglas Miles	<p>INTRODUCTION: Myasthenia gravis (MG) is an autoimmune disorder of the neuromuscular junction, while Parkinson disease (PD) is a progressive neurodegenerative condition of the central nervous system. The prevalence of both conditions has increased in the United States over recent decades. Although case studies suggest a possible connection between MG and PD, their co-occurrence has not yet been quantified on a large scale.</p> <p>OBJECTIVE: This study investigates the prevalence of MG and PD co-occurrence among hospitalized patients using the National Inpatient Sample (NIS) database.</p> <p>METHODS: A retrospective analysis was conducted using the 2022 NIS database. Chi-squared analysis was performed to assess whether the observed MG and PD co-occurrence deviated from expected values under the assumption of independence. Binomial logistic regression was used to evaluate the association while adjusting for confounders, including age and sex. Odds ratios (OR) with 95% confidence intervals (CI) were reported. Statistical significance was set at $p < 0.05$.</p> <p>RESULTS: The chi-squared test indicated that MG and PD co-occurred significantly more than expected ($\chi^2 = 948.5$, $df = 1$, $p < 0.001$). The observed frequency of MG-PD co-occurrence was 1,175, compared to an expected 494 cases. Logistic regression confirmed that PD (OR = 1.33, 95% CI: 1.25–1.41, $p < 0.001$), age (OR = 1.034, $p < 0.001$), and sex (OR = 0.873, $p < 0.001$) were significant predictors of MG.</p> <p>SUMMARY/CONCLUSION: MG and PD co-occur at a higher-than-expected frequency among hospitalized patients. This association remains significant after adjusting for age and sex, warranting further research.</p>
2	RELATIONSHIP BETWEEN DISEASE BURDEN AND HEALTHCARE EXPENDITURE AMONG PATIENTS WITH GENERALIZED MYASTHENIA GRAVIS (GMG) IN THE UNITED STATES: A RETROSPECTIVE DATABASE ANALYSIS	James F. Howard Jr., Seth Anderson, Joseph Tkacz, Kathleen Wilson, Jill Schinkel, Dustin Cavida, Blanca Canales, Kristina Patterson	<p>INTRODUCTION: There is a dearth of real-world evidence assessing HCRU-related outcomes associated with an aging gMG population with more current data and by gMG disease severity.</p> <p>OBJECTIVE: To assess healthcare resource use and costs by gMG severity.</p> <p>METHODS: This was a retrospective database analysis of the Medicare Fee-for-Service and MORE2 Registry® of commercial, Medicare Advantage, and Managed Medicaid claims spanning 1/1/2018-6/30/2024. Inclusion criteria were as follows: 1) ≥ 1 inpatient or ≥ 2 outpatient claims with diagnosis of gMG (earliest diagnosis=index date), 2) 12 months of health plan enrollment preceding (baseline) and following (follow-up) the index date, 3) age 18+ years on index, and 4) absence of ocular MG. Patients were segmented into subgroups based on the presence of a gMG exacerbation or the presence of a myasthenic crisis in follow-up. Per-patient-per-month (PPPM) healthcare resource utilization and costs were calculated for all patients during the 12-month follow-up period.</p> <p>RESULTS: A total of 44,525 patients with gMG were included. The mean±SD age of the sample was 71.2±14.2 years; 50.6% of the sample were male. Mean PPPM total healthcare costs for the full sample were \$4,765±\$11,132, which were over two- and three-fold greater for patients with ≥ 1 exacerbation ($n=6,817$) (\$9,781±\$16,032) and ≥ 1 myasthenic crisis ($n=530$) (\$16,371±\$18,990), respectively.</p> <p>SUMMARY/CONCLUSION: This real-world analysis demonstrates the substantial economic burden associated with uncontrolled gMG within the U.S., which is notably greater among severe patients. Results highlight the unmet need within the current gMG treatment landscape, as additional therapeutic options may be warranted to better manage the disease, and associated costs.</p> <p>INTRODUCTION: Lambert Eaton Myasthenic Syndrome (LEMS) is a rare neuromuscular autoimmune disease that causes progressive muscle weakness and fatigue. Amifampridine phosphate (3,4-DAPP) is a potassium channel blocker approved for the treatment of LEMS, with a recommended sequential and time-dependent dose titration at initiation.</p> <p>OBJECTIVE: This Real-World retrospective analysis aimed to describe the time to achieve a stable dose of amifampridine phosphate (3,4-DAPP).</p> <p>METHODS: Data for this analysis were sourced through a specialty pharmacy. This analysis included patients initiating 3,4-DAPP between 1/2019 to 1/2025. Data were analyzed descriptively for patient demographics, diagnosis, dose at initiation, subsequent titration, and time to stable dose at 1, 2, 3, and 6 months from initiation. Stable dose was defined as the same continuous dose for 6 months.</p> <p>RESULTS: During the evaluated time-period, 444 LEMS patients (57% female) with a mean age of 63 years, receiving on-label 3,4-DAPP at initiation (mean dose 33.2 mg/day) were titrated overtime to a final mean dose of 62.3 mg/day. The proportion of patients reaching their first stable dose of 3,4 DAPP was 12.6%, 35.1%, 50.2%, and 66.7% at 1, 2, 3, and 6 months, respectively.</p> <p>SUMMARY/CONCLUSION: Among LEMS patients receiving 3,4 DAPP, many patients had an extended dose titration period to potentially address symptoms and reach a stable dose. More frequent and early HCP interactions may be needed to improve efficiency in achieving a stable dose and symptom relief. Given the limitations of this retrospective analysis, these findings warrant additional review as new data become available.</p>
3	PATIENT CHARACTERISTICS AND TIME TO STABLE DOSE WITH AMIFAMPRIDINE PHOSPHATE IN THE TREATMENT OF LAMBERT EATON MYASTHENIC SYNDROME IN THE UNITED STATES	Yaacov Anziska, Steven Woods, Olive Buhule, Reid Badgett, Paula Alvarez, Kenneth Ecker	<p>INTRODUCTION:</p> <p>OBJECTIVE: To determine the impact of race and income on myasthenia gravis (MG) disease management and prognosis.</p> <p>METHODS: Utilizing the Myasthenia Gravis Foundation of America (MGFA) Registry, we investigated the association between race and income and MG. Bivariate analysis was performed, focusing on race (white vs. non-white, Hispanic vs. non-Hispanic), and income (those earning <\$50,000 annually vs. >\$50,000). Multivariate logistic regression analysis was completed with three different outcomes: worsening of MG-ADL score by >2 points, disease exacerbation, and a combined endpoint of hospital and intensive care unit admissions.</p> <p>RESULTS: There were no significant differences between whites and non-whites in terms of MG severity, medical treatments used, or hospitalizations. Biologics and PLEX were used only in non-Hispanics (55% vs. 0%), while IVIG was given more often to Hispanics (24% vs. 3%); there were no differences in other medications used or disease outcomes. For those earning less than \$50,000, 33% utilized steroids while 63% of higher earners did, 2.2% of lower earners and 5.3% of high earners received biologics, and 18% of low-earners and 12% of higher earners were given IVIG. Those earning less than \$50,000 had more emergency room visits (0.64 versus 0.37,) and worsening MG symptoms (33% versus 19%).</p> <p>SUMMARY/CONCLUSION: Lower income and Hispanic patients received fewer biologics and more IVIG, while lower-income patients had less-controlled disease. There was no difference in terms of hospitalizations and death for these groups. Further study should focus on identifying reasons for these differences to create ways of delivering high-quality care to these patients.</p>
4	THE IMPACT OF RACE AND INCOME ON MYASTHENIA GRAVIS MANAGEMENT AND OUTCOMES	Yaacov Anziska	<p>INTRODUCTION: Myasthenia Gravis (MG) is a chronic autoimmune disorder characterized by fluctuating muscle weakness. The HumaMG app enables remote real-world data (RWD) collection directly from patients, deployed in the US via a direct-to-patient (D2P) model and in the UK through remote patient monitoring (RPM) at St George's Hospital.</p> <p>OBJECTIVE: To describe user characteristics, assess data completeness and retention patterns across deployment models to evaluate the feasibility of generating RWD.</p> <p>METHODS: A cross-sectional analysis was conducted on app data from 305 out of 386 users (approximately 79%) who consented to data sharing. Demographics, clinical characteristics, treatment data, patient-reported outcomes (PROs), and retention were analyzed descriptively.</p> <p>RESULTS: Among the 305 users who consented, 263 (86.2%) were enrolled via D2P (US) and 42 (13.8%) via RPM (UK). Most users were female (74%), and 34% of patients were aged 55 years or older. Early-onset MG patients accounted for 80% of the users, with double vision (44%) being the most common initial symptom. Approximately half of patients had undergone thymectomy. Initial PROs indicated moderate disease burden (median MG-ADL: 8.0, IQR: 7.0; median EQ-5D-5L: 62.5, IQR: 26.0. 78% entered medication data. Retention at 3 months was higher in the RPM group (57.2% vs 37.5%), suggesting deeper engagement when embedded in clinical care.</p> <p>SUMMARY/CONCLUSION: HumaMG provides valuable insights into the MG patient population and disease burden. Despite the large difference in patient numbers at baseline between D2P and RPM, the latter suggested stronger engagement and retention. This supports the potential of the tool for scalable, high-quality RWD collection.</p>
5	GATHERING REAL-WORLD INSIGHTS FROM PEOPLE LIVING WITH MYASTHENIA GRAVIS: ANALYSIS OF USER CHARACTERISTICS AND ADHERENCE PATTERNS IN PATIENTS USING THE HUMAMG APP	Pablo Garcia-Reitboeck, Erkuden Goikoetxea, Jean-Christophe Steels, Thais Tarancon, Mert Aral, Julie Horan	

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6	STEROID-SPARING BENEFITS OF RAVULIZUMAB IN REFRACTORY MYASTHENIA GRAVIS: A REAL-WORLD RETROSPECTIVE ANALYSIS	Priyanshu Bansal, Shaifali Arora, George Small	
7	MEASURING THE EFFECT OF ROZANOLIXIZUMAB TREATMENT IN THE MYCARIN STUDY USING THE MYASTHENIA GRAVIS IMPAIRMENT INDEX	Carolina Barnett-Tapia, Elena Cortés-Vicente, Robert Pascuzzi, Kimiaki Utsumigawa, Jos Bloemers, Fiona Grimson, Thais Tarancón, Vera Bril	<p>INTRODUCTION: Generalized myasthenia gravis (gMG) is an autoimmune disease characterized by fluctuating muscle weakness. In the MycarinG study (NCT030971422), rozanolixizumab significantly improved myasthenia gravis (MG)-specific outcomes versus placebo in patients with gMG. The MG Impairment Index (MGIi) incorporates patients' perspectives and physician evaluation of MG impairment, comprising 22 patient-reported and 6 examination items.</p> <p>OBJECTIVE: Evaluate the effect of rozanolixizumab on MG symptoms in MycarinG using the MGIi.</p> <p>METHODS: Patients were randomized 1:1.1 to once-weekly rozanolixizumab 7mg/kg, 10mg/kg or placebo for 6 weeks. MGIi was an optional assessment. Exploratory MGIi endpoints included change from baseline (CFB) to Day 43 in total score (range: 0–84) and ocular and generalized subscores (higher scores reflect greater impairment). MGIi responder (≥ 5.5-point improvement) and item-level analyses were conducted post hoc.</p> <p>RESULTS: 200 patients received rozanolixizumab 7mg/kg (n=66), 10mg/kg (n=67) or placebo (n=67). 144/200 (72.0%) completed the MGIi assessment at baseline and Day 43. Mean CFB in MGIi total score was –12.4 with rozanolixizumab 7mg/kg, –16.1 with 10mg/kg and –3.4 with placebo. Mean CFB in the subscores was also greater with rozanolixizumab versus placebo (ocular: –2.9, –5.2 and –1.0, respectively; generalized: –9.5, –10.9 and –2.4, respectively). At Day 43, 57.1%, 83.3% and 40.4% of patients, respectively, were responders. For most items, greater proportions of patients achieved a score of 0 (i.e., symptom absence) with rozanolixizumab than with placebo.</p> <p>SUMMARY/CONCLUSION: These findings further support efficacy analyses from MycarinG, highlighting the benefit of rozanolixizumab in patients with gMG, and demonstrate the utility of the MGIi in evaluating patient-relevant symptoms following treatment. Funding: UCB.</p> <p>INTRODUCTION: Myasthenia gravis (MG) is an antibody-mediated disorder of the neuromuscular transmission driven mainly by immunoglobulin G (IgG) targeting the acetylcholine receptor (AChR). Autoantibodies instigate inflammation and tissue damage through different mechanisms including receptor blockade, internalization and complement activation. While treatments targeting autoantibodies benefit many, a subset of patients remains refractory, highlighting disease heterogeneity and the need for personalized therapies.</p> <p>OBJECTIVE: To evaluate the therapeutic potential of S-1117, a pan-IgG-specific protease, in AChR antibody-mediated pathology and to investigate the contribution of non-IgG autoantibodies to complement activation.</p> <p>METHODS: A suite of live cell-based assays assessing receptor binding, blockade, internalization, and complement activation were conducted using human-derived AChR monoclonal antibodies and MG patient sera before and after cleavage with S-1117 (IgG-specific protease). The pathogenic contribution of AChR-specific immunoglobulin M (IgM) was examined using an IgM-specific cleaving enzyme.</p> <p>RESULTS: S-1117 efficiently cleaved the IgG Fc domain and abrogated autoantibody-mediated complement-deposition. However, in a subset of patients, complement activation persisted despite IgG inactivation. This residual activity was attributed to pathogenic AChR-specific IgM, which either acted synergistically with IgG or served as the primary driver of complement deposition. Treatment with an IgM-specific protease fully eliminated this effect.</p> <p>SUMMARY/CONCLUSION: These findings underscore the therapeutic potential of S-1117 in disabling AChR-IgG Fc-mediated pathogenic functions and reveal a previously unrecognized subset of myasthenia gravis driven by AChR-specific IgM. Dual targeting of IgG- and IgM-mediated mechanisms with isotype-specific proteases offers a novel therapeutic strategy and supports a framework for mechanism-based patient stratification, advancing precision medicine in antibody-mediated MG.</p>
8	IMMUNOGLOBULIN-SPECIFIC PROTEASES DISARM PATHOGENIC ACETYLCHOLINE RECEPTOR-SPECIFIC AUTOANTIBODIES IN MYASTHENIA GRAVIS	Alexandra C. Bayer, Liliana M. Sanmarco, Alex Pellerin, Agustín Plascencia, Jordan M. Anderson, Richard J. Nowak, Gianvivi Masi, Minh C. Pham, Fatemeh Khani Habibabadi, Heather Vital, Nathan Higinson-Scott, Kevin L. Otipoby, Yi Xing, Ivan D. Maccanfroni, Kevin C. O'Connor	
9	COMPOSITE RESPONSE TO NIPICALIMAB BASED ON BOTH MYASTHENIA GRAVIS ACTIVITY OF DAILY LIVING AND QUANTITATIVE MYASTHENIA GRAVIS SCORES IN PATIENTS WITH GENERALIZED MYASTHENIA GRAVIS	Said R. Beydoun, Maria Ait-Tihyaty, Ibrahim Turkoz, Kavita Gandhi, Sindhu Ramchandran	<p>INTRODUCTION: Nipocalimab demonstrated efficacy using the Myasthenia Gravis-Activities of Daily Living (MG-ADL) and Quantitative MG (QMG) scales in 24-week double-blind phase-3 Vivacity-MG3 study (NCT04951622) in patients with generalized MG (gMG). A meaningful treatment response incorporating both patient and clinician perspectives reflects clinical improvement and functional impact on the patient's quality-of-life.</p> <p>OBJECTIVE: To evaluate the likelihood of composite treatment response (CR) using both MG-ADL and QMG scales in patients with gMG receiving nipocalimab+standard-of-care [SOC] (nipocalimab) or placebo+SOC (placebo).</p> <p>METHODS: CR was defined as MG-ADL total score improvement of ≥ 2-points and QMG total score improvement of ≥ 3 points from baseline. The proportions of patients achieving CR (at each visit) and sustained CR (SCR) were examined. Generalized estimating equations evaluated differences in CR rates over 24 weeks.</p> <p>RESULTS: Of 153 patients (efficacy analysis set), a greater proportion of nipocalimab-treated versus placebo-treated patients achieved CR from week 8 through week 24. At week 8, 51.9% of nipocalimab-treated versus 23.7% of placebo-treated patients achieved CR; by week 24, proportion of patients achieving CR was 46.8% versus 21.1%, respectively. Nipocalimab-treated patients were 4-times more likely to achieve CR (odds ratio [95% CI]: 4.02 [2.33, 6.97]) over 24-weeks and were >4-times more likely to achieve SCR for ≥ 8, 12, 16, 20-weeks versus placebo. For >8-weeks, nipocalimab-treated patients were >6-times more likely to attain SCR (odds ratio [95% CI]: 6.34 [2.85, 14.07]) than placebo.</p> <p>SUMMARY/CONCLUSION: Patients with gMG demonstrated significantly higher likelihood of achieving CR and SCR based on both patient-reported MG-ADL and clinician-reported QMG assessments when treated with nipocalimab+SOC versus placebo+SOC.</p> <p>INTRODUCTION: Severe symptoms of generalized myasthenia gravis (gMG) can lead to hospitalization. Patient factors/categories associated with hospitalization are not clearly understood.</p> <p>OBJECTIVE: To identify predictors of higher rates of gMG-related hospitalization in a longitudinal cohort of patients.</p> <p>METHODS: PREDICT was a retrospective, observational study in patients receiving care for gMG for ≥ 1 year at Mass General Brigham from 2010–2023. Data were extracted from medical records following chart review. A marginal means regression model was used for analysis. Hazard ratios (HRs) and 95% CIs were calculated.</p> <p>RESULTS: In total, 296 patients with gMG were included in the study: 129 (43.6%) had ≥ 1 gMG-related hospitalization. Overall, 123 patients (42.2%) were female, and 265 (89.5%) were White. At gMG diagnosis, median age was 66, and Myasthenia Gravis Foundation of America Clinical Classification (MGFA CC) was III in 54 (18.2%) patients, IV in 20 (6.8%), and V in 9 (3%). Overall, 52 patients (17.6%) had a history of thymoma, and the median Charlson Comorbidity Index (CCI) score was 3. At diagnosis, 21 (7.1%) had an autoimmune comorbidity, and 233 (78.8%) were acetylcholine receptor antibody positive. Higher gMG-related hospitalization rate was significantly (all $P < 0.05$) associated with Black/African American race (HR [95% CI]: 2.20 [1.30, 3.73]), MGFA CC III (1.82 [1.12, 2.91]), IV (3.13 [1.85, 5.31]), or V (2.51 [1.23, 5.10]) at diagnosis, and highest CCI category at diagnosis (1.98 [1.19, 3.30]), but not age at diagnosis, history of thymoma, autoimmune comorbidities at diagnosis, or antibody status.</p> <p>SUMMARY/CONCLUSION: Patients exhibiting these factors/categories may be at risk for more frequent hospitalizations.</p>
10	PREDICTORS OF MYASTHENIA GRAVIS HOSPITALIZATIONS IN THE PREDICT STUDY	Shamik Bhattacharyya, Sathya Narasimhan, Danielle Kei A. Pua, Yihan Zhang, Prashanth Rajarajan, Mattia Wruble Clark, James V. Nguyen, Alice Tang, Chien-Lin Su, Michael Blackowicz, Chloe Sader, Joome Suh	

11	ADHERENCE TO AND PERSISTENCE WITH BIOLOGICS AMONG US PATIENTS WITH GENERALIZED MYASTHENIA GRAVIS: A CLAIMS-BASED STUDY	Moon Kim, Ratna Bhavaraju-Sanka, Andrea Meyers, Rajvi J. Wani, Kaideing Zhu, Blanca I. Canales, Kristina R. Patterson, Jenny Park, Beth Stein	<p>INTRODUCTION: Several biologics to treat generalized myasthenia gravis (gMG) have been approved, yet adherence and persistence remain poorly characterized. Eculizumab (every 2 weeks), ravulizumab (every 8 weeks), and zilucoplan (daily) use fixed intervals, whereas efgartigimod and rozanolixizumab cycles include variable gaps.</p> <p>OBJECTIVE: Quantify real-world adherence to and persistence with different biologics among U.S. adults with gMG.</p> <p>METHODS: Retrospective cohort study using Optum Clinformatics Data Mart (10/01/2015–06/30/2024). Adults (≥ 18 y) with incident, treatment-naïve gMG and (1) ≥ 2 outpatient visits or (2) ≥ 1 inpatient and ≥ 1 outpatient visit were included. Proportion of days covered (PDC) $\geq 80\%$ defined adherence. Persistence was continuous therapy allowing permissible gaps up to the label-specified dosing interval + 90 days.</p> <p>RESULTS: Of 6,042 incident gMG patients, 411 (5.9%) initiated ≥ 1 biologic—eculizumab 90 (1.5%), efgartigimod 223 (3.7%), ravulizumab 104 (1.7%), rozanolixizumab 11 (0.2%), zilucoplan 1 (<0.1%). Among biologic-treated patients, 69 (16.8%) received multiple biologics. Within each biologic cohort, adherent proportions were eculizumab 70.8%, efgartigimod 64.7%, and ravulizumab 98.0%. Median persistence (IQR) months were eculizumab 9.5 (15.5), efgartigimod 4.6 (9.4), ravulizumab 4.4 (7.6), although variable follow-up may limit these estimates. For efgartigimod, median gap length between cycles was 6.0 weeks (IQR 3.2); 43.6% had <6-week gaps. Dosing deviations occurred in 18% of efgartigimod patients receiving single infusions every ~6.6 weeks.</p> <p>SUMMARY/CONCLUSION: This study highlights unmet needs and barriers as demonstrated by modest uptake of biologics, heterogeneous adherence, and varying persistence rates. These findings suggest biologics with fixed dosing schedules and longer dosing frequencies may improve patient adherence.</p>
12	IMPACT ON HEALTHCARE RESOURCE UTILIZATION IN EARLY INITIATORS OF RAVULIZUMAB OR EFGARTIGIMOD FOR TREATMENT OF GENERALIZED MYASTHENIA GRAVIS IN THE USA	Riley Snook, Michael Blackowicz, Emma Weiskopf, Dan Fogarty, Neha Arora, Raghav Govindarajan	<p>INTRODUCTION: Generalized myasthenia gravis (gMG) is an autoimmune neuromuscular disorder characterized by fatigable muscle weakness. Suboptimally treated gMG can result in clinical deterioration, hospitalizations, and increased healthcare resource utilization. Ravulizumab, a complement C5 inhibitor, and efgartigimod, an FcRn inhibitor, are US FDA-approved therapies for anti-acetylcholine receptor antibody positive gMG.</p> <p>OBJECTIVE: This study aims to present real-world evidence demonstrating the impact of both ravulizumab and efgartigimod on gMG-related healthcare resource utilization in patients initiating treatment within 2 years of diagnosis.</p> <p>METHODS: Patients initiating ravulizumab or efgartigimod within 2 years of diagnosis were identified in Atlas (Definitive Healthcare), a hybrid open/closed US claims database set between 01/2017 to 12/2024. Patients had ≥ 1 year of continuous follow-up before and after initiation. The study compared hospitalizations and other healthcare resource utilization before and after initiation of ravulizumab or efgartigimod.</p> <p>RESULTS: There were 114 ravulizumab patients and 639 efgartigimod patients who met the inclusion criteria. Initiators of ravulizumab within 2 years of diagnosis experienced an 81% (IRR=0.19; 95% CI: 0.12, 0.30; p<0.001) reduction in all-cause hospitalization compared to a 32% reduction (IRR=0.68; 95% CI: 0.53, 0.87; p=0.002) in early initiators of efgartigimod (pcomparison=0.001). Similar trends observed for other healthcare resource endpoints will additionally be reported.</p> <p>SUMMARY/CONCLUSION: Patients who initiated ravulizumab or efgartigimod within 2 years of diagnosis had significant reductions in healthcare resource utilization. However, those who started on ravulizumab had statistically significantly greater reduction. Timely initiation of potentially disease-modifying targeted treatments, such as C5 inhibitor therapy, may have implications for reducing healthcare resource utilization in patients with gMG.</p>
13	LONG-TERM SAFETY OF CYCLIC ROZANOLIXIZUMAB TREATMENT IN PATIENTS WITH GENERALIZED MYASTHENIA GRAVIS: A FINAL ANALYSIS OF PHASE 3 STUDIES	Ali Habib, Carlo Antozzi, Julian Grosskreutz, Sabrina Sacconi, Kimiaki Utsugisawa, John Vissing, Tuan Vu, Fiona Grimsom, Niamh Houston, Thais Tarancon, Vera Bril	<p>INTRODUCTION: In Mycarin® (MG0003; NCT03971422) and completed open-label extension study, MG0007 (NCT04650854), repeated cyclic rozanolixizumab treatment demonstrated a consistent long-term safety profile in adults with generalized myasthenia gravis.</p> <p>OBJECTIVE: Evaluate incidence of pre-defined treatment-emergent adverse events (TEAEs).</p> <p>METHODS: Following one 6-week cycle of rozanolixizumab 7mg/kg, 10mg/kg, or placebo in Mycarin®G, patients could enter MG0007 to receive a further 6-week cycle (rozanolixizumab 7mg/kg or 10mg/kg), subsequent cycles were administered upon symptom worsening. Final data were pooled across Mycarin®G and MG0007 for patients receiving ≥ 1 rozanolixizumab treatment cycle with a ≤ 3-week follow-up period. Pre-defined TEAEs, including headaches, gastrointestinal disorders, infections, hypersensitivity, and anaphylactic reactions, were evaluated.</p> <p>RESULTS: Overall, 188 patients received ≥ 1 rozanolixizumab treatment cycle (mean: 2.9 cycles per year [standard deviation 1.8]). Across 13 cycles, incidence of TEAEs ranged from 42.3% (n/N=11/26 [Cycle 12]) to 79.3% (n/N=149/188 [Cycle 1]); most mild/moderate. Across all cycles, pre-defined TEAE incidences were: headaches (headache, migraine, migraine with aura), 51.6% (n/N=97/188); gastrointestinal disorders, 43.6% (n/N=82/188); infections, 58.0% (n/N=109/188); hypersensitivity reactions, all non-serious, 15.4% (n/N=29/188). No anaphylactic reactions occurred. Excluding headaches and gastrointestinal disorders (more frequent in Cycle 1), pre-defined TEAE incidences were similar across cycles. Eight (4.3%) patients experienced severe headaches. Twelve (6.4%) patients experienced serious infections. One aseptic meningitis event led to study discontinuation. No clinically meaningful changes in lipid or albumin levels were identified. Four deaths occurred, all deemed unrelated to rozanolixizumab by investigators.</p> <p>SUMMARY/CONCLUSION: Rozanolixizumab was well tolerated; data were consistent with the known rozanolixizumab safety profile. Pre-defined TEAE incidence did not increase with repeated cyclic treatment. Funding: UCB.</p>
14	DESIGN OF A PHASE 3 RANDOMIZED, DOUBLE-BLINDED, PLACEBO-CONTROLLED STUDY EVALUATING SUBCUTANEOUS EFGARTIGIMOD PH20 ADMINISTERED BY PREFILLED SYRINGE IN ADULTS WITH OCULAR MYASTHENIA GRAVIS	Yern C. Juel, Carolina Barnett-Tapia, James F. Howard Jr., Jeffrey Guptill, Rosa H. Jimenez, Fien Gistelnick, Sophie Steeland, Fien M. Verhamme, Sui H. Wong	<p>INTRODUCTION: Efgartigimod PH20 is an immunoglobulin G1 (IgG1) antibody Fc-fragment coformulated with recombinant human hyaluronidase PH20 that selectively reduces IgG levels by blocking neonatal Fc receptor-mediated IgG recycling. There is an unmet need for approved, effective treatments for patients with ocular myasthenia gravis (oMG). Retrospective analysis of data supporting the approval of efgartigimod for treatment of adults with generalized myasthenia gravis indicated an improvement in ocular symptoms. The Phase 3 ADAPT OCULUS trial (NCT06558279) will investigate the efficacy and safety of efgartigimod in participants with oMG.</p> <p>OBJECTIVE: To present the design of the ADAPT OCULUS trial evaluating the efficacy and safety of efgartigimod PH20 SC in adults with oMG.</p> <p>METHODS: Adults with confirmed oMG and a Myasthenia Gravis Impairment Index (MGI) patient-reported outcome (PRO) subcomponent ocular score ≥ 6 who are on stable MG therapy will be randomized 1:1 to receive 4 once-weekly efgartigimod PH20 subcutaneous (SC) 1000 mg or placebo injections administered via prefilled syringe, followed by 4 weeks of follow-up. Participants may continue in the ≤ 2-year open-label extension.</p> <p>RESULTS: The primary endpoint is change in MGI PRO ocular score from baseline to Week 4. Key secondary endpoints include, among others, changes from baseline to Week 4 in MGI ocular score (PRO plus physical examination) and total score. Safety assessments include adverse event incidence and severity.</p> <p>SUMMARY/CONCLUSION: This is the first Phase 3 clinical trial evaluating the safety and efficacy of efgartigimod PH20 SC in patients with oMG that addresses the unmet need for treatment in oMG.</p>
15	DELAY IN THE DIAGNOSIS OF MYASTHENIA GRAVIS. A MULTICENTRIC RETROSPECTIVE STUDY IN ARGENTINA.	Francisco Caiza-Zambrano, Luciano León-Cejás, Anabella Gomez, Ana Laura Blazquez, Anabella Frances, Camila Aguirre, Juliana Diaz, Cecilia Quarracino, Miguel Saucedo, Florencia Diaz Sica, Gabriela Peretti, Facundo Heredia, Eugenia Conti, Cintia Marchesoni, Ricardo Reisin	<p>INTRODUCTION: Diagnosing myasthenia gravis (MG) can be challenging due to the variability in clinical presentation. Studies in developed countries have identified a diagnosis delay of more than a year in approximately 10% of patients. Data in Latin America is lacking.</p> <p>OBJECTIVE: To determine the mean diagnosis delay in patients with MG from Argentina.</p> <p>METHODS: A retrospective study was conducted in patients with MG who received treatment in ten centers. Medical records were reviewed to attain sociodemographic data and disease characteristics. Diagnosis delay (time from symptom onset to diagnosis) was calculated.</p> <p>RESULTS: Eighty-four patients were included. Most of them were women (58%). Mean age at symptom onset was 40.9 (SD 20.4) and ocular symptoms were the most frequent (67.9%). More than half (58.3%) come from the public health system. Neurology was consulted first by the 8.3% (n=7) of patients. Mean diagnosis delay was 431.1 (SD 710.3) days, and 40.5% of patients experienced diagnosis delay >1 year. Among patients with >1 year, 29.4% had initially received a different diagnosis and 35.2% were Myasthenia Gravis Foundation of America class III at the time of the diagnosis.</p> <p>Diagnosis was faster in patients who first consulted a neurologist compared to those who were evaluated by other specialists (mean 116.5 days Vs 499.1 days; p=0.015). There was no difference between patients evaluated in the public or private health system.</p> <p>SUMMARY/CONCLUSION: Although the development of new therapies has advanced in recent years, further work is needed on medical education about the disease to improve the diagnosis of MG.</p>

16	DESIGN OF A DIGITAL SOLUTION TO IMPROVE MYASTHENIA GRAVIS PATIENT SYMPTOM TRACKING IN ROUTINE CLINICAL CARE	Srikanth Muppidi, Joshua Alpers, Ashley E. L. Anderson, Nicholas Streicher, Ananda Vishnu Pandurangadu, Hari Jayaraman, Archit Gupta, Nolan Campbell, Zia Choudhry	<p>INTRODUCTION: The fluctuating nature of myasthenia gravis (MG) symptoms creates challenges for disease management. Tracking of patient-reported outcomes (PROs) in routine clinical practice could be improved.</p> <p>OBJECTIVE: Determine design requirements for a digital tool utilizing validated PROs to improve symptom tracking in routine practice.</p> <p>METHODS: A literature review and preliminary interviews with patients with MG (n=3) and healthcare practitioners (HCPs; n=4) were conducted to assess the current state of MG symptom tracking. Structured workshops with HCPs (n=5) and validation interviews with patients (n=10) and HCPs (n=9) were held to design a novel digital tool and understand factors influencing adoption. Participants were US-based. Transcripts were analyzed for themes regarding challenges, preferred solutions, and benefits/applications of the proposed digital tool.</p> <p>RESULTS: Key design requirements included a two-sided digital solution where patients input validated PROs between clinic visits, and HCPs visualize longitudinal data on demand via integration with electronic health records. The MG-ADL score was the preferred primary visual, with ability to overlay subscores and other contextual data. Free text patient diary entries with artificial intelligence-generated summaries for HCPs were desired for additional contextualization and personalization. Factors influencing patient adoption included HCP use and potential for one central MG management tool. HCPs noted streamlined visualizations enabling quick data synthesis to support treatment decisions and features to simplify insurance prior authorization/reauthorization would facilitate adoption.</p> <p>SUMMARY/CONCLUSION: Patients and HCPs agreed the proposed solution would enhance clinical care by improving MG symptom tracking and ultimately treatment decisions. These results support continued development of the digital tool and studies investigating clinical utility.</p>
17	EFFICACY AND SAFETY OF NIPOCALIMAB VS EFGARTIGIMOD IN A RANDOMIZED, OPEN-LABEL, PHASE 3B, INTERVENTIONAL TRIAL INCLUDING WITHIN CLASS SWITCHING FROM EFGARTIGIMOD TO NIPOCALIMAB (EPIC): STUDY DESIGN	Srikanth Muppidi, Andrea Corse, Heinz Wiendl, Ibrahim Turkoz, Ruben Faelsen, Zia Choudhry, John J Sheehan, Maria Ait-Tihyaty, Nolan Campbell	<p>INTRODUCTION: Nipocalimab and efgartigimod are FcRn-targeting treatments for generalized myasthenia gravis (gMG) with differing molecular structures, binding affinities, and dosing. Currently, there are no trials directly comparing efficacy of nipocalimab vs efgartigimod and no data to inform switch strategies from efgartigimod to nipocalimab.</p> <p>OBJECTIVE: EPIC aims to evaluate efficacy of nipocalimab vs efgartigimod in participants initiating FcRn treatment for gMG and to evaluate efficacy and safety of nipocalimab in participants switching from efgartigimod to nipocalimab.</p> <p>METHODS: EPIC is a phase 3b, randomized, open-label, interventional study in adults with gMG. FcRn-naïve participants (n=80) are randomized 1:1 to receive nipocalimab every 2 weeks for 12 weeks (Arm 1) or efgartigimod every week (10 mg/kg) for 4 weeks (Arm 2). Participants in Arm 2 and additional participants with ≥1 on-label efgartigimod cycle (minimum n=35) can enroll in the treatment switch phase of the study to be followed on nipocalimab for 12 weeks (Arm 3).</p> <p>RESULTS: This study addresses whether nipocalimab provides superior efficacy to efgartigimod in the latter part of efgartigimod cycles that cover most dosing patterns utilized in clinical practice. Key primary and secondary efficacy endpoints are change from baseline in total IgG, MG-ADL, and QMG scores averaged over Weeks 8–12, and at Week 8 between Arms 1 and 2. Key endpoints for treatment switch are change in MG-ADL and safety in Arm 3.</p> <p>SUMMARY/CONCLUSION: EPIC is the first randomized trial comparing advanced treatments for patients with gMG and is designed to provide critical insights to inform clinical decisions when initiating or switching in the FcRn-targeting class.</p> <p>INTRODUCTION: The Myasthenia Gravis (MG) Foundation of America Global MG Patient Registry (MGFAPE) captures data that may facilitate understanding of MG risk factors.</p> <p>OBJECTIVE: Identify baseline risk factors for ≥1 self-reported exacerbation(s) in the past 6-months at first follow-up, and for MG-ADL score ≥2 point increase between baseline and first follow-up.</p> <p>METHODS: 1319 MGFAPE surveys completed between July 2013–June 2023 by US-based adults with self-reported MG and first follow-up data available within 12 months of enrollment were analyzed. Exacerbations in the past 6 months and changes in MG-ADL scores at first follow-up were evaluated.</p> <p>RESULTS: Of 1319 patients, 1187 (90%) reported the presence/absence of exacerbation(s); 460 (39%) reported ≥1 exacerbation. Identified factors positively associated with report of experiencing ≥1 exacerbation(s) in the previous 6 months were comorbid anxiety/depression (2x more likely), living alone (74%+), corticosteroids (36%+), and MG-ADL score point-increase (19%+ per-point) at enrollment. Factors inversely associated with ≥1 exacerbation(s) included ocular versus generalized first MG symptoms (51%-), nonsteroidal immunosuppressants at enrollment (25%-), and each additional year post-diagnosis (2%-). Of 1232 (93%) patients reporting MG-ADL scores, 210 (17%) had ≥2 point increase. Factors positively associated with MG-ADL score increase ≥2 included first MG symptoms generalized vs ocular (-138%+), and comorbid anxiety/depression (73%+); those inversely associated were plasma exchange (excluding rescue therapy, 83%-), physical activity at enrollment (37%-), and increased age at first follow-up (2%-/year).</p> <p>SUMMARY/CONCLUSION: Findings demonstrated that many US individuals with MG reported uncontrolled disease. Important risk factors identified for exacerbation or symptom worsening included living alone, generalized MG symptomatology, and comorbid anxiety/depression.</p>
18	IDENTIFYING RISK FACTORS FOR EXACERBATION AND SYMPTOM WORSENING—A RETROSPECTIVE COHORT STUDY OF PATIENTS WITH MYASTHENIA GRAVIS IN THE UNITED STATES	Zia Choudhry, Minjee Park, Pushpa Narayanaswami, Nizar Souayah, Raghav Govindarajan, Michael Kutch, Amani Zurinaga Gutierrez, Aurelie Chekroun Martineo, Nolan Campbell, Richard Nowak	<p>INTRODUCTION: Cell-based assays (CBAs) are essential for diagnosing antibody-mediated disorders including myasthenia gravis (MG) and myelin oligodendrocyte glycoprotein antibody-associated disease (MOGAD). However, current CBAs require repeated transient transfection of autoantigens, which is laborious and resource-intensive. More importantly, variability in transfection efficiency introduces batch-to-batch inconsistency, compromising assay reproducibility.</p> <p>OBJECTIVE: To generate stable cell lines expressing disease-associated autoantigens for assessing antibody profiles in MG and MOGAD.</p> <p>METHODS: HEK293T cells were transfected with plasmids encoding human muscle-specific tyrosine kinase (MuSK) or myelin oligodendrocyte glycoprotein (MOG), each co-expressing green fluorescence protein (GFP). Fluorescence-activated cell sorting was conducted to isolate single-cell clones with high or low levels of autoantigen expression. Live CBAs using flow cytometry were performed to determine binding properties of patient-derived monoclonal antibodies (mAbs) and serum samples.</p> <p>RESULTS: Monoclonal cell lines stably expressing varying levels of MuSK-GFP or MOG-GFP were successfully established and maintained phenotypic stability for at least one month in continuous culture. They exhibited optimal performance in CBAs when tested with MuSK- or MOG-specific mAbs without cross-reactivity or nonspecific binding. Notably, autoantigen expression levels, cell input numbers, and the affinity of mAbs affected the binding patterns. In preliminary experiments, both MuSK-GFP and MOG-GFP stable cell lines effectively discriminated patients from healthy controls, providing sensitivity equivalent to or superior to that of standard CBAs.</p> <p>SUMMARY/CONCLUSION: With consistent and permanent autoantigen expression, stable MuSK-GFP and MOG-GFP cell lines hold promise for facilitating diagnostic workflows and improving testing accuracy. Additionally, variables influencing the antibody-to-antigen ratio should be carefully controlled to ensure robust assay repeatability.</p>
19	DEVELOPMENT AND VALIDATION OF AUTOANTIGEN-EXPRESSING STABLE CELL LINES FOR AUTOANTIBODY CHARACTERIZATION IN AUTOIMMUNE NEUROLOGICAL DISORDERS	Kangzhi Chen, Gianvito Masi, Erin Longbrake, Richard Nowak, Kevin O'Connor	<p>INTRODUCTION: Cell-based assays (CBAs) are essential for diagnosing antibody-mediated disorders including myasthenia gravis (MG) and myelin oligodendrocyte glycoprotein antibody-associated disease (MOGAD). However, current CBAs require repeated transient transfection of autoantigens, which is laborious and resource-intensive. More importantly, variability in transfection efficiency introduces batch-to-batch inconsistency, compromising assay reproducibility.</p> <p>OBJECTIVE: To generate stable cell lines expressing disease-associated autoantigens for assessing antibody profiles in MG and MOGAD.</p> <p>METHODS: HEK293T cells were transfected with plasmids encoding human muscle-specific tyrosine kinase (MuSK) or myelin oligodendrocyte glycoprotein (MOG), each co-expressing green fluorescence protein (GFP). Fluorescence-activated cell sorting was conducted to isolate single-cell clones with high or low levels of autoantigen expression. Live CBAs using flow cytometry were performed to determine binding properties of patient-derived monoclonal antibodies (mAbs) and serum samples.</p> <p>RESULTS: Monoclonal cell lines stably expressing varying levels of MuSK-GFP or MOG-GFP were successfully established and maintained phenotypic stability for at least one month in continuous culture. They exhibited optimal performance in CBAs when tested with MuSK- or MOG-specific mAbs without cross-reactivity or nonspecific binding. Notably, autoantigen expression levels, cell input numbers, and the affinity of mAbs affected the binding patterns. In preliminary experiments, both MuSK-GFP and MOG-GFP stable cell lines effectively discriminated patients from healthy controls, providing sensitivity equivalent to or superior to that of standard CBAs.</p> <p>SUMMARY/CONCLUSION: With consistent and permanent autoantigen expression, stable MuSK-GFP and MOG-GFP cell lines hold promise for facilitating diagnostic workflows and improving testing accuracy. Additionally, variables influencing the antibody-to-antigen ratio should be carefully controlled to ensure robust assay repeatability.</p>

			<p>INTRODUCTION: Generalized myasthenia gravis (gMG) causes muscle weakness, fatigue, and/or ocular symptoms. Patient perspectives can help better understand symptom variety and current treatment burden.</p> <p>OBJECTIVE: Characterize the experience of living with gMG.</p> <p>METHODS: US adults with self-reported gMG/ocular MG diagnosis or their caregivers were recruited to participate in a 13-question online survey. Survey responses guided/facilitated four 2-hour virtual Patient Engagement Research Council (PERC) focus group discussions. A patient journey map was generated from insights.</p> <p>RESULTS: Of 16 participants (n=4 caregivers; n=12 patients), the highest proportion per sociodemographic-category were white (62.5%), identified as female (50%), and ages 50-59 years (37.5%). Pre-session survey responses indicated frequently reported first symptoms were vision problems (63%), extreme fatigue (50%), eyelid drooping (44%), and muscle weakness (44%). Most participants (62.5%) were diagnosed within one year of seeking healthcare, but 25% experienced 1-4-year diagnostic delays and 60% experienced misdiagnosis. About half had tried 4-6 gMG treatments and 44% were dissatisfied with the time to find an effective treatment. Insurance issues led to treatment delays in 69% of participants and 81% felt gMG symptoms were dismissed. Survey results were validated by the PERC discussions, as most participants reported emotionally challenging gMG journeys. One participant cited frustration over the "trial-and-error" nature of their treatment plan. Patients suggested mapping out the patient journey non-linearly to reflect disease instability and the cyclical nature of treatment planning.</p> <p>SUMMARY/CONCLUSION: Leveraging these insights may help improve gMG management and patient outcomes, as US patients with gMG continue to experience substantial disease instability despite increased treatment options.</p>
20	MAPPING OUT THE PATIENT JOURNEY OF GENERALIZED MYASTHENIA GRAVIS: INSIGHTS AND CHALLENGES	Zia Choudhry, Louis Jackson, Nolan Campbell, Lisa Shea, Sindhu Ramchandren, Maria Ait-Tihyaty, Ktly Gwathmey, Kavita Grover, Bassam Mulo	<p>INTRODUCTION: Dysregulated Th1-differentiation plays an essential role for autoimmune diseases, such as autoantibody-mediated myasthenia gravis (MG). Our previous study has shown that carbonic anhydrase 3 (Car3) was specifically insufficient in skeletal muscle from MG patients, which plays an essential role for the pathogenesis of MG.</p> <p>OBJECTIVE: Here, we will elucidate the underlining mechanism between Car3 and dysregulated Th1 in skeletal muscle.</p> <p>METHODS: Experimental autoimmune myasthenia gravis (EAMG) animal model established using AChR antigen with wild type and Car3 deficient (Car3^{-/-}) mice. The animal phenotype severity and levels of Th1 and intracellular complement C3a were compared.</p> <p>RESULTS: We found that Car3^{-/-} mice display considerably increased muscle weakness in EAMG animal model. More prominently, the EAMG Car3^{-/-} mice had significantly higher levels of anti-AChR autoantibodies, and substantial Th1 differentiation in skeletal muscle. Further, we detected more intracellular complement C3a component in Car3^{-/-} skeletal muscle. Mechanistically, Car3 deficiency facilitates the production of intracellular C3a mediated by cathepsin S. After extracellular release, C3a contributed to Th1 differentiation by binding with C3a receptor in T cells, consequently inducing autoantibody production.</p> <p>SUMMARY/CONCLUSION: Our results demonstrate that Car3 dampens intracellular activation of C3 in skeletal muscle cells and suppresses Th1 differentiation accordingly. This study also implies that altered muscular immunological microenvironment revealed a novel communication between skeletal muscle system and immune system.</p>
21	MUSCLE CARBONIC ANHYDRASE 3 INHIBITS COMPLEMENT C3A-MEDIATED FOLLICULAR HELPER T CELL DIFFERENTIATION.	Ailian Du, Congfeng Xu	<p>INTRODUCTION: Generalized myasthenia gravis (gMG) is a chronic, antibody-mediated autoimmune disease associated with unpredictable, fluctuating muscle weakness.</p> <p>OBJECTIVE: This study used generative artificial intelligence (GenAI) to explore patient and caregiver experiences of gMG symptom instability and unpredictability, and experiences with healthcare providers (HCPs).</p> <p>METHODS: Fifteen focus groups were conducted with patients with gMG (n=11) or their caregivers (n=6) in the US. Structured guides elicited overall disease perspectives, journey, symptom instability, treatment perspectives, and gaps in care. Data analysis guided by Leventhal's Common-Sense Model of Self-Regulation (CSM) framework was assisted using Johnson & Johnson's private and proprietary GenAI tool. Emotional and cognitive representations of the CSM framework were described in relation to participants' gMG experience.</p> <p>RESULTS: Participants described a range of negative emotions related to gMG symptom instability, including anxiety, depression, fear, isolation, and frustration; positive emotions were associated with support groups, resilience, and self-advocacy. Perspectives on gMG disease experience involved common symptoms, triggers, and HCP communication, with consequences of gMG including negative impacts on quality of life, ability to work, and social function. Participants noted their gMG timeline included frequent hospital visits and HCP communication, as well as difficulty/stress managing treatment. Discussions of disease control highlighted perceptions of disease instability, and treatment challenges and inconsistency.</p> <p>SUMMARY/CONCLUSION: This GenAI-assisted analysis demonstrated that gMG symptom instability negatively impacted patients and caregivers, affecting all representations of the CSM framework. Personalized treatment programs may be utilized to address poor cognitive and emotional responses to gMG, thereby enhancing treatment experience and resulting in sustained symptom control for patients with gMG.</p>
22	PATIENT AND CAREGIVER EXPERIENCES OF SYMPTOM INSTABILITY AND UNPREDICTABILITY FROM A MYASTHENIA GRAVIS PATIENT ENGAGEMENT RESEARCH COUNCIL: A GENERATIVE AI-ASSISTED ANALYSIS	Nicholas Streicher, OluYemisi Falope, Bruce West, Victor Wang, Rebecca Genin, LouisJackson, Zia Choudhry, Kavita M. Grover	<p>INTRODUCTION: In the VIVACITY-MG3 study, nipoalimab+standard-of-care (SOC) demonstrated statistically significant improvements in Quantitative Myasthenia Gravis (QMG) total score versus placebo+SOC (between-group difference in LS-mean=-2.81, 95%CI=-4.22,-1.41) over 22-24 weeks.</p> <p>OBJECTIVE: To evaluate improvements in QMG items/domains for nipoalimab+SOC versus placebo+SOC.</p> <p>METHODS: In post-hoc analyses of primary efficacy dataset, baseline floor effects (score=0) for QMG items/domains were evaluated using item-level frequency distributions and mean domain scores. Analysis of covariance models compared mean change-from-baseline (CFB) for bulbar, respiratory, limb, and ocular domains at 24-weeks between nipoalimab+SOC and placebo+SOC. Based on items/domains score distributions, item-responders had ≥1-point improvement; domain-responders had ≥2-points improvement, except for 1-item respiratory domain (≥1-point). Generalized estimating equations evaluated improvement likelihood in items/domains over 24-weeks.</p> <p>RESULTS: Among 149 patients, proportion with item-level floor effects at baseline ranged from 2.6% (right/left leg outstretch) to 56.2% (forced expiratory volume). Baseline mean(range) domain scores in nipoalimab+SOC and placebo+SOC: bulbar (0.8 [0-5]; 1.2 [0-6]), limb (8.8 [2-16]; 9.8 [1-20]), ocular (4.3 [0-8]; 4.0 [0-8]); respiratory (0.6 [0-3]; 0.7 [0-3]), respectively. Nipoalimab+SOC demonstrated significant improvements in mean CFB for domains versus placebo+SOC (p<0.05) over 24-weeks. Likelihood of achieving item response was greater with nipoalimab+SOC over 24-weeks (odds-ratio [OR], 95% CI): 1.3 (0.6-2.7)[left-hand grip] to 3.7 (1.9-7.3)[facial muscles]. OR (95%CI) of domain response over 24-weeks were: bulbar 6.6 (2.5-17.2); limb 1.7 (1.0-2.8); ocular 3.0 (1.7-5.3); respiratory 1.4 (0.6-3.1), generally favoring nipoalimab.</p> <p>SUMMARY/CONCLUSION: QMG total score changes were driven by improvements in all QMG items/domains generally favoring nipoalimab+SOC. Likelihood of achieving response thresholds on QMG items/domains generally favored nipoalimab+SOC versus placebo+SOC over 24-weeks.</p>
23	ASSESSING SEVERITY IN GENERALIZED MYASTHENIA GRAVIS: A PHASE 3 STUDY OF NIPOCALIMAB USING QUANTITATIVE MYASTHENIA GRAVIS ITEMS AND DOMAINS	Mazen Dimachkie, Constantine Farmakidis, Kavita Gandhi, Maria Ait-Tihyaty, Ibrahim Turkoz, Sheryl Pease, Zia Choudhry, Charlotte Gary, Antoine C. El Khoury, Sindhu Ramchandren	<p>INTRODUCTION: The International Consensus Guidelines for Management of MG recommends thymectomy in AChR antibody-positive non-thymomatous generalized MG patients to potentially avoid or minimize the dose or duration of immunotherapy in patients who are <45 years. There is no data on the impact of thymectomy in older non-thymomatous MG patients.</p> <p>OBJECTIVE: To assess clinical outcomes of thymectomy in older myasthenia gravis patients and determine effect on dose reduction of steroids and steroid sparing agents.</p> <p>METHODS: Charts of MG patients (> 50 years), who had thymectomy between Jan 2016-Nov 2023 (n=13) were retrospectively analyzed for change in clinical status and medication regimens. Overall statistical summary of demographic and clinical information was reported with median and range for continuous variables and counts and percentages for categorical. Dosage change (prednisone and pyridostigmine) and utilization of other treatments was analyzed. Statistical significance was defined as p-value < 0.05. All analyses were performed using R 4.4.0.</p> <p>RESULTS: Median age of patients was 66 years, 54% being males, with median disease duration of 7 years, and 2 years of post-thymectomy follow-up. MGFA grades generally dropped after thymectomy. All patients reduced the prednisone dosage after thymectomy, with some discontinuing it completely. Pyridostigmine dosage either remained the same or decreased, except for one who increased dosage and another who started after thymectomy. Use of IVig dropped from 6 patients to 1 patient. Plasmapheresis was discontinued in all 3 patients post-thymectomy.</p> <p>SUMMARY/CONCLUSION: The above findings suggest that thymectomy in older patients may be effective in reducing MG symptoms and reliance on aggressive treatments.</p>
24	IMPACT OF THYMECTOMY IN THE OLDER MYASTHENIA GRAVIS (MG) PATIENTS.	Cherine Fawaz, Kavita Grover	

25	ZILUCOPLAN TREATMENT OF SEVERE EXACERBATIONS LEADING TO HOSPITALIZATION IN GENERALIZED MYASTHENIA GRAVIS: STUDY DESIGN	Miriam Freimer, Mazen M. Dimachkis, Omar Sinno, Babak Borojerdi, Melissa Brock, Shital Patel, Lori Jensen, Natasa Savic, Tuan Vu	<p>INTRODUCTION: Severe exacerbations of generalized myasthenia gravis (gMG) requiring hospitalization can progress to myasthenic crisis, a life-threatening complication often requiring intubation and mechanical ventilation. Plasma exchange and IVIg (standard of care [SOC] for severe exacerbations) may be associated with serious side effects and morbidity.</p> <p>OBJECTIVE: To report the design of a clinical study of zilucoplan, a complement component 5 inhibitor, in adults with anti-acetylcholine receptor antibody-positive gMG with severe exacerbations requiring hospitalization.</p> <p>METHODS: In this Phase 4, open-label, multicenter study at three sites in the US, patients experiencing severe exacerbations (e.g., bulbar and/or respiratory symptoms requiring hospitalization or neck extension weakness) with Myasthenia Gravis-Activities of Daily Living (MG-ADL) score ≤ 6 in non-ocular symptoms will be offered SOC or study enrollment to determine whether zilucoplan rapidly alleviates the most severe gMG symptoms. Fifteen patients will receive daily subcutaneous zilucoplan 0.3mg/kg (inpatient followed by outpatient treatment) for a total duration of 12 weeks. Patients must have up-to-date meningococcal vaccinations or initiate meningococcal vaccination with antibiotic prophylaxis. Primary efficacy endpoint is change from baseline (CFB) in MG-ADL score at Week 2. Secondary endpoints include CFB through to Week 12 in MG-ADL, Quantitative Myasthenia Gravis and Myasthenia Gravis-Quality of Life 15-item revised scores, and in forced vital capacity and negative inspiratory force. Incidence of treatment-emergent adverse events (TEAEs), TEAEs leading to withdrawal of study medication, time to discharge and duration of ICU stay will be assessed.</p> <p>SUMMARY/CONCLUSION: This study will evaluate the efficacy and safety of zilucoplan in patients with severe gMG exacerbations requiring hospitalization. Funding: UCB (research collaboration).</p>
26	THE LONG-TERM EFFECTIVENESS OF ZILUCOPLAN IN MYASTHENIA GRAVIS: PREDICTIVE MODELING IN A US REAL-WORLD DATABASE	Miriam Freimer, Pauline Guilmin, Nina Temam, Ghinwa Y. Hayek, Babak Borojerdi, Fiona Grimson, Natasa Savic, M. Isabel Leite	
27	HOSPITAL RESOURCE UTILIZATION ASSOCIATED WITH NIPICALIMAB VERSUS PLACEBO: POST-HOC ANALYSIS OF THE VIVACITY-MG3 TRIAL IN GENERALIZED MYASTHENIA GRAVIS	John Vissing, Geoffroy Coteur, Ibrahim Turkoz, John Sheehan, Kavita Gandhi	<p>INTRODUCTION: Nipocalimab+standard-of-care (SOC), demonstrated stable and sustained disease control vs placebo+SOC in the double-blind, 24-week, phase-3 study (VIVACITY-MG3) in adult patients with generalized myasthenia gravis (gMG). gMG may worsen into crises/exacerbations requiring hospital admissions (HA) and emergency department visits (EDV).</p> <p>OBJECTIVE: To assess and compare all-cause hospital resource utilization (HURU) in patients with gMG on nipocalimab+SOC vs placebo+SOC in VIVACITY-MG3.</p> <p>METHODS: Details on HA, including length of stay (LoS), and EDV were collected every 12-weeks using the HURU questionnaire (HURU-Q). Incidence rate of HA/EDV was reported per 100 patient-years (pt-yr) for patients on nipocalimab+SOC/placebo+SOC in double-blind phase and for those continuing treatment with nipocalimab+SOC in open-label extension (OLE) phase. Stepwise multiple logistic regression models were used to identify potential clinical and demographic characteristics associated with incidence of HA/EDV events.</p> <p>RESULTS: During the double-blind phase, 9.1% and 15.8% of patients on nipocalimab+SOC (n=77) and placebo+SOC (n=70), respectively, experienced ≥ 1 HA/EDV event. Incidence rate of all-cause HA/EDV events was 51% numerically lower with nipocalimab+SOC vs placebo+SOC (incidence rate ratio [IRR]: 0.49); 23.4 vs 47.9 events/100pt-yr; mean HA-LoS was 6 days shorter with nipocalimab+SOC than placebo+SOC (8.4 vs 14.6 days). During the OLE phase, the incidence rate of HA/EDV with nipocalimab+SOC was maintained at 27.3 events/100pt-yr over a median follow-up of 54.1 weeks. Independent of treatment, key characteristics associated with HA/EDV incidence included baseline QMG respiratory score and worsening in total in MG-Activities of Daily Living score before the event.</p> <p>SUMMARY/CONCLUSION: The incidence rate of all-cause hospitalizations, EDV, and LoS per admission were lower with nipocalimab+SOC vs placebo+SOC.</p>
28	MANUAL PUSH ADMINISTRATION OF ROZANOLIXIZUMAB IN GENERALIZED MYASTHENIA GRAVIS	Rachana K. Gandhi Mehta, Carlo Antozzi, Gerardo Gutiérrez-Gutiérrez, Ali A. Habib, Zabeen K. Mahuwala, Kimaki Utsumigawa, Marion Boehnlein, Virginie Kerbusch, Andrea Lavrov, Thais Tarancon, Vera Beil	<p>INTRODUCTION: Generalized myasthenia gravis (gMG) is a chronic autoimmune disease. Rozanolixizumab, indicated for the treatment of adults with gMG, is administered by healthcare professionals (HCPs) using an infusion pump (IP). Manual push (MP) is an alternative administration method where medication is injected using an infusion set with a hand-pushed syringe plunger.</p> <p>OBJECTIVE: To describe three studies in which rozanolixizumab was administered by MP: UP0106 (Phase 1; NCT04828343), MG0007 (Phase 3; NCT04650854) and MG0020 (Phase 3; NCT05681715).</p> <p>METHODS: In UP0106, healthy volunteers were randomized to receive a single fixed dose of rozanolixizumab or placebo via IP or MP. In MG0007, six-week treatment cycles (weight-tiered dosing) were administered to patients with gMG via IP upon symptom worsening; the option for MP administration was later implemented. In MG0020, patients with gMG self-administered once-weekly rozanolixizumab (weight-tiered dosing) via IP and MP (6 weeks each); ability to self-administer rozanolixizumab was assessed at Weeks 12 and 18.</p> <p>RESULTS: In UP0106, 16/32 participants received HCP-administered MP infusions; median infusion duration: 1.35 minutes. In MG0007, 8/157 patients received 88 HCP-administered MP infusions; median infusion duration per cycle: 2–10 minutes. In MG0020, 55 patients self-administered 304 IP and 316 MP infusions; median (range) infusion durations: 12 (8–30) and 5 (1–30) minutes, respectively. The MP self-administration success rate was 100%. Rozanolixizumab safety and tolerability remained consistent regardless of method; no injection site reactions occurred during self-administration.</p> <p>SUMMARY/CONCLUSION: Rozanolixizumab was successfully administered via MP by HCPs or patients; infusion times were generally shorter than IP with no difference in safety profile. Funding: UCB.</p>
29	POST-HOC ANALYSIS OF CLINICALLY RELEVANT ANTI-VACCINE AND ANTI-VIRUS ANTIBODIES IN PATIENTS TREATED WITH NIPICALIMAB IN VIVACITY-MG3 STUDY	Faye Yu, Eugene Myshkin, Sindhu Ramchandren, Ricardo Rojo Cella, Robert Edwards, Matthew J. Loza, Desislava Dimitrova, Carolyn Cuff, Sheng Gao	<p>INTRODUCTION: Nipocalimab is a fully-human, high-affinity, aglycosylated, effecterless monoclonal antibody designed to selectively block neonatal fragment-crystallizable receptor, thereby lowering IgG levels, including pathogenic autoantibodies. In the phase 3 Vivacity-MG3 study (NCT04951622), nipocalimab treatment demonstrated rapid, substantial and sustained lowering of total IgG.</p> <p>OBJECTIVE: To evaluate the impact of nipocalimab on pre-existing clinically relevant anti-vaccine antibodies and humoral response to SARS-CoV-2 challenge in Vivacity-MG3 participants in the double-blind period.</p> <p>METHODS: Participants received nipocalimab (30 mg/kg loading dose followed by 15 mg/kg afterwards) or placebo intravenously every 2 weeks for 24 weeks. Serum IgG antibody levels against tetanus toxoid (TT) and varicella zoster virus (VZV) were measured at baseline and post-treatment samples in a subset of participants. In participants with available samples and documented SARS-CoV-2 vaccination or infection during the study, antibodies against different epitopes of SARS-CoV-2 were measured.</p> <p>RESULTS: Nipocalimab reduced pre-existing anti-TT and anti-VZV antibodies similarly to total IgG (observed median pre-dose/minimal reduction at week 24: 69%). The majority of nipocalimab-treated participants who were immune to TT (n=18) and VZV (n=19) at baseline maintained protective antibody levels during the double-blind treatment period. One nipocalimab-treated participant received TT vaccination during treatment and exhibited increased and sustained anti-TT levels above the protective threshold post-vaccination. In nipocalimab-treated participants, SARS-CoV-2 vaccination (n=12) during treatment increased anti-spike antibodies, while SARS-CoV-2 infection (n=9) led to increased anti-spike and anti-nucleocapsid antibodies.</p> <p>SUMMARY/CONCLUSION: Nipocalimab-treated patients largely remained protected for TT and VZV and demonstrated preserved humoral responses to TT vaccination and SARS-CoV-2 infection/vaccination, supporting compatibility with recommended vaccination schedules.</p>

30	SINGLE-FIBER EMG REVEALS NEUROMUSCULAR TRANSMISSION DISTURBANCES IN PEDIATRIC PRADER-WILLI SYNDROME: A DIAGNOSTIC CONSIDERATION IN MYASTHENIC DISORDERS	Malin Garrett, Maja Norling, Christoffer Ehrstedt, Cecilia Montgomery, Ricard Nergårdh, Anna Rostedt Punga	<p>INTRODUCTION: Children with Prader-Willi syndrome (PWS) frequently present with hypotonia and muscle fatigue, symptoms traditionally attributed to the underlying genetic disorder. Although one previous study has investigated single-fiber EMG (SFEMG) findings in a small number of PWS cases, the results remain inconclusive regarding whether abnormal neuromuscular transmission contributes to the observed weakness.</p> <p>OBJECTIVE: To evaluate neuromuscular transmission in children with PWS.</p> <p>METHODS: SFEMG was performed in the orbicularis oculi muscle. Repetitive nerve stimulation (RNS) was conducted on the abductor digiti minimi (ADM), trapezius, and deltoid muscles. Muscle strength and function were assessed using the Gross Motor Function Measure-88 (GMFM-88).</p> <p>RESULTS: Eleven children with PWS (9 boys, 2 girls; age range 2–13 years; median age 10), all receiving growth hormone therapy, were included. SFEMG was successfully conducted in all participants. RNS data were obtained from the ADM in 10 children, the trapezius in 9, and the deltoid in 8. Increased jitter, indicating abnormal neuromuscular transmission, was observed in 4 children (36%) on SFEMG. RNS results were normal across all muscle groups. GMFM-88 scores varied substantially among participants.</p> <p>SUMMARY/CONCLUSION: Abnormal neuromuscular transmission, as evidenced by increased jitter on SFEMG, was identified in a substantial proportion of children with PWS. The lack of correlation between SFEMG abnormalities and GMFM-88 scores may reflect differences in the muscle groups assessed by each method. These findings underscore the importance of distinguishing neuromuscular transmission failure in PWS from juvenile and congenital myasthenic syndromes.</p>
31	AN ANALYSIS OF DIGITAL CONVERSATIONS TO UNDERSTAND PATIENT PERCEPTIONS OF THE DOSING AND ADMINISTRATION OF NEONATAL FC RECEPTOR INHIBITORS FOR TREATMENT OF MYASTHENIA GRAVIS	Andrew Gordon, Nolan Campbell, Caroline Brethenoux, Patrick Furey, Alex Lorenzo, Alyssa DeLace, Rosario Alvarez, Laura González Quijano, Zia Choudhry, Nikita Maniar	<p>INTRODUCTION: Neonatal Fc receptor (FcRn) inhibitors are a promising therapeutic option for individuals with myasthenia gravis (MG). Treatment dosing and administration may affect the overall patient experience and treatment satisfaction.</p> <p>OBJECTIVE: To understand unprompted perceptions of the dosing and administration of FcRn inhibitors among patients with MG and those specifically with FcRn treatment experience.</p> <p>METHODS: US-based digital public-domain conversations among adults who engaged in online conversations about MG over a 12-month period (August 2023 to August 2024) were analyzed. Advanced search and artificial intelligence-powered algorithms were used to extract and organize data. Natural language processing identified frequent topics, sentiments, and key drivers of sentiments.</p> <p>RESULTS: Overall, 16,285 unique public-domain digital conversations about dosing and administration of treatment among patients with MG were extracted from message boards (42%), topical sites (33%), comments (9%), social networks (8%), and blogs (8%); of these, 14,301 (88%) were from self-identified FcRn treatment users. The majority of conversations conveyed negative sentiment for both the overall patient cohort (56%) and treatment users (61%), with the remainder of sentiment neutral in tone. No positive sentiment towards dosing/administration was identified. The most common drivers of negative sentiment were dosing and administration burden (overall: 21%; treatment: 24%), trial-and-error treatment plan (overall: 26%; treatment: 21%), and complex cycle regimens (overall: 20%; treatment: 20%). Unclear dosage standards (overall: 19%; treatment: 18%) and infusion issues (overall: 14%; treatment: 17%) were less common.</p> <p>SUMMARY/CONCLUSION: Digital conversations among patients with MG revealed considerable frustration and uncertainty surrounding the dosing and administration of FcRn inhibitors, highlighting opportunities for improvement.</p> <p>INTRODUCTION: Exacerbations and crises in generalized myasthenia gravis (gMG) are associated with increased healthcare resource utilization (HURU). Identifying characteristics and symptoms of at-risk patients is essential to optimize resource allocation to preventative strategies.</p> <p>OBJECTIVE: To evaluate factors associated with exacerbations or crises in US patients with gMG.</p> <p>METHODS: Adults with gMG were identified from Komodo Research Database (01/2017-09/2023). Index date was the first gMG diagnosis by a neurologist. Baseline (12 months pre-index) demographics, comorbidities, gMG-related treatments, and HURU were analyzed. A multivariable Cox proportional hazards model assessed factors associated with post-index clinical events (i.e., exacerbations/crises). Exacerbations and crises were defined based on diagnosis codes, care settings, and airway management procedures. Patients without an event were censored at insurance or data end.</p> <p>RESULTS: Among 6,195 patients (mean age: 61.1 years; female: 49.1%), 49.2% experienced an event (exacerbation: 48.8%; crisis: 3.1%) on average 5.4 months post-index. In the adjusted model, higher event risk was associated with male gender (hazard ratio [HR]=1.15; p=0.017), gMG symptoms, including dysarthria (HR=1.45; p<0.001), dysphagia (HR=1.28; p<0.001), dyspnea (HR=1.23; p=0.001), ptosis (HR=1.20; p=0.001), general and other muscle-specific weaknesses (HR=1.38; p<0.001), and weight loss (HR=1.27; p=0.009), absence of other autoimmune disorders (HR=1.18; p=0.040), use of immunoglobulin (HR=1.54; p<0.001) and non-steroidal immunosuppressants (HR=1.37; p=0.026).</p> <p>SUMMARY/CONCLUSION: Almost half of patients experienced an exacerbation/crisis within ≤3 years of diagnosis. Weakness symptoms, and the need for acute care therapy were the primary factors associated with exacerbations and crises. These findings underscore the need for more effective symptom management and proactive disease control.</p>
32	FACTORS ASSOCIATED WITH EXACERBATIONS OR CRISES IN GENERALIZED MYASTHENIA GRAVIS	Kavita Grover, Kavita Gandhi, Martin Cloutier, Geoffrey Coteur, Nida Imran, Maryia Zhdanova, Antoine El Khoury, Porpong Boonmak, Anabelle Tariif-Samson, Yuxi Wang, Zia Choudhry, Nicholas Silvestri	<p>INTRODUCTION: Tailored exercise is safe for individuals with mild-moderate myasthenia gravis (MG) and improves muscle strength and physical function. Data are limited regarding participation in physical activity (PA), adherence to exercise threshold (ET) recommendations, and physical therapy (PT) participation among MG patients.</p> <p>OBJECTIVE: 1) To report rates and characteristics of adults with MG participating in PA and meeting ET. 2) To determine whether participation in PT is associated with higher rates of PA and/or meeting ETs.</p> <p>METHODS: Demographic, MG and PA characteristics from The MG Foundation of America's (MGFA's) Global Patient Registry were analyzed using descriptive, univariate and multivariate analyses provided by Alira Health including individuals with baseline enrollment between from July 2013-October 2024.</p> <p>RESULTS: Of 2,941 participants, most were white (80%), female (62%), aged 40-64 (52%) and with household income <\$100,000 (67%). 61% reported PA; 23% met recommended ET of ≥ 150 minutes/week of moderate PA. PT participation was 17% in no-exercise, 18% in PA, and 14% in ET groups (p<0.075). In multivariate analysis, individuals with household income <\$100,000 (p<0.001), female sex (p<0.003) and higher MG-ADL score (p<0.001) were less likely to engage in PA. Similarly, individuals with household income <\$100,000 (p<0.029), female sex (p<0.001) and higher MG-ADL scores (p<0.036) were less likely to meet ET.</p> <p>SUMMARY/CONCLUSION: Rate of meeting ET among individuals with MG is low. Participation in PT did not increase the likelihood of meeting ET in this group. Increasing ET attainment is an unmet need which could augment pharmacotherapies for individuals with MG.</p>
33	PHYSICAL ACTIVITY AND FACTORS ASSOCIATED WITH EXERCISE PARTICIPATION AMONG INDIVIDUALS IN THE MGFA PATIENT REGISTRY	Zoe Sheitman, Amanda Guidon	<p>INTRODUCTION: Gefurulumab, a novel dual-binding nanobody, inhibits complement component 5 and is in clinical development for anti-acetylcholine receptor antibody-positive (AChR-Ab+) generalized myasthenia gravis (gMG). Its low molecular weight enables subcutaneous self-administration by AI or PFS. The HF validation study ensures user interfaces are safe and effective for intended users, uses, and use environments.</p> <p>OBJECTIVES: Demonstrate gefurulumab AI and PFS user interfaces are safe and effective for use by patients, caregivers, and healthcare providers (HCPs) in intended use environments.</p> <p>METHODS: Patients with gMG and caregivers of patients with gMG were randomized to a device type (AI/PFS containing surrogate solution) and dosage. HCPs were assigned to the AI. Participants simulated use tasks and answered knowledge-based questions. Moderators queried participants about use deviations and experience and assessed performance by identifying use issues and their root causes. Participants rated their experience via questionnaires, were unprompted to review instructions for use, and were untrained, representing a worst-case scenario.</p> <p>RESULTS: 75 participants (30 patients, 30 caregivers, 15 HCPs) were enrolled. Most participants successfully used the device during injection simulation: AI, 95.6% (total, 43/45; patients, 15/15; caregivers, 14/15; HCPs, 14/15); PFS, 93.3% (total, 28/30; patients, 13/15; caregivers, 15/15). Use deviations (errors, deviations, close calls) were observed in ~5% of all use tasks (AI, 4%, PFS, 6%) with no consistent patterns of use issues observed with critical tasks. Patients rated user experience positively (AI, 93%-100%; PFS, 87%-93%).</p> <p>SUMMARY/CONCLUSION: This HF validation study demonstrates that gefurulumab AI and PFS user interfaces can be used safely and effectively by intended users with positive user experiences.</p>
34	USABILITY OF GEFURULIMAB AUTOINJECTOR (AI) AND PREFILLED SYRINGE (PFS) DEVICES: A HUMAN FACTORS VALIDATION STUDY	Kelly Gwathmey, Panru Jing, Scott Laorr, Joe Koo, Sanjay Rakhade, Emmett Alton Sartor, JT Tibung, Jason Murray, Christina Laskar	

35	RESET-MG: CLINICAL TRIAL EVALUATING RESE-CEL (RESECBARTAGENE AUTOLEUCEL), A FULLY HUMAN, AUTOLOGOUS 4-1BB CD19-CAR T CELL THERAPY IN GENERALIZED MYASTHENIA GRAVIS	Ali Habib, Christina Ulane, Ran Reshef, Jonathan Hogan, Yvonne White, Jennifer Gresh, Wayne Hall, Shelby Wilkinson, Carl DiCasoli, Jemel Volkov, Thomas Furmanak, Daniel Nunez, Samik Basu, Raj Tummalala, David Chang	<p>INTRODUCTION: Generalized myasthenia gravis (gMG) is a B-cell-mediated autoimmune disease, with most patients having autoantibodies directed against the neuromuscular junction. Most available treatments require chronic administration for sustained efficacy, which may increase the risk of side effects, and many patients remain refractory despite several treatments. Rese-cel (formerly CABA-201) is an investigational, fully human, autologous 4-1BB CD19-CAR T cell therapy, designed to deeply and transiently deplete CD19 positive cells following a single, weight-based infusion, with the potential to enable an "immune system reset" with durable responses.</p> <p>OBJECTIVE: RESET-MG (NCT06359041) is a Phase 1/2 trial evaluating the safety and efficacy of rese-cel in 2 independent cohorts of anti-AChR-antibody positive and anti-AChR-antibody negative patients.</p> <p>METHODS: Eligible patients are 18 to 70 years of age with gMG, MGFA Classification II-IV disease, and an MG-ADL score of ≥ 6 despite ≥ 2 prior or current treatments. A single infusion of 1×10^6 CAR T cells/kg is administered following preconditioning with fludarabine and cyclophosphamide. All non-glucocorticoid immunomodulatory agents are discontinued by preconditioning and glucocorticoids are subsequently tapered. Adverse events, MG medication use, MG-ADL, QMG and translational endpoints are assessed.</p> <p>RESULTS: No serious adverse events or dose limiting toxicities reported through week 4 in the first patient infused with rese-cel. Rese-cel peaked Day 8 post-infusion, with B cells depleted by 8 days post-infusion through week 4. Additional patients are enrolled, and clinical trial updates will be shared at the meeting.</p> <p>SUMMARY/CONCLUSION: Rese-cel was well-tolerated by the first patient infused in the RESET-MG trial.</p>
36	REPEATED CYCLES OF ROZANOLIXIZUMAB TREATMENT IN PATIENTS WITH ANTI-MUSCLE-SPECIFIC TYROSINE KINASE ANTIBODY-POSITIVE GENERALIZED MYASTHENIA GRAVIS	Ali A. Habib, Artur Druzdz, Dale J. Lange, Renato Mantegazza, Hiroyuki Naito, Robert M. Pascuzzi, Sabrina Sacconi, Kimiaki Utsugisawa, John Vissing, Tuan Vu, Jann-Hong Yeh, Fiona Grimson, Irene Palido-Valdeolivas, Thais Tarancón, Vera Bril	<p>INTRODUCTION: MycarinG (Phase 3, MG0003/NCT03971422) demonstrated efficacy of six once-weekly rozanolixizumab infusions versus placebo in adults with generalized myasthenia gravis (gMG). Patients could then enroll in open-label extensions MG0004 (NCT04124965) then MG0007 (NCT04650854), or MG0007 directly.</p> <p>OBJECTIVE: Evaluate the efficacy and safety of repeated rozanolixizumab cycles in patients with anti-muscle-specific tyrosine kinase antibody-positive (MuSK Ab+) gMG.</p> <p>METHODS: In MG0004, patients received once-weekly rozanolixizumab infusions for ≤ 52 weeks. In MG0007, following an initial rozanolixizumab cycle, subsequent cycles were administered upon symptom worsening (investigator's discretion). Final rozanolixizumab data (7 mg/kg and 10 mg/kg) were pooled across MycarinG, MG0004 (first 6 weeks) and MG0007 for patients with ≥ 2 symptom-driven cycles (efficacy) and MycarinG/MG0007 for patients with ≥ 1 treatment cycle (safety). Efficacy outcomes included change from baseline (CFB) to Day 43 in each cycle for myasthenia gravis (MG)-specific scores.</p> <p>RESULTS: Overall, 12/129 patients who received ≥ 2 symptom-driven rozanolixizumab cycles had MuSK Ab+ gMG. Across Cycles 1-9, mean (standard deviation) CFB in MG-Activities of Daily Living score ranged from -3.0 (3.6; Cycle 5, n=8) to -7.0 (3.5; Cycle 1, n=12). Mean CFB in MG Composite and Quantitative MG scores ranged from -5.8 (7.4; Cycle 9, n=5) to -13.9 (6.6; Cycle 1, n=12) and -5.2 (1.9; Cycle 8, n=5) to -10.6 (6.0; Cycle 3, n=8), respectively. Treatment-emergent adverse events occurred in 14/18 (77.8%) patients with MuSK Ab+ gMG who had ≥ 1 treatment cycle, most were mild or moderate.</p> <p>SUMMARY/CONCLUSION: Rozanolixizumab efficacy in patients with MuSK Ab+ gMG was maintained over repeated treatment cycles, with an acceptable safety profile. Funding: UCB.</p>
37	EFgartigimod USE IN A PREGNANT FEMALE WITH MYASTHENIA GRAVIS	Spencer Hall, Rabia Choudry, Naraharisetty Anita Rao	<p>INTRODUCTION: A 26-year-old female with a medical history of rheumatoid arthritis in remission, costochondritis, adjustment / anxiety disorder, and spontaneous pneumothorax. In 2016 was diagnosed with antibody positive myasthenia gravis at age 24. She was started on Efgartigimod after initially experiencing minimal symptom improvement on Pyridostigmine and Prednisone. She underwent thyrectomy after completing one cycle of Efgartigimod and did not require prophylactic IVIG.</p> <p>OBJECTIVE/METHODS/RESULTS: Patient was G1P1 at the time of her next scheduled cycle of Efgartigimod, but after four doses, patient discovered she was pregnant for the duration of those four doses. The medication was stopped, and patient was followed by neurology and gynecology for the rest of her pregnancy. Patient developed premature rupture of membranes two weeks prior to due date, was induced by oxytocin, and then underwent a Cesarean section due to non-reassuring fetal heart rate. The child was then born without complications and has no reported medical issues to date over one year since birth.</p> <p>SUMMARY/CONCLUSION: Efgartigimod was approved in 2021 in the United States for acetylcholine receptor antibody positive myasthenia gravis. As this medication has only recently been approved, there is minimal literature for its use in pregnant females. An observational study from the medication company is ongoing, using a pregnancy registry to eventually determine safety of use in pregnancy. This patient became pregnant during her cycle of infusions and later gave birth to a healthy child without complications to child or patient. Patient has since been placed on Efgartigimod / hyaluronidase subcutaneous injections with significant symptom control.</p>
38	HIGH CARDIOVASCULAR DISEASE BURDEN AMONG PATIENTS WITH MYASTHENIA GRAVIS IN US	Kristin Heerlein, Jana Podhorna, Cecile Blein, Charlotte Ward, Cynthia Qi, Rahul Malik, Ami Shah, Eliot A Brinton, Jeffrey Rosenfeld	<p>INTRODUCTION: Immune-mediated diseases increase risk of cardiovascular disease (CVD), the leading cause of death and disability in the US. Immunosuppressive treatments, particularly corticosteroids, may further increase CVD risk. In patients with myasthenia gravis (MG), CVD risk and the effect of corticosteroid treatment on CVD risk are poorly understood.</p> <p>OBJECTIVE: To describe the prevalence of CVD and CVD risk factors in US patients with MG.</p> <p>METHODS: Optum® Market Clarity claims data (2022-2023) from patients with ≥ 2 MG-related claims in 2 years were assessed for CVD and CVD risk factors using International Classification of Diseases tenth revision (ICD-10) codes. We stratified patients by degree of steroid use (none, short-, or long-term [≥ 90 cumulative days]) and age.</p> <p>RESULTS: Among 4,758 patients with MG (mean age, 63 years; 53% female), 64% currently used steroids. Over 90% had ≥ 1 CVD risk factor and $>50\%$ had ≥ 3 risk factors, with most common being hypertension (70%), hyperlipidemia (70%), and obesity (49%). CVD was prevalent in $>40\%$, most commonly chronic ischemic heart disease (27%), coronary atherosclerosis (13%), and cerebrovascular accidents (8%). CVD and its risk factors were most prevalent in long-term steroid users. CVD prevalence remained high across all age groups.</p> <p>SUMMARY/CONCLUSION: Patients with MG have considerable CVD burden, irrespective of age. CVD risk reduction requires control of modifiable risk factors and use of steroid-sparing treatment as possible in managing MG. These findings emphasize the need to consider the potential impact of comorbidities when making thoughtful treatment choices in patients with MG who have CVD and/or CVD risk.</p>
39	RITUXIMAB FOR REFRACTORY SEROPOSITIVE MYASTHENIA GRAVIS (MG)	Irum Hina, Rohma Ahmed, Ehtesham Khalid	<p>INTRODUCTION: Rituximab is an anti-CD20 chimeric monoclonal antibody which has been in market since 1997 for hematological malignancies, but it has proven effectiveness for multiple other inflammatory disorders including rheumatoid arthritis and myasthenia gravis. We would like to present a case who was maintained on Rituximab after diagnosis for period of approximately 4 years.</p> <p>OBJECTIVE: Non-conventional treatment of myasthenia gravis</p> <p>METHODS: 48-years-old male with initial symptoms of shortness of breath on exertion, orthopnea, swallowing difficulty and mild proximal weakness who was diagnosed with strongly positive acetylcholine receptor antibodies (titer-15.8). Computed tomography of chest was negative for abnormal thymic tissue. He was treated in intensive care for 20 days with tracheostomy and plasma exchange in first year of diagnosis. He was started on Rituximab within a year of symptoms onset beside low dose steroids. He did well for three years on low dose steroids and received Rituximab again in 2024 as 6 monthly regular infusion.</p> <p>RESULTS: There was improvement in symptoms of Myasthenia gravis after starting rituximab. He developed urinary tract infections due to uncontrolled diabetes and enlarged prostate but otherwise did well in terms of infections during this period of 4 years.</p> <p>SUMMARY/CONCLUSION: This case highlights the use of rituximab as maintenance therapy with good control of refractory seropositive myasthenia gravis with minimally increased chances of infection. It also proves convenience of 6 monthly dosing beside efficacy of treatment within a year of symptom onset.</p>

			<p>INTRODUCTION: Patients with myasthenia gravis (MG) experience burdens including difficulty breathing and sleeping, severe fatigue, reduced participation in daily activities, and mental health decline.</p> <p>OBJECTIVE: To assess patient versus provider awareness of disease burden and minimal symptom expression (MSE) in clinical practice. Perceptions regarding MG activities of daily living (MG-ADL) and MG quality-of-life (QoL) scores were also assessed.</p> <p>METHODS: A 20-minute online survey was conducted among patients with generalized MG (gMG) and providers in the US from October to November 2024.</p> <p>RESULTS: 202 patients and 140 providers completed the survey. Forty percent of patients reported high disease burden, whereas 21% of providers felt their patients had high disease burden. Patients and providers generally agreed that lower MG-ADL scores aligned with well-managed disease. However, 31% of providers and only 19% of patients perceived scores of ≥ 10 as the highest acceptable long-term score. Patients were twice as likely as providers to report high negative impact of gMG on multiple life aspects (lifestyle adjustments due to symptoms, treatments, and side effects). Providers estimated 31% of patients achieved MSE, versus 10% of patients reported achieving MSE. Despite data on MSE attainment with newer medications, only 30% of patients and ~50% of providers responded that MSE was an achievable outcome.</p> <p>SUMMARY/CONCLUSION: While patients and providers recognized MG-ADL as an important measure of disease severity, significant disconnects exist regarding acceptable total scores, disease burden, the negative impact of gMG on QoL, and the likelihood of attaining MSE. Continued education on MG-ADL and gMG burden is critical to bridge these gaps.</p>
40	DIFFERENCES BETWEEN PATIENT AND PROVIDER PERCEPTIONS IN ASSESSING MYASTHENIA GRAVIS DISEASE BURDEN AND MINIMAL SYMPTOM EXPRESSION: A REAL-WORLD SURVEY	Tuan Vu, Mamatha Pasnoor, Arash Mahajerin, Brant Hubbard, Lauren G Jarman, Jeffrey Rosenfeld	
41	COMPARATIVE ANALYSIS OF PATIENT SOCIAL MEDIA SENTIMENT FOR VYVGART IN gMG COMPARED TO TREATMENTS IN OTHER COMPLEX DISEASE AREAS	Tom Hughes, Kaushik Bhattacharya, Aditya Batra, Shreyas Jarmale, Anshul Surinder Sharma	<p>INTRODUCTION: Social media data is a valuable complement to traditional data sources, capturing unfiltered and unprompted patient sentiment about disease burden, diagnosis, treatment, quality-of-life, and insurance challenges. Comparing patient sentiment towards VYVGART for generalized myasthenia gravis with treatments for other complex diseases provides helpful context to interpret sentiment results.</p> <p>OBJECTIVE: To compare positive, negative and neutral patient sentiment for VYVGART in gMG against treatments used in other complex diseases.</p> <p>METHODS: English-language social media posts on VYVGART in gMG and comparator treatments (vasomotor symptoms (VMS), partial-onset seizures, and multiple myeloma (MM)) were sourced via Brandwatch. An average 12-month analysis timeframe across treatments ensured accurate benchmarking. Sentiment analysis compared the distribution of positive, negative and neutral sentiment across drug classes.</p> <p>RESULTS: 49% of VYVGART-related patient posts were positive compared to an average of 30% across three treatments for VMS, partial-onset seizures, and MM. 27% of VYVGART-related patient posts were negative compared to an average of 26% across three treatments for VMS, partial-onset seizures, and MM. 24% of VYVGART-related patient posts were neutral compared to an average of 44% across three treatments for VMS, partial-onset seizures, and MM.</p> <p>SUMMARY/CONCLUSION: Compared to treatments for VMS, partial-onset seizures and MM, VYVGART generated higher positive sentiment, comparable negative sentiment and lower neutral sentiment among patients. These findings suggest a favorable real-world perception of VYVGART and reinforce the value of social media in capturing stakeholder experiences.</p>
42	ASSESSING SOCIAL MEDIA SENTIMENT TO ANALYZE PATIENT, CAREGIVER AND HEALTHCARE PROFESSIONALS EXPERIENCE WITH VYVGART AND VYVGART HYTRULO FOR GENERALIZED MYASTHENIA GRAVIS	Tom Hughes, Albert Whangbo, Kaushik Bhattacharya, Aditya Batra, Shreyas Jarmale, Anshul Surinder Sharma	<p>INTRODUCTION: Social media data is a valuable complement to traditional data sources, capturing unfiltered and unprompted patient, caregiver and healthcare professional sentiments about treatments, quality-of-life, steroid use reduction, cost burdens and insurance challenges.</p> <p>OBJECTIVE: Categorize and quantify VYVGART and VYVGART Hytrulo gMG posts from patients, caregivers, and healthcare-professionals, identifying trends, concerns, and shared experiences; analyze subtopics and sentiments to provide global insights across stakeholders; and provide findings to inform future research strategy, and tactics.</p> <p>METHODS: Social media posts related to VYVGART and VYVGART Hytrulo in gMG were identified using predefined keywords via Brandwatch. Posts were categorized by stakeholders, and analyzed using ZS GenAI algorithms with manual validation. Content was structured into four major themes—brand awareness, access, treatment experience, and treatment support.</p> <p>RESULTS: 1046 relevant posts by patients, caregivers, and healthcare professionals in English were analyzed globally, posted from August-2021 to September-2024 on social-media platforms. 49% of patient sentiment for VYVGART were positive, highlighting symptom relief, improved quality-of-life, and convenience, though challenges like insurance issues, side effects, and varied treatment effectiveness were also reported. 43% of caregiver sentiment was neutral, highlighting their patient's experiences on VYVGART, seeking insights from others, noting improved quality-of-life, but were concerned about cost, eligibility, insurance challenges. 78% of healthcare-professionals sentiment was neutral, highlighting VYVGART's efficacy, FDA approval, trial results, and high-cost.</p> <p>SUMMARY/CONCLUSION: Social media conversations on VYVGART and VYVGART Hytrulo revealed distinct perspectives – patients noted improved quality-of-life, caregivers highlighted access and eligibility concerns, while healthcare-professionals focused on treatment efficacy, trial results, and high cost.</p>
43	SAFETY PROFILE OF NIPOCALIMAB, A NEW NEONATAL FRAGMENT CRYSTALLIZABLE RECEPTOR BLOCKER IN THE PHASE 3 VIVACITY-MG3 STUDY	Hans Katzberg, Maria Ait-Thiyyat, Ibrahim Turkoz, Kavita Gandhi, John Sheehan, Sindhu Ramchandran	<p>INTRODUCTION: Nipocalimab demonstrated efficacy and tolerable safety in phase-2 VIVACITY-MG and phase-3 Vivacity-MG3 study (NCT04951622).</p> <p>OBJECTIVE: To report comprehensive safety profile of nipocalimab from double-blind (DB), placebo-controlled Vivacity-MG3 study in adults with generalized myasthenia gravis (gMG) and its open-label extension (OLE) phase.</p> <p>METHODS: The safety analysis sets (DB and OLE) included all patients receiving ≥ 1 dose (partial-complete) of any study treatment in that phase. Patients completed/discontinued DB phase could enter the ongoing OLE phase.</p> <p>RESULTS: From DB phase, 196 (nipocalimab:placebo: 98/98) and of whom 176 (nipocalimab:placebo: 88/88) patients who entered OLE were included in safety analysis (median follow-up: DB/OLE: 24/72-weeks). The proportion of patients experiencing ≥ 1 adverse events (AEs) in DB and OLE phase was similar between the groups; most common AEs: headache, MG, nasopharyngitis. Overall, 12(6.1%) patients in DB phase and 13(7.4%) in OLE discontinued treatment due to AEs. In DB phase, 23(11.7%) and in OLE 44(26.1%) patients experienced ≥ 1 SAE. There were 7 deaths (DB, n=3; OLE, n=4). Mild increases in total cholesterol, HDL, and LDL were observed in nipocalimab-treated patients; in the DB phase, levels decreased by week 8-10 and plateaued by week 24. There was no difference in rate of major adverse cardiovascular events with nipocalimab vs placebo. 10-year cumulative cardiovascular risk estimate (SCORE2) remained similar between nipocalimab-treated and placebo-treated patients after 24-weeks of exposure; this trend was maintained for up to 72-weeks of follow-up through OLE.</p> <p>SUMMARY/CONCLUSION: Nipocalimab was generally well-tolerated in adults with gMG, with no new safety concerns identified over a follow-up period of 96-weeks through DB and OLE.</p>
			<p>INTRODUCTION: Patients with Myasthenia Gravis (MG) may continue to exhibit significant symptom burden despite treatment with FcRn inhibitors and immunosuppressive therapy.</p> <p>OBJECTIVE: Assessment of clinical outcomes in Acetylcholine receptor (AChR) antibody positive generalized Myasthenia Gravis (gMG) patients treated with Zilucoplan alongside ongoing FcRn inhibitors and immunosuppressive therapy.</p> <p>METHODS: A retrospective review was conducted on two AChR+ gMG patients who continued to experience significant symptoms despite treatment with FcRn inhibitors and immunosuppressive therapy. Zilucoplan was introduced as an adjunct therapy. Myasthenia Gravis Activities of Daily Living (MG-ADL) scores were assessed before and after initiating Zilucoplan. Changes in medication regimens and adverse effects were also recorded.</p>
44	CONCOMITANT USE OF COMPLEMENT AND FCN INHIBITORS FOR MANAGEMENT OF REFRACTORY ACETYLCHOLINE RECEPTOR (AChR) ANTIBODY POSITIVE GENERALIZED MYASTHENIA GRAVIS (MG)	Jasmeet Kaur, Raghav Govindarajan, Nakul Katyal	<p>RESULTS: We evaluated two Caucasian female patients, aged 40 and 55, who exhibited persistent symptom burden despite treatment with high-dose prednisone, pyridostigmine, and FcRn inhibitors (either efgartigimod or rozanolixizumab). Prior to the introduction of Zilucoplan, the Mean MG-ADL score was 6.5 (individual scores of 7 and 6). Following Zilucoplan therapy, both patients demonstrated clinically meaningful improvement, defined as a greater than 2-point reduction in MG-ADL score, with a post-treatment mean MG-ADL score of 4 (both scoring 4). The mean daily prednisone dose was reduced from 35 mg to 25 mg. One patient experienced mild, transient burning at the injection site. No serious adverse effects were observed.</p> <p>SUMMARY/CONCLUSION: Our study highlighted that concomitant use of complement and FcRn inhibitors may offer enhanced clinical outcomes in refractory MG. This observation warrants further investigation in larger cohorts.</p>

45	THE EFFECT OF EFGARTIGIMOD ON AChR-AB MEDIATED PATHOGENIC MECHANISMS IN PATIENTS WITH GMG	Fatemeh Khani-Habibbadi, Fien M. Verhaunne, Mahan Moshir, Vijayaraghava Rao, Sophie Steadand, Peter Ulrichts, Minh C. Pham, Bhaskar Roy, Richard Nowak, Kevin O'Connor	<p>INTRODUCTION: Efgartigimod, a neonatal Fc receptor (FcRn) blocker, effectively reduces total IgG, including pathogenic AChR autoantibodies in myasthenia gravis (MG). However, clinical responses vary, potentially due to heterogeneity in antibody-mediated pathogenic mechanisms.</p> <p>OBJECTIVE: To understand how efgartigimod treatment-mediated AChR autoantibody reduction impacts specific pathogenic mechanisms these autoantibodies mediate, including complement activation, AChR internalization and ACh-binding site blockade.</p> <p>METHODS: Serum samples (N=150) were sourced from 50 AChR-Ab+ gMG patients participating in the phase 3 ADAPT study (clinicaltrials.gov:NCT03669588), randomized to receive efgartigimod (N=40) or a matching placebo (N=10) in cycles of 4 weekly infusions. Samples were collected at baseline (day 0), day 29, and day 57 in the first cycle. Live cell-based assays quantified AChR-specific IgG subclasses, and their ability to mediate complement activation (C3b deposition), receptor internalization, and ACh-binding site blockade.</p> <p>RESULTS: Efgartigimod treatment decreased serum levels of all detectable AChR-specific IgG subclasses (IgG1, IgG2, and IgG3). At baseline, AChR-Ab-mediated C3b deposition, AChR internalization and ACh-binding site were detected in 42 (84%), 41 (82%), 10 (20%) patients, respectively. At day 29 (1 week after the 4th infusion), all three AChR-Ab-mediated pathomechanisms were decreased after efgartigimod treatment, whereas no changes were observed in the placebo group. However, variation in the magnitude of reduction was observed across individuals.</p> <p>SUMMARY/CONCLUSION: Efgartigimod reduces all AChR autoantibody-driven pathogenic mechanisms in MG; however, the magnitude of this reduction varies among patients. Understanding the variability in these reductions and their impact on clinical outcomes is crucial for optimizing treatment strategies.</p>
46	ECULIZUMAB-AEEB BIOSIMILAR TO ECULIZUMAB REFERENCE PRODUCT - PHARMACOKINETIC/PHARMACODYNAMIC DATA TO FURTHER SUPPORT CLINICAL SIMILARITY AND SCIENTIFIC JUSTIFICATION FOR EXTRAPOLATION	Austin Kulasekararaj, Jia Cao, Benjamin Shander, Daniel Mytych, Vincent Chow, Jennifer Liu, Haby Henary	<p>INTRODUCTION: Eculizumab-aeeb (BKMV%, previously ABP 959) is an FDA-approved biosimilar to eculizumab reference product (RP) for the treatment of paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS) and generalized myasthenia gravis (gMG), with FDA interchangeability designation. Eculizumab, a monoclonal antibody that inhibits complement component-5 protein, is also authorized for treating neuromyelitis optica spectrum disorder (NMOSD). Eculizumab-aeeb exhibits similar characteristics as the RP, including complement pathway inhibition, and function in simulated models of aHUS and NMOSD. Clinical comparability of eculizumab-aeeb and RP has been confirmed in patients with PNH.</p> <p>OBJECTIVE: To provide additional pharmacokinetic/pharmacodynamic data to support the totality of evidence and scientific justification for extrapolation across all approved indications that share a common mechanism of action (MoA).</p> <p>METHODS: Pharmacokinetic/pharmacodynamic equivalence of eculizumab-aeeb and eculizumab-RP (US and EU) was assessed in a randomized, double-blind, single-dose, 3-arm, parallel-group trial in healthy adults (N=219).</p> <p>RESULTS: The geometric means and geometric means coefficient of variation of area under the total serum concentration–time curve from time 0 extrapolated to infinity (AUC_{0-∞}, h·µg/mL) for unbound drug was: eculizumab-aeeb, 5072.1 (25); eculizumab-US, 5527.6 (31); and eculizumab-EU, 5070.3 (31). Area-between-the-effect curve for 50% total hemolytic complement activity for unbound eculizumab-aeeb, eculizumab-US, and eculizumab-EU was also similar.</p> <p>SUMMARY/CONCLUSION: Drug exposure and complement activity were similar with eculizumab-aeeb and eculizumab-RP. Along with the consistent MoA across all RP indications, the comprehensive totality of evidence demonstrating similarity of eculizumab-aeeb with eculizumab-RP supports extrapolation for all RP indications.</p> <p>INTRODUCTION: Rozanolixizumab, a neonatal Fc receptor blocker, is approved for treatment of adults with anti-acetylcholine receptor (AChR) or anti-muscle-specific tyrosine kinase antibody-positive (Ab+) generalized myasthenia gravis (gMG).</p> <p>OBJECTIVE: To understand the profile of patients initiating rozanolixizumab treatment in the United States (US).</p> <p>METHODS: This retrospective, observational cohort study included adults with gMG who were enrolled in a patient support program (ONWARDSM) and/or had specialty pharmacy data. De-identified data (July 2023–February 2025) were analyzed descriptively. The primary objective was to describe patient, disease and treatment characteristics and rozanolixizumab utilization at baseline. Describing utilization patterns during follow-up was a secondary objective.</p> <p>RESULTS: 877 patients with a myasthenia gravis (MG) diagnosis and rozanolixizumab initiation at baseline were included. 452/877 (51.5%) were female and mean (standard deviation [SD]) age was 62.2 (16.6) years. Most patients had MGFA Disease Class II or III (168/439 [38.3%] and 203/439 [46.2%], respectively) and anti-AChR Ab+ gMG (432/591 [73%]); the mean (SD) MG Activities of Daily Living score (n=695) was 8.4 (3.8). The most common prior therapies were oral corticosteroids (324/546 [59.3%]) and IVig (229/546 [41.9%]). Mean (SD) follow-up for patients with ≥1 additional treatment date (N=393) was 234.2 (33.8) days with a mean (SD) 41.6 (26.1) days between cycles. Patients with ≥1 year of follow-up since rozanolixizumab initiation (n=100) started a mean 3.7 cycles in Year 1.</p> <p>SUMMARY/CONCLUSION: In the US, rozanolixizumab treatment has been initiated in a broad population of patients with gMG, with treatment patterns generally consistent with those observed in clinical trials (mean 4.1 cycles). Funding: UCB.</p> <p>INTRODUCTION: Myasthenia gravis (MG) is an autoimmune neuromuscular disorder characterized by fluctuating motor weakness due to immunological attack against post synaptic membrane of neuromuscular junction.</p>
47	CHARACTERISTICS OF PATIENTS WITH GENERALIZED MYASTHENIA GRAVIS INITIATING ROZANOLIXIZUMAB TREATMENT IN THE UNITED STATES	Minjee Park, Angela Ting, Aaksha Nair, Alexandria Harrold, Juachen Zhou, Mohita Kumar	<p>CASE REPORT: I am 32 year old female neurologist post partum 2 months, presented with ptosis, fluctuating flaccid dysarthria, proximal muscle weakness with power of 4/5, poor gag and cough reflex, unable to blow or whistle with MG Composite scale of 27. In view of fluctuating weakness pyridostigmine and subsequently steroids were initiated and noticed improvement. Anti acetylcholine receptor (Anti-AChR), anti titin, anti- Low density lipoprotein receptor-related protein 4 (anti LRP4) antibodies were positive. Immunoglobulin was given. But, I failed to respond. I desaturated after 2 weeks with impending bulbar crisis, immediately IV plasmapheresis was initiated and there after thymectomy was done. Asymptomatic, for 10 days and worsened again. I have taken 2 doses of rituximab and noticed worsening of symptoms. Stabilized with iv neostigmine without intubation and recovered slowly.</p> <p>CONCLUSION: Interesting aspect in this case is presence of triple antibodies ,rapid progression, severity of disease, not responding to conventional rescue therapies like immunoglobulins, plasmapheresis and worsening of bulbar symptoms post rituximab . Clinical symptoms of anti- AChR positive MG combined with titin antibodies are associated with more severe and rapid progression of disease. The hypothesis of post rituximab treatment worsening include antibodies release from degraded lymphocytes, increased activity of cholinesterase and increase in immune reactions.</p> <p>INTRODUCTION: Myasthenia gravis (MG) and neuromuscular education is strongly needed among neurology residents.</p> <p>OBJECTIVE: To study the effects of a combined intervention on the attitude towards MG and neuromuscular disease.</p> <p>METHODS: The combined intervention comprised two NCS/EMG lectures, one two-hour hands-on session, regular discussion of cases with MG and other neuromuscular conditions during morning conference, and a grand round. A survey was performed before and repeated six months after the intervention. Answers ranged from 0 to 5 (0, not at all; 1, a bit; 2, less than average; 3, average; 4, above average; 5, very well).</p>
48	"ANTIBODIES AT PLAY : A NEUROLOGIST'S ROLLERCOASTER RIDE WITH TRIPLE POSITIVE MYASTHENIA"	Kunkala Lavanya	<p>RESULTS: 33 and 25 residents responded to the survey before and after the intervention. Residents who could sufficiently (scores 4&5) identify signs and symptoms of MG grew from 54.6 % to 64%, and those choosing "5" rose from 9.1% to 36% (P=0.040). Similarly, residents who feel very comfortable with examining MG patients grew from 9.1% to 36% (P=0.040) with an increased score (3 [3, 4] vs 4 [3, 5], P=0.027). Residents very familiar with treatment options also grew from none to 20% (P=0.011) with an increased score (3 [3, 3] vs 3 [3, 4], P=0.039). In terms of neuromuscular diseases, 12% and 16% of residents chose "very well" for knowledge and confidence after intervention, respectively, while none chose it before. "Lack of patient population/exposure" was the most significant barrier to MG care. Most residents believed hands-on NCS/EMG and case discussion could help improve neuromuscular education.</p> <p>CONCLUSION: A six-month combined intervention helped improve resident attitude towards MG and neuromuscular education.</p>
49	THE EFFECTS OF SIX-MONTH INTERVENTION ON THE ATTITUDE TOWARDS MYASTHENIA GRAVIS AND NEUROMUSCULAR DISEASE AMONG NEUROLOGY RESIDENTS	Mao Liu, Kereisha Donegal, Anziska Yaacov	

50	RETROSPECTIVE ANALYSIS OF EFFICACY AND OUTCOMES OF DAILY VERSUS ALTERNATE DAY PLASMA EXCHANGE IN MYASTHENIC CRISIS: A SINGLE CENTER EXPERIENCE.	Alexis Lizarraga, Phillip Mongiovi	<p>INTRODUCTION: Plasmapheresis is first-line therapy for myasthenic patients with severe weakness. There is no consensus on the optimal schedule of plasma exchanges (PLEX) in manifest or impending myasthenic crisis (MC). One 2005 study of 33 patients found no difference between daily versus alternate day PLEX.</p> <p>OBJECTIVE: To clarify outcomes of daily versus every other day or greater PLEX in MC.</p> <p>METHODS: This is an observational retrospective cohort study of inpatients admitted for MC. Primary outcome measures were total duration of hospital stay and MG-ADL score at outpatient follow up. Daily PLEX was defined as ≥ 3 consecutive PLEX, and qOD PLEX as ≥ 3 non-consecutive PLEX.</p> <p>RESULTS: Of 30 total patients, 24 were AchR Ab (+), 2 MuSK Ab (+), 1 LRPA Ab (+) and 3 seronegative. Average age was 58.4 years. 15 patients had daily PLEX and 15 patients had qOD PLEX. 4 patients were intubated in the daily PLEX group and 3 in the qOD PLEX group. Mean hospital length of stay for the daily PLEX group was 12.5 days, and 14.1 days for the qOD PLEX group, and this difference was not significant ($p=0.65$). Mean MG-ADL score for the daily PLEX group was 5.9, and the mean MG-ADL score for the qOD PLEX group was 4.9 ($p=0.85$).</p> <p>SUMMARY/CONCLUSION: In this small, retrospective cohort, no difference in length of hospital stays or MG-ADL at follow up were seen between patients receiving daily or ≥ 3 qOD PLEX for MC. Larger, prospective studies are needed to clarify optimal PLEX regimens for patients with severe myasthenia gravis.</p>
51	STRUCTURAL INSIGHTS INTO THE HEXAMERIZATION AND COMPLEMENT ACTIVATION OF A HUMAN ANTI-ACETYLCHOLINE RECEPTOR ANTIBODY	Andrew Borbi, Edvin Pozharskiy, Qifang Xu, Purushottam Tiwari, Jie Luo	<p>INTRODUCTION: Myasthenia gravis (MG) is an antibody-mediated autoimmune disease characterized by skeletal muscle weakness and fatigability. The pathogenesis of autoantibodies depends on their specificity and isotype, with most cases being caused by autoantibodies targeting nicotinic acetylcholine receptors (AChRs). Binding to AChRs at the neuromuscular junction and subsequent activation of the complement cascade are the key processes in the pathogenesis of MG. Autoantibodies bind their targets via their variable region and induce effector functions through interactions between their fragment crystallizable (Fc) region and the globular head of complement component C1q. Immunoglobulin (Ig) G exists as a monomer and noncovalent interactions between Fc segments of IgG antibodies facilitate their hexamerization upon binding to endplate AChRs. IgG3 is unique among the IgG subclasses with enhanced effector functions, presumably attributing to its long hinge region and distinct Fc-Fc interface. Combined mutation of residues E345R, I436G, and S440Y, referred to as RGY, promotes hexamer formation in IgG1, IgG2, and IgG4, but not in IgG3. Using AlphaFold2-generated structural models, we identified four key residues on the IgG3 Fc-Fc interface that are distinct from other subclasses. Combining mutations at these residues with the RGY mutation enabled the formation of IgG3 hexamers in solution and significantly enhanced C1q binding to surface-bound IgG3 antibody. Replacing the hinge region with a IgG2 hinge completely abolished C1q binding, despite the antibody retaining similar affinity for the surface antigen. By comparing cryo-electron microscopy structures of hexamers formed by these IgG3 variants, we proposed a novel model for IgG oligomerization on the postsynaptic membrane.</p>
52	PHASE 1 STUDY EVALUATING GEFURILIMAB PHARMACOKINETICS AND SAFETY FOLLOWING DELIVERY VIA AUTOINJECTOR OR PREFILLED SYRINGE IN HEALTHY ADULTS	Alanna McEneny, Xiangyu Cong, Min Yee, Olivia Tong	<p>INTRODUCTION: Gefurilumab, a novel, dual-binding nanobody, binds to C5 and albumin and is in clinical development for the treatment of patients with anti-acetylcholine receptor antibody-positive generalized myasthenia gravis. Gefurilumab has a low molecular weight, enabling subcutaneous (SC) self-injection by autoinjector (AI) or prefilled syringe (PFS).</p> <p>OBJECTIVES: To compare pharmacokinetic (PK) exposure and safety of gefurilumab in healthy adults following a single SC dose administered by AI versus PFS.</p> <p>METHODS: In this phase 1, open-label, randomized, parallel-group study (NCT06208488), healthy participants, aged 18-65yrs, were stratified by weight and randomized equally to 1 of 6 combination groups of device and injection site (abdomen, thigh, upper arm). Participants received 1 SC gefurilumab dose on day 1; visits occurred throughout the 92-day evaluation period. The primary endpoints were PK parameters for each device (areas under the serum concentration-time curve [AUCinf, AUClast], maximum concentration [Cmax]), PK across injection sites, immunogenicity, pharmacodynamics, and safety were also assessed.</p> <p>RESULTS: Overall, 175 participants were randomized: AI (n=87), PFS (n=88). Baseline characteristics were similar between cohorts. Geometric least square mean ratios (90% CI) comparing AUPPS for Cmax, AUCinf, and AUClast were 97.60% (94.46–100.84), 99.64% (96.07–103.34), and 98.82% (95.17–102.60), respectively. Secondary analyses found no meaningful differences in PK parameters across injection sites. Serum free C5 concentrations over time, adverse event (AE) profiles, and antidrug antibody responses were similar between cohorts. Most AEs were mild; no AEs led to discontinuation.</p> <p>SUMMARY/CONCLUSION: Exposure following a single gefurilumab SC dose administered by AI was comparable to PFS; PK parameters were comparable across injection sites. Gefurilumab was well tolerated.</p>
53	EARLY USE OF RITUXIMAB IN MYASTHENIA GRAVIS IN A RESOURCE-LIMITED SETTING: A RETROSPECTIVE COHORT STUDY FROM A TERTIARY CENTER IN PAKISTAN	Zainab Memon, Shanawer Khan, Sara Khan	<p>INTRODUCTION: Myasthenia gravis (MG) is an autoimmune disorder of the neuromuscular junction, most mediated by acetylcholine receptor antibodies. Standard treatments include corticosteroids, acetylcholinesterase inhibitors, and conventional immunosuppressants, while IVIg or plasma exchange is reserved for refractory cases or crises. Rituximab, a CD20-targeting monoclonal antibody, has shown efficacy in treatment-resistant MG, particularly MuSK-positive cases. However, its early use—before crisis—remains underexplored, especially in resource-limited settings where IVIg and frequent hospitalizations are often inaccessible.</p> <p>OBJECTIVE: This study evaluates the efficacy and safety of early rituximab use in such settings.</p> <p>METHODS: In this retrospective cohort study (Dec 2021–June 2024), 12 patients with generalized MG were treated with rituximab and followed for 12 months. Clinical outcomes, including MGFA-Post Intervention Status, corticosteroid dose reduction, and adverse effects, were assessed.</p> <p>RESULTS: Seven patients had a history of myasthenic crisis before rituximab; only one had a recurrence after treatment. Overall, 91.7% showed significant clinical improvement or reduced need for symptomatic and immunosuppressive therapy. At 12 months, MGFA post-intervention status indicated complete stable remission in 10%, pharmacologic remission in 90%, and minimal manifestations in 20%. Mean corticosteroid doses dropped by 22.5 mg after the first rituximab cycle and 16.4 mg after the second. Seventy-five percent experienced no major treatment-related complications.</p> <p>SUMMARY/CONCLUSION: Early rituximab use in generalized MG appears effective and steroid-sparing, with potential to lessen disease burden and healthcare costs in resource-constrained settings.</p>
54	A CASE OF TREATMENT WITH EFARTIGIMOD PH20 SC IN A PATIENT WITH OCULAR MYASTHENIA GRAVIS	J. Douglas Miles	<p>INTRODUCTION: oMG is a form of myasthenia gravis (MG) in which ocular muscles easily fatigue and weaken, causing symptoms including diplopia and ptosis. 12-80% of patients presenting with only ocular symptoms develop generalized myasthenia gravis (gMG).</p> <p>Efartigimod is a human IgG1 antibody Fc-fragment that reduces total IgG levels, including pathogenic IgG autoantibodies, through neonatal Fc receptor blockade. Because Efartigimod IV and SC have demonstrated tolerability and efficacy in patients with gMG, including associated ocular symptoms associated, we hypothesized that this patient would benefit from efartigimod.</p> <p>Patient's oMG treatment followed this dosing scheme: efartigimod PH20 SC 1000mg administered subcutaneously in cycles of 4 once-weekly injections followed by 6 weeks between cycles, with initiation of subsequent cycles determined by clinical judgement.</p> <p>CASE REPORT: 58-year-old male first experienced diplopia April 2022 and presented to hospital with diplopia May 2023. Baseline MG-ADL score was 5 (constant diplopia, daily ptosis). Patient is AChR-Ab positive, MGFA class I. Oral prednisone resulted in monocular vision changes, causing discontinuation. Pyridostigmine provided incomplete symptom relief. Patient worried about job stability due to disease progression. Current oMG treatment includes efartigimod PH20 SC and pyridostigmine 60mg as needed. Clinical improvements occurred one month after last dose of patient's first cycle (MG-ADL 2; diplopia 2, ptosis 0). March 2024 MG-ADL was 3 (diplopia 1, ptosis 2). January 2025 MG-ADL was 1 (diplopia 0, ptosis 1). No adverse effects reported with efartigimod PH20 SC.</p> <p>SUMMARY/CONCLUSION: This case demonstrates potential for symptomatic improvement of oMG when treated with efartigimod PH20 SC, though further study is warranted.</p>

55	TREATMENT UTILIZATION AND CLINICAL OUTCOMES BY SEROSTATUS IN A REAL-WORLD US GENERALIZED MYASTHENIA GRAVIS POPULATION	Lesley-Ann Miller-Wilson, Joe Conyers, Shiva Lauretta Birja, Hannah Connolly, Gregor Gibson, Lincy Lal, Yury Edwards	<p>INTRODUCTION: Generalized myasthenia gravis (gMG) is characterized by impaired transmission at the neuromuscular junction that is primarily driven by autoantibodies, including those directed against the acetylcholine receptor (AChR-Ab+) and other targets (non-AChR-Ab+), although some patients may test as seronegative.</p> <p>OBJECTIVE: To describe treatment utilization and clinical outcomes in gMG patients based on serostatus.</p> <p>METHODS: Data were drawn from the Adelphi gMG II Disease Specific Programme™, conducted in the US from February–August 2024. Neurologists provided cross-sectional and chart-pulled patient data (demographics, Myasthenia Gravis-Activities of Daily Living [MG-ADL] score, clinical events, treatment utilization) in patients with various serostatus.</p> <p>RESULTS: Fifty-two neurologists provided data on 336 patients with gMG (mean [SD] time since diagnosis, 3.8 [5.6] years, 53.9% male, mean [SD] age, 54.9 [13.3] years). Most patients were AChR-Ab+ (73.5%), 10.1% were non-AChR-Ab+, 11.3% were seronegative and 5.1% had unknown serostatus. Maintenance treatment was prescribed in 95.5% of AChR-Ab+ patients (mean [SD] number of regimens used since diagnosis, 1.8 [1.0]), 94.1% of non-AChR-Ab+ patients (1.4 [1.0]), 89.5% of seronegative patients (1.4 [1.0]) and 52.9% of patients with unknown serostatus (0.6 [0.6]). Mean (SD) MG-ADL scores in AChR-Ab+, non-AChR-Ab+, seronegative, and unknown serostatus patients were 4.3 (3.3), 4.9 (4.0), 3.2 (3.1), and 3.8 (3.3), respectively. Since diagnosis, myasthenic crises or symptom exacerbations were reported in 45.8%, 39.4%, 27%, and 7.1% of patients, respectively.</p> <p>SUMMARY/CONCLUSION: Patients with gMG experience clinical events and activity impairment despite treatment and regardless of serostatus. Additional treatment options are needed for all patients to optimize clinical outcomes.</p> <p>INTRODUCTION: Generalized myasthenia gravis (gMG) is a rare neuromuscular disease with a considerable clinical and humanistic burden.</p> <p>OBJECTIVE: To explore concordance between physicians, patients, and caregivers regarding patient symptomatology, quality of life (QoL), and treatment satisfaction.</p> <p>METHODS: Data were from the Adelphi gMG Disease Specific Programme, a cross-sectional survey of neurologists, patients with gMG, and caregivers conducted from February–August 2024. Outcomes were examined within matched physician-to-patient (PhysPat) and physician-to-caregiver (PhysCare) samples.</p> <p>RESULTS: There were 37 PhysPat matches (54.1% female; mean [SD] age, 59.1 [11.2] years; mean [SD] time since diagnosis, 5.8 [4.8] years) and 23 PhysCare matches (69.6% female, mean [SD] age, 46.1 [13.0] years; mean [SD] time since diagnosis, 3.4 [2.5] years). Discordant reporting was observed in both the PhysPat (physician vs patient) and PhysCare (physician vs caregiver) cohorts for patient physical fatigue (PhysPat, 40.5% vs 62.9%; PhysCare, 43.5% vs 82.6%), patient diplopia (PhysPat, 78.4% vs 62.9%; PhysCare, 78.3% vs 56.5%), and the proportion of patients with “good” or “very good” overall QoL (PhysPat, 70.2% vs 54.0%; PhysCare, 56.5% vs 52.1%). Treatment prescriptions were reported by 97.3% of PhysPat physicians and 65.2% of PhysCare physicians; of those, 25.0% and 53.3%, respectively, prescribed complement inhibitors or neonatal fragment crystallizable receptor inhibitors. Regarding treatment satisfaction, participants from both the PhysPat (physician, 13.9%; patient, 19.5%) and PhysCare (physician, 26.6%; caregiver, 26.6%) cohorts indicated “neutral” or “dissatisfied”.</p> <p>SUMMARY/CONCLUSION: Improved communication between physicians, patients, and caregivers is needed to better understand and address patient needs. More targeted therapies are needed to optimize patient care and improve treatment satisfaction.</p>
56	PHYSICIAN, PATIENT, AND CAREGIVER CONCORDANCE IN A REAL-WORLD US GENERALIZED MYASTHENIA GRAVIS POPULATION	Lesley-Ann Miller-Wilson, Joe Conyers, Shiva Lauretta Birja, Hannah Connolly, Gregor Gibson, Lincy Lal, Yury Edwards	<p>INTRODUCTION: Myasthenia gravis (MG) is a rare neuromuscular condition causing muscle weakness and fatigue.</p> <p>OBJECTIVE: To describe physician- and patient-reported outcomes in patients with MG stratified by MG Foundation of America (MGFA) classification.</p> <p>METHODS: Physicians provided patient-level data via the Adelphi MG II Disease Specific Programme™ (DSP) from February–August 2024, a different cohort of patients (PAT) self-reported data in October 2024 via an online survey.</p> <p>RESULTS: Fifty-two physicians reported on 390 DSP patients (mean [SD] age, 55.1 [13.7] years; mean [SD] time since diagnosis, 3.8 [5.6] years; 46.9% female); 243 PAT patients self-reported (mean [SD] age, 49.1 [14.6] years; mean [SD] time since first symptoms, 13.0 [2.2] years; 87.7% female). The proportions of MGFA class I, class II, and class III/IV patients in the DSP cohort were 20.5%, 63.3% and 16.2%; corresponding proportions in the PAT cohort were 6.6%, 30.0%, and 63.4%. In the DSP cohort, mean (SD) MG-Activities of Daily Living scores were 1.9 (1.5), 4.3 (2.9), and 7.9 (3.7) among MGFA class I, II, and III/IV patients, respectively; corresponding scores in the PAT cohort were 4.6 (3.1), 6.5 (3.2), and 9.5 (3.2). MG-related hospitalizations in the year prior to the survey occurred in 7.1%, 16.3% and 18.3% of MGFA class I, II, and III/IV DSP patients, respectively; corresponding proportions in the PAT cohort were 12.5%, 23.3% and 48.7%.</p> <p>SUMMARY/CONCLUSION: Increased activity impairment and worse clinical outcomes were observed with higher MGFA classification in both cohorts. Treatments delivering greater improvements for patients with moderate-to-severe MG are needed.</p>
57	PHYSICIAN- AND PATIENT-REPORTED OUTCOMES BY DISEASE SEVERITY IN A UNITED STATES REAL-WORLD MYASTHENIA GRAVIS POPULATION	Lesley-Ann Miller-Wilson, Joe Conyers, Shiva Lauretta Birja, Hannah Connolly, Gregor Gibson, Lincy Lal, Yury Edwards	<p>INTRODUCTION: Lambert-Eaton syndrome (LEMS) is a primary autoimmune or paraneoplastic disease of the neuromuscular junction caused by the generation of antibodies against voltage-gated calcium channels (VGCC) type P/Q. It leads to proximal muscle weakness and autonomic symptoms. It may be initially confused with polyneuropathies due to the lack of identification of the characteristic electrophysiological facilitation pattern.</p> <p>CASE REPORT: A 50-year-old man presented with symptoms characterized by proximal muscle weakness (climbing, descending stairs, and getting up and down from a chair), fatigue upon exertion, ptosis, and diplopia. The evaluation revealed generalized areflexia and proximal weakness. An initial electrodiagnostic study showed motor axonal polyneuropathy. Twenty months later, a second study revealed three key findings: (1) resting neuroconduction of the median and ulnar nerves showed compound muscle action potential amplitudes of 2.0 mV and 0.7 mV, respectively, with more than a 100% increase following post-exercise facilitation, (2) Repetitive stimulation at a low frequency (3 Hz) produced a decremental response greater than 10% in the tibialis anterior and abductor pollicis brevis, and (3) with the latter showing an increase exceeding 100% at a high frequency stimulus (30 Hz). Serological testing detected anti-VGCC P/Q-type antibodies at levels above 30 pmol/L, confirming the LEMS diagnosis.</p> <p>SUMMARY/CONCLUSION: This case underscores the importance of incorporating repetitive nerve stimulation and post-exercise assessments when neuromuscular weakness is suspected. LEMS can simulate polyneuropathy, and its timely diagnosis allows for better therapeutic guidance, especially when an associated paraneoplastic disease is suspected.</p>
58	BEYOND POLYNEUROPATHY: THE CLASSIC ELECTRODIAGNOSTIC TRIAD AND ANTIBODY DETECTION FOR THE DIAGNOSIS OF PRESYNAPTIC MYASTHENIC SYNDROME (LAMBERT-EATON).	Mauricio Muñoz Mojica, Fernando Ortiz-Corredo	<p>INTRODUCTION: Caregivers provide vital support for people with gMG (PWgMG); however, their lived experiences remain under-explored.</p> <p>OBJECTIVE: To understand the experiences of caregivers supporting PWgMG, including physical, social and mental aspects, and their perceptions on ideal treatment.</p> <p>METHODS: Sixty-minute-structured-qualitative-virtual interviews were conducted with caregivers (≥18 years, same household and/or caring ≥30 hours per week) in Germany, UK, and US. Transcribed data underwent thematic analysis.</p> <p>RESULTS: Twenty-four caregivers (n=8 per country) were interviewed (71% female; 92% primary caregivers). Caregivers reported gMG management had a negative impact on their mental/emotional health (83%; e.g., worry/stress), while half of caregivers reported impacts on physical health (50%; e.g., chronic back pain). Most caregivers (71%) felt their employment (e.g., reducing hours) or relationships/social lives were affected, often feeling isolated. Around half (54%) reported impacts on their financial situation (e.g., less income as a sole provider). Informal external sources of support (e.g., friendships) were often used to improve mental/emotional wellbeing. Caregivers reported they would benefit from forms of support/guidance including practical, emotional, household, and respite care. Most caregivers (83%) had some level of involvement in treatments/medical appointments (e.g., advocating for PWgMG's needs). Caregivers described an ideal treatment as offering full symptom control (e.g., no fluctuations) in the least burdensome way (allowing PWgMG to become more independent and participating in activities of daily living).</p> <p>SUMMARY/CONCLUSION: The consequences of gMG affect both PWgMG and caregivers' lives. Caregivers provide support across all aspects of the emotional, mental and physical lives of PWgMG. Improving treatment options for gMG is likely to benefit PWgMG and their caregivers.</p>
59	THE ALL-ENCOMPASSING ROLE OF CARING FOR PEOPLE WITH GENERALIZED MYASTHENIA GRAVIS (GMG): A QUALITATIVE STUDY OF CAREGIVER EXPERIENCES	Pushpa Narayanaswami, Sarah Bailey, Sophie Lehenrer, Claudia Schlemminger, Kathryn Wiltz, Sophi Tatlock, Jana Raab	

60	UNDERSTANDING THE LIVED EXPERIENCES OF PEOPLE WITH GENERALIZED MYASTHENIA GRAVIS AND THE IMPACT ON DAILY LIFE: A MIXED METHODS STUDY	Pushpa Narayanaswami, Sophie Lehnerer, Kathryn Wiltz, Janet Smith, Claudia Schlemminger, Sophie Tadlock, Jana Raab	<p>INTRODUCTION: Generalized MG (gMG) is a rare autoimmune neuromuscular disorder that can profoundly impact daily life.</p> <p>OBJECTIVE: To understand lived experiences of people with gMG (PWgMG), including diagnostic challenges and persistent needs despite treatment.</p> <p>METHODS: Qualitative interviews with PWgMG and specialist healthcare professionals (HCPs) informed a quantitative survey of PWgMG (themes: pre-diagnosis, diagnosis, treatment, living with gMG) in Germany, Italy, Spain, UK, and US. Interview data underwent thematic analysis. Statistics are descriptive.</p> <p>RESULTS: Fourteen PWgMG (71% female) and 10 HCPs were interviewed. PWgMG were surveyed in pilot (n=5) and quantitative phases (n=110; 64% female). The most common pre-diagnosis symptoms among surveyed PWgMG (n=110) were fatigue (67%), ptosis (59%), and generalized muscle weakness (58%). Pre-diagnosis, most PWgMG (89%) reported symptoms affecting daily life. PWgMG typically visited 2–3 HCPs pre-diagnosis and 25–65% were initially misdiagnosed, including with mental health (44%) and neurological (29%) conditions. PWgMG approached patient organizations more commonly in UK (68%) and Spain (53%) than Italy (26%), Germany (11%), or US (10%). PWgMG expressed needs for resources to facilitate shared decision-making with HCPs and increase gMG awareness. The most common concerns at treatment initiation were side effects/long-term safety (46%). Despite treatment, PWgMG reported ongoing fatigue (69%) and burdens on social interactions (49%), hobbies (49%), and work/study (47%). Interviewed HCPs identified needs for improved gMG awareness and reported challenges related to insurance, comorbidities, speed-of-action, side effects, and family planning.</p> <p>SUMMARY/CONCLUSION: There remains an ongoing need to support communication and shared decision-making between PWgMG and HCPs to reduce the impact of gMG on daily life.</p>
61	MYASTHENIA GRAVIS INEBILIZUMAB TRIAL (MINT): EFFICACY, PHARMACODYNAMICS, AND IMMUNOGENICITY IN AChR+ COHORT (WEEK 52)	Richard J. Nowak, Kimiaki Utsugisawa, Michael Benatar, Emma Cufaloni, M. Isabel Leite, John Vissing, Fengming Tang, Cody J. Peet, Kristen A. Clarkson, Sue Cheng, James F. Howard Jr.	<p>INTRODUCTION: Autoreactive B-cells are central to upstream immunopathogenesis of generalized Myasthenia Gravis (gMG) through the production of autoantibodies. MINT met its primary efficacy endpoint, demonstrating a significant improvement in the Myasthenia Gravis Activities of Daily Living (MG-ADL) score at Week-26.</p> <p>OBJECTIVE: To evaluate the efficacy, pharmacodynamics, and immunogenicity of inebilizumab, a monoclonal antibody targeting CD19+ B-cells, in acetylcholine receptor antibody-positive (AChR+) gMG.</p> <p>METHODS: MINT (NCT04524273), a phase 3 clinical trial in adults with gMG, included a protocol-specified steroid taper. The randomized control period (RCP) was 52-Weeks for the AChR+ cohort and included additional secondary/exploratory endpoints: change from baseline (CFB) in MG-ADL and Quantitative Myasthenia Gravis (QMG) scores at Week-52, CD20+ B-cell count, and anti-drug antibodies (ADA). AChR+ participants were randomized (1:1) to receive 300mg of intravenous inebilizumab/placebo on RCP Day-1, Day-15, and Day-183.</p> <p>RESULTS: Of 238 randomized participants, 190 were AChR+ (inebilizumab: 95/placebo: 95). MG-ADL score showed improvement with inebilizumab vs. placebo at Week-52 (adjusted difference, -2.8; 95% CI, -3.9 to -1.7; nominal p<0.001). Similarly, CFB in QMG score was greater in the inebilizumab group vs. placebo at Week-52 (adjusted difference, -4.3; 95% CI, -5.9 to -2.8; nominal p<0.001). CD20+ B-cell counts fell by 93.3% from baseline two weeks after the initial dose and remained low throughout treatment period in the AChR+ subpopulation. ADA prevalence for inebilizumab vs. placebo-treated participants was 4.2% vs. 2.1% in AChR+ subpopulation.</p> <p>SUMMARY/CONCLUSION: Inebilizumab leads to targeted depletion of B-cells and provides durable improvement in the AChR+ gMG subpopulation through Week-52.</p>
62	DISEASE BURDEN, IMPACT ON DAILY FUNCTIONING, AND PSYCHOLOGICAL WELL-BEING IN PATIENTS WITH OCULAR MYASTHENIA GRAVIS: INSIGHTS FROM A U.S. PATIENT PANEL	Martina Orlovic, Rosa Jimenez, Jeffrey Guptill, Yen Wong, Janet Bernard, Pritikanta Paul, Carolina Barnett-Tapia	<p>INTRODUCTION: Ocular myasthenia gravis (oMG) is a rare autoimmune disorder characterized by fluctuating weakness of the extraocular muscles.</p> <p>OBJECTIVE: To examine disease burden, impact on daily functioning and psychological well-being of oMG patients.</p> <p>METHODS: 23 U.S. oMG patients, identified via patient groups, participated in 60-minute qualitative telephone interviews conducted with secure screen-sharing (Feb-Mar 2025). Participants provided a healthcare provider clinical note confirming their diagnosis prior to the interview.</p> <p>Results: Among patients aged 29–80 years, 83% experienced both ptosis and diplopia. Additional symptoms included eye fatigue, blurred vision, and migraines. Persistent symptoms were reported by 65%, and over half experienced ongoing pain, fatigue, and sleep disturbances. oMG significantly impacted patients' ability to work, leading to reduced productivity, financial strain, and employment changes. A total of 91% (n=21/23) reported disruptions in daily activities such as driving and screen use. Among working-age participants (n=16/23), 94% reported impacts on work—particularly in roles requiring prolonged computer use or visual focus. Emotional effects were nearly universal, including frustration, anxiety, and fear of progression to generalized MG. Sixty-five% reported reduced self-confidence and social withdrawal, and 58% experienced stigma or misunderstanding. oMG had a moderate to significant impact on mental health for the majority of patients. Forty-one% described strain in family relationships and only 9% had formal caregiving support.</p> <p>SUMMARY/CONCLUSION: oMG imposes a substantial burden on patients, affecting physical function, emotional well-being, daily life and ability to work. Persistent symptoms and disturbance of daily activities highlight the need for improved care pathways and expanded treatment options.</p>
63	DIAGNOSIS JOURNEY, TREATMENT, AND MANAGEMENT OF PATIENTS WITH OCULAR MYASTHENIA GRAVIS: INSIGHTS FROM A U.S. PATIENT PANEL	Martina Orlovic, Rosa Jimenez, Jeffrey Guptill, Yen Wong, Janet Bernard, Pritikanta Paul, Carolina Barnett-Tapia	<p>INTRODUCTION: Ocular myasthenia gravis (oMG) is an autoimmune disease characterized by weakness of the extraocular muscles.</p> <p>OBJECTIVE: To explore diagnosis, treatment and management of oMG patients.</p> <p>METHODS: Twenty-three U.S. oMG patients, identified via patient groups, participated in 60-minute qualitative telephone interviews conducted with secure screen-sharing (Feb-Mar 2025). Participants provided a healthcare provider clinical note confirming their diagnosis prior to the interview.</p> <p>RESULTS: While 57% of patients sought care within one week of symptom onset, time to diagnosis ranged from 1 day to 12 months (mean: 2.4 months). Neurologists most consistently identified oMG, whereas delays were often linked to initial evaluation by ophthalmologists. Patients reported unmet needs in faster access to specialists, shorter diagnostic wait times, and greater provider awareness—26% noted insufficient oMG knowledge among non-neurologists. All patients currently used pyridostigmine; 57% used it with corticosteroids (CS). Overall, 82% (19/23) had received CS since diagnosis, with 2 declining due to safety concerns. Among those treated, 37% were on ≥20 mg/day for extended periods, and 47% (9/19) reported side effects. One patient underwent surgery for ptosis bilaterally. While many reported treatment satisfaction, only 2 achieved full symptom resolution, with symptoms recurring if treatment was missed. Adjuvant strategies included eye patches and prism glasses (70%) and lifestyle changes. Patients reported interest in alternative treatments with fewer side effects (43%) and longer lasting effect (35%).</p> <p>CONCLUSION: Diagnosis of oMG remains delayed due to fragmented care pathways and limited provider familiarity. Patients underscore the need for accelerated diagnosis, more efficacious treatment options with good tolerability, and enhanced oMG awareness.</p>

64	FATIGUE ASSESSED BY NEURO-QUALITY OF LIFE IN PHASE 3 VIVACITY-MG3 TRIAL OF NIPOCALIMAB VERSUS PLACEBO IN GENERALIZED MYASTHENIA GRAVIS	John Vissing, Kavita Gandhi, Sheryl Pease, Nida Imran, Maria Ait-Tihyaty, Ibrahim Turkoz, Geoffroy Coteur, Charlotte Gary, Zia Choudhry, Sindhu Ramchandran	<p>INTRODUCTION: Generalized myasthenia gravis (gMG) is an autoantibody-mediated disease, with muscle weakness, fatigue and associated impacts. Fatigue often correlates with gMG disease severity emphasizing the need to manage both effectively. In Vivacity-MG3 (NCT04951622), nipoalimab+standard-of-care (SOC) demonstrated improved and sustained efficacy vs placebo+SOC.</p> <p>OBJECTIVE: To evaluate changes in Neuro-QoL-Fatigue, a patient-reported assessment of fatigue and its associated impact, and disease-severity measures between Vivacity-MG3 arms.</p> <p>METHODS: Mean changes-from-baseline (CFB) in Neuro-QoL-Fatigue total score over 24 weeks(W) were compared using mixed-model-repeated-measures. Proportion-of-patients achieving meaningful-within-person-improvement (MWPI) of 6.7-points from baseline at 24W were examined using Chi-square test. Logistic regression models evaluated likelihood of sustaining MWPI for ≥8, 12, 16, or ≥20W. Mean CFB at 24W was evaluated by stratifying-patients based on baseline disease-severity scores observed above median on Myasthenia-Gravis-Activities-of-Daily-Living (MG-ADL) and Quantitative-Myasthenia-Gravis (QMG) scales (severe disease defined as MG-ADL <9 and QMG >15).</p> <p>RESULTS: LS-mean (95% CI) difference in CFB on Neuro-QoL-Fatigue was greater (p=0.001) with nipoalimab+SOC (-7.4 [-11.94, -2.93]) by 4W and numerically higher at 24W (-4.3 [-9.16, 0.62]) vs placebo+SOC. At 24W, 6.2% more patients on nipoalimab+SOC (42/67) achieved MWPI than placebo+SOC (35/62) (p=0.05). Nipoalimab+SOC-treated patients were approximately twice more likely to sustain MWPI for ≥8, 12, 16, 20W (p=0.05). Among those with severe disease at baseline, mean improvement was numerically greater at 24W with nipoalimab+SOC vs placebo+SOC (difference=-9.0; 95% CI=-22.0, 4.1).</p> <p>SUMMARY/CONCLUSION: Nipoalimab+SOC-treated patients showed improvement on Neuro-QoL-Fatigue as early as W4. Nipoalimab+SOC-treated patients were also significantly more likely to sustain MWPI over time. Patients with more severe disease at baseline showed numerically greater improvements with nipoalimab+SOC than placebo+SOC.</p>
65	ASSESSMENT OF PATIENT-REPORTED OUTCOMES FROM THE PHASE 3 VIVACITY-MG3 STUDY OF NIPOCALIMAB IN GENERALIZED MYASTHENIA GRAVIS	Elena Cortés Vicente, Sheryl Pease, Nida Imran, Kavita Gandhi, Maria Ait-Tihyaty, Ibrahim Turkoz, Geoffroy Coteur, Charlotte Gary, Zia Choudhry, Sindhu Ramchandran, John Vissing	<p>INTRODUCTION: Due to the heterogeneity of generalized myasthenia gravis (gMG), it is crucial to capture health-related quality of life data, including treatment satisfaction for this rare condition. Examining patient-reported outcomes (PROs) helps to better understand the overall impact of the disease and the effectiveness of treatments from the patients' perspectives. Nipoalimab+SOC demonstrated positive efficacy in Vivacity-MG3 (NCT04951622) vs placebo+SOC in gMG.</p> <p>OBJECTIVE: To evaluate the comprehensive PRO measures from the Vivacity-MG3 trial, which offers valuable insights into treatment satisfaction and overall disease status from the viewpoint of patients treated with nipoalimab+SOC vs placebo+SOC.</p> <p>METHODS: The efficacy analysis population included participants who were antibody-positive for a gMG-related pathogenic antibody (anti-acetylcholine receptor [AChR], anti-muscle-specific tyrosine kinase [MuSK], or anti-low density lipoprotein receptor-related protein 4 [LRP4]). PROs were reported from week-2 (W2) through week-24 (W24) descriptively and included: EuroQol-5-Dimension Visual Analogue Scale (EQ-5D VAS), Patient Global Impression of Severity/Change-Fatigue (PGIS/PGIC), and Treatment Satisfaction Questionnaire for Medication (TSQM-9).</p> <p>RESULTS: EQ-5D-5L VAS mean (95% confidence interval [CI]) change-from-baseline scores were significantly improved for nipoalimab+SOC (11.1[7.1, 15.2]) vs placebo+SOC (1.3[-2.2, 4.8]) by W2; improvement was sustained up to W24. At W24, 56.5% of nipoalimab+SOC-treated patients reported their fatigue as "much better" or "moderately better" since the start of study medication, a difference of 15.5% vs placebo. Mean scores (95% CI) in TSQM-9 Global Satisfaction domain at W24 were numerically higher in nipoalimab (65.7[59.4, 72.0]) vs placebo (56.1 [50.1, 62.1]).</p> <p>SUMMARY/CONCLUSION: Nipoalimab-treated patients reported numerically greater improvements on patient-reported health status and treatment satisfaction compared with placebo-treated patients.</p>
66	EDUCATIONAL NEEDS ASSESSMENT OF NURSES INVOLVED IN THE CARE OF PATIENTS WITH MYASTHENIA GRAVIS	Amy Perrin Ross, Georgina Burke, Caroline Carmichael, Emiko Chiba, Jerrica Farias, Jude Kings, Naira Vidal-Fernández, Tuan Vu, Natasha Monin, Thais Tarancon, Chioma Ezenduka	<p>INTRODUCTION: Nurses with specialized knowledge have an integral and diverse role in providing coordinated patient care and support. Myasthenia gravis (MG) is a heterogeneous disease where specialized nurses are essential for effective patient management.</p> <p>OBJECTIVE: To elucidate the educational needs of nurses caring for adults with MG.</p> <p>METHODS: We conducted 1-hour semi-structured interviews with nurses who have specialized knowledge of MG and a literature search (July 8, 2024) to answer, "What are the key learning and practice needs of nurses involved in the treatment and management of MG?" Interviews were analyzed using principles of thematic analysis. The literature search used four databases to identify articles published since 2018; search terms included "myasthenia gravis" and "nurse." Retrieved articles were analyzed to identify system, practice, knowledge, and skills gaps and (where possible) mapped to the interview themes.</p> <p>RESULTS: Interviews with nurses (n=6) generated five overarching themes: (1) individual professional development, (2) multifaceted role, (3) self-perceived and (4) multidisciplinary team challenges and learning needs, (5) perceived learning solutions and preferences. Nurses fulfilled roles as coordinators, educators, and patient advocates but reported an absence of educational provisions throughout their career. The literature search yielded 24 articles, most (n=17/24) mapped to the interview themes. Themes (1) and (3) were uniquely reflected in the interviews, demonstrating the importance of firsthand insights.</p> <p>SUMMARY/CONCLUSION: Nurses have an important, multifaceted role with unique perspectives. The educational gap identified for nurses caring for patients with MG and those wishing to specialize highlights the need for the development of tailored, nurse-specific learning resources. Funding: UCB</p>
67	TREATMENT-RELATED CHARACTERISTICS AMONG YOUNGER WOMEN WITH GENERALIZED MYASTHENIA GRAVIS	Jacqueline Pesa, Louis Jackson, Alex Keenan, Nolan Campbell, Gregor Gibson, Joe Conyers, Neelam Goyal	<p>INTRODUCTION: In women, the diagnosis of generalized myasthenia gravis (gMG) is often made during childbearing years. There is limited research in women during this critical life stage that presents differentiating considerations for gMG management.</p> <p>OBJECTIVE: Describe current and historical treatment patterns among younger women with gMG.</p> <p>METHODS: Data were drawn from the Adelphi gMG Disease Specific Programme, an extensive cross-sectional dataset of US-based MG-treating neurologists and their consulting MG patients (January–August 2024). Descriptive data are presented for women ages 18–45 (younger women) along with comparison groups (younger men: 18–45 years; older women: ≥46 years; older men: ≥46 years).</p> <p>RESULTS: Data were collected from 40 neurologists with respect to 266 gMG patients: 55 younger women, 24 younger men, 73 older women, and 114 older men. Among younger women, 1 was pregnant and 4 had plans to become pregnant within 12 months. Physicians cited 18% of younger women refused treatment as the reason for lack of prescription of MG medication (vs 3% older women, 8% younger men, 6% older men). A total of 27% of younger women received Methotrexate and 18% Mycophenolate mofetil (contraindicated in pregnancy). Additionally, 42% of younger women were not in remission at the time of survey (vs 25% older women, 33% younger men, 32% older men).</p> <p>SUMMARY/CONCLUSION: gMG may impact younger women differently from other groups. This analysis found a high percentage not receiving MG treatment and use of medications contraindicated in pregnancy. There is a need for education on treatment options and benefit/risk of maintenance therapies in this population.</p>

			<p>INTRODUCTION: Cardiovascular (CV) risk factors and comorbidities are common in patients with generalized myasthenia gravis (gMG). This may impact gMG treatment choice decisions, since lipid increases have been observed with some other FeRn inhibitors.</p> <p>OBJECTIVE: To assess frequency of CV risk factors and CV-related adverse events (AEs) in participants with gMG receiving intravenous efgartigimod or subcutaneous efgartigimod PH20 (coformulated with recombinant human hyaluronidase PH20).</p> <p>METHODS: Post hoc analyses using pooled data from ADAPT, ADAPT-SC, and ADAPT NXT trials assessed concomitant CV diseases or risk factors. CV-related AEs and low-density lipoprotein (LDL) levels were evaluated using data from the placebo-controlled ADAPT study.</p> <p>RESULTS: The pooled analysis included 263 participants. Mean age was 51.7 years, 83.3% (n=219) had CV risk factors, and 5.3% (n=14) had a CV comorbidity. Corticosteroid use was similar in participants with and without CV burden (65.8% [n=144] vs 65.9% [n=29]). Participants with CV burden were older (mean, 55.2 vs 34.2 years) and more likely to be male (39.7% [n=87] vs 11.4% [n=5]). In ADAPT, there were no notable differences in CV-related AEs between efgartigimod and placebo. Increases in LDL levels were not observed with efgartigimod treatment, including patients with elevated LDL at baseline (>130 mg/dL). Apolipoprotein B (ApoB), a more accurate marker of CV risk, did not increase with efgartigimod treatment in assessed participants.</p> <p>SUMMARY/CONCLUSION: CV burden was high in participants with gMG. Careful assessment of CV risk factors and comorbidities, such as hyperlipidemia, is important when making treatment decisions. Efgartigimod treatment did not impact frequency of CV-related AEs or increase LDL or ApoB levels.</p>
68	<p>EVALUATION OF CARDIOVASCULAR COMORBIDITY BURDEN IN PATIENTS WITH GENERALIZED MYASTHENIA GRAVIS TREATED WITH EFGARTIGIMOD</p>	<p>Jana Podhorna, Sihui Zhao, Kristin Heerlein, Sophie Steeland, Jeffrey Guptill, Christiane Schneider-Gold</p>	<p>INTRODUCTION: FeRn antagonists have shown that deeper reductions of immunoglobulin G (IgG) are associated with improved outcomes in patients with myasthenia gravis. FeRn antagonists are typically administered weekly as large-volume subcutaneous or intravenous infusions to achieve IgG reductions of 60-80% after weeks of treatment. An alternative for rapid (within minutes) and deep (>99%) IgG depletion is the bacterial IgG protease IdeS. However, wild-type (WT) IdeS suffers from short pharmacokinetics and high immunogenicity. We engineered an IdeS variant, CYR212, to remove immunogenic epitopes and that is fused to serum albumin for half-life extension.</p> <p>OBJECTIVE: To evaluate CYR212 pharmacokinetics, pharmacodynamics, and immunogenicity.</p> <p>METHODS: WT IdeS and CYR212 were administered intravenously or subcutaneously to rabbits from 0.00002 to 1 mg/kg, with up to 3 doses at biweekly intervals. Serum was analyzed by ELISA to quantify IgG, IdeS/CYR212 exposures, and anti-drug antibodies. CYR212 was also administered to hFcRn/Ab-KO mice and data facilitated simulations of human pharmacokinetics.</p> <p>RESULTS: CYR212 provided IgG reductions from 55% at 0.00002 mg/kg to >99.9% at 0.1 mg/kg for weeks from a single dose. While repeat doses of WT IdeS were neutralized by high titers of anti-drug antibodies, CYR212 had undetectable or low immunogenicity and could be dosed at least 3 times with excellent potency. CYR212 bioavailability and efficacy via subcutaneous route were high, and modeling from mouse and rabbit data predicts a human half-life of 9.5 days.</p> <p>SUMMARY/CONCLUSION: CYR212 provides deep and durable IgG depletion at exceptionally low doses, enabling low-volume subcutaneous administration with long dosing intervals. CYR212 has properties for potential best-in-class status.</p>
69	<p>PRECLINICAL EVALUATION OF AN AUTOANTIBODY PROTEASE FOR THE TREATMENT OF MYASTHENIA GRAVIS AND RELATED AUTOIMMUNE DISEASES</p>	<p>Maximilian Sauer, Benjamin Dutzar, Emily Frazier, Kui Chan, Daniel Farrell, David Thieker, Paul DaRosa, Yifan Song, Eric Tarcha, Erik Procko</p>	<p>INTRODUCTION: While clinical trials demonstrated comparable safety and efficacy with intravenous (IV) and subcutaneous (SC) efgartigimod among adults with anti-acetylcholinesterase antibody-positive generalized myasthenia gravis (gMG), data from clinical practice is limited.</p> <p>OBJECTIVE: To evaluate patient characteristics, dosing patterns, and outcomes among patients with gMG using IV or SC efgartigimod.</p> <p>METHODS: Adults with gMG enrolled in the USA My VYVGART Path patient support program by December 2024 who were treated with IV or SC efgartigimod were included. Patient characteristics and time between treatment cycles were analyzed. To assess outcomes, Myasthenia Gravis Activities of Daily Living (MG-ADL) before efgartigimod initiation was compared with best (lowest) MG-ADL captured after initiation at the patient-level.</p> <p>RESULTS: Among 2878 patients who initiated efgartigimod after SC approval (July 2023), 69% (n=1993) used only IV and 22% (n=622) used only SC (9% used both and were excluded). Mean (SD) age and baseline MG-ADL was 65.8 (15.4) years and 8.1 (3.7) for IV, and 67.0 (15.3) years and 7.6 (3.4) for SC. Average (SD) efgartigimod cycles initiated was 2.9 (2.0) for IV and 2.3 (1.6) for SC. Four weeks was the most common gap between cycles for both formulations. For patients with MG-ADL available (IV: 862 [43%]; SC: 214 [34%]), mean (SD) best improvement from baseline was 5.4 (3.3) for IV and 4.8 (3.0) for SC.</p> <p>SUMMARY/CONCLUSION: Among patients with gMG, rapid and clinically meaningful outcomes were observed regardless of efgartigimod formulation, underscoring the value of formulation flexibility and administration options that can be tailored to patient preference.</p>
70	<p>PATIENT CHARACTERISTICS, DOSING PATTERNS, AND OUTCOMES ASSOCIATED WITH INTRAVENOUS AND SUBCUTANEOUS EFGARTIGIMOD AMONG PATIENTS WITH GENERALIZED MYASTHENIA GRAVIS IN CLINICAL PRACTICE</p>	<p>Cynthia Qi, Ratna Bhavaraju-Sanka, A. Gordon Smith, Rohit R. Menon, Helen Zhang, Mai Sato, Gil Wolfe</p>	<p>INTRODUCTION: Comorbidities have been associated with mortality-risk in patients with myasthenia gravis (MG); however, their contribution and other causes of mortality in MG patients have not been well-characterized.</p> <p>OBJECTIVE: To evaluate drivers of mortality among patients with MG in the United States.</p> <p>METHODS: This retrospective cohort study used data from the National Veterans Affairs Health Care Network and Medicare databases (1999–2021). Patients enrolled in both databases who had ≥2 MG-related diagnostic codes and a consistent Mortality Data Repository (MDR) record were included. Index was first recorded MG diagnosis, with follow-up to end-of-study, death, or disenrollment. Primary contributing causes of mortality were identified from MDR records. Multivariate Cox analysis evaluated factors associated with MG mortality, with non-MG death as a competing risk.</p> <p>RESULTS: 9,212 patients with MG were included (mean age 72.7 years, 97.2% male); common comorbidities included obesity (93.5%) and hypertension (82.4%). During follow-up (mean 7.7 years), 5,026 (54.6%) patients died; 695 (13.8%) had MG as primary cause of death, and 4,331 (86.2%) had non-MG primary causes. For patients with MG death, most common contributing non-MG causes were respiratory diseases (21.1%) and circulatory system diseases (16.2%). The most frequent non-MG primary causes were circulatory system diseases (28.0%) and neoplasms (16.6%). In multivariate analysis, factors significantly associated with MG death (hazard ratio [HR]: 2) included MG crises (HR: 5.64, p<0.001) and steroid use (HR: 2.03 moderate-/3.23 high-dose; p<0.001).</p> <p>SUMMARY/CONCLUSION: Multiple conditions contribute to mortality in patients with MG. Clinicians should consider comorbidities and other drivers of overall mortality risk to develop individualized treatment strategies for MG patients.</p>
71	<p>DRIVERS OF MORTALITY IN PATIENTS WITH MYASTHENIA GRAVIS IN THE UNITED STATES NATIONAL VETERANS AFFAIRS HEALTH CARE NETWORK AND MEDICARE DATABASES</p>	<p>Cynthia Qi, Yuli Lin, Yuebing Li, Tuan Vu, Deborah Gelinas, Alexis Lizarraga, Cecile Blein, Femke De Ruysck, Litzheng Shi</p>	<p>INTRODUCTION: Several novel biologics have recently been approved or are under review in the United States for generalized myasthenia gravis (gMG), yet their relative benefits compared to existing ones remain incompletely assessed.</p> <p>OBJECTIVE: To evaluate and compare the efficacy of treatments for anti-acetylcholine receptor antibody-positive (anti-AChR Ab+) gMG.</p> <p>METHODS: A network meta-analysis (NMA) was conducted using data from placebo-controlled trials of efgartigimod IV (ADAPT), rozanolixizumab (MycarinG), ravlizumab (CHAMPION-MG), zilucoplan (RAISE), inebilizumab (MINT), and nopicalmab (VYACTY-MG) in patients with anti-AChR Ab+ gMG. Efficacy outcomes included change from baseline, and ≥3- and ≥5-point reductions in Myasthenia Gravis-Activities of Daily Living (MG-ADL) and Quantitative Myasthenia Gravis (QMG) scores. NMA results were used to estimate the number needed to treat (NNT) vs. placebo and relative to the therapy with the lowest NNT.</p> <p>RESULTS: The NMA results suggest efgartigimod IV was associated with the greatest improvement in changes from baseline in MG-ADL (not significantly better than other treatments) and QMG (significantly better than inebilizumab, nopicalmab, ravlizumab, and zilucoplan). Efgartigimod IV achieved the lowest NNTs for ≥5-point MG-ADL reduction (significantly lower than ravlizumab and zilucoplan), ≥3-point QMG reduction (significant vs. all except rozanolixizumab), and ≥5-point QMG reduction (significant vs. zilucoplan). Rozanolixizumab had the lowest NNT for ≥3-point MG-ADL reduction, though differences vs. others were not statistically significant.</p> <p>SUMMARY/CONCLUSION: Within the limitations of this meta-analysis, efgartigimod was associated with the most favorable comparable efficacy across multiple MG-ADL and QMG endpoints in AChR Ab+ gMG.</p>
72	<p>COMPARATIVE BENEFITS OF IMMUNOMODULATORY THERAPIES FOR GENERALIZED MYASTHENIA GRAVIS</p>	<p>A. Gordon Smith, Cynthia Qi, Ali Habib, Hongbo Yang, Mandy Du, Jingyi Liu, Deborah Gelinas, Eddie Brauer, Kristin Heerlein, Glenn Phillips, Gil Wolfe</p>	<p>INTRODUCTION: Several novel biologics have recently been approved or are under review in the United States for generalized myasthenia gravis (gMG), yet their relative benefits compared to existing ones remain incompletely assessed.</p> <p>OBJECTIVE: To evaluate and compare the efficacy of treatments for anti-acetylcholine receptor antibody-positive (anti-AChR Ab+) gMG.</p> <p>METHODS: A network meta-analysis (NMA) was conducted using data from placebo-controlled trials of efgartigimod IV (ADAPT), rozanolixizumab (MycarinG), ravlizumab (CHAMPION-MG), zilucoplan (RAISE), inebilizumab (MINT), and nopicalmab (VYACTY-MG) in patients with anti-AChR Ab+ gMG. Efficacy outcomes included change from baseline, and ≥3- and ≥5-point reductions in Myasthenia Gravis-Activities of Daily Living (MG-ADL) and Quantitative Myasthenia Gravis (QMG) scores. NMA results were used to estimate the number needed to treat (NNT) vs. placebo and relative to the therapy with the lowest NNT.</p> <p>RESULTS: The NMA results suggest efgartigimod IV was associated with the greatest improvement in changes from baseline in MG-ADL (not significantly better than other treatments) and QMG (significantly better than inebilizumab, nopicalmab, ravlizumab, and zilucoplan). Efgartigimod IV achieved the lowest NNTs for ≥5-point MG-ADL reduction (significantly lower than ravlizumab and zilucoplan), ≥3-point QMG reduction (significant vs. all except rozanolixizumab), and ≥5-point QMG reduction (significant vs. zilucoplan). Rozanolixizumab had the lowest NNT for ≥3-point MG-ADL reduction, though differences vs. others were not statistically significant.</p> <p>SUMMARY/CONCLUSION: Within the limitations of this meta-analysis, efgartigimod was associated with the most favorable comparable efficacy across multiple MG-ADL and QMG endpoints in AChR Ab+ gMG.</p>

	CORTICOSTEROID TOXICITY AND RELATED HEALTHCARE RESOURCE UTILIZATION IN PATIENTS WITH MYASTHENIA GRAVIS IN THE USA	Thomas Ragole, Michael Blackowicz, Emma Weiskopf, Ashwin Anand, Mike Sicilia, Gil Wolfe	<p>INTRODUCTION: Myasthenia gravis (MG) is an autoimmune neuromuscular disorder characterized by fatigable muscle weakness. Corticosteroids are frequently prescribed to patients with MG, despite being linked to significant adverse effects, especially with long-term use.</p> <p>OBJECTIVE: This study assessed the association of corticosteroid-induced toxicity with healthcare resource utilization in a real-world cohort of adults with MG.</p> <p>METHODS: This retrospective cohort study examined patients with MG who were treated with corticosteroids, utilizing data linkage from a multi-payer claims database and electronic health records (CHRONOS, Fortian Inc.) from January 1, 2016, to September 30, 2024. Eligible participants were corticosteroid-naïve at baseline and had at least one post-baseline measure of cumulative worsening score (CWS) on the Glucocorticoid Toxicity Index – Metabolic Domains (GTI-MD, Massachusetts General Hospital, Sterias) at least six months after the initial observed MG claim. Mixed effects regression models with repeated measures were employed to evaluate the dose-response relationship between cumulative corticosteroid toxicity and healthcare resource utilization after 2 years of corticosteroid use.</p> <p>RESULTS: There were 300 patients with and 441 patients without corticosteroid use that met the eligibility criteria of a valid GTI-MD score. Compared to patients with low corticosteroid toxicity (CWS<9), patients with the highest toxicity (CWS≥75) had 3.1 times more non-gMG-related hospitalizations (95% CI: 1.5-6.1), 2.1 times more ER visits (95% CI: 1.6-2.7), 2.9 times more outpatient hospital visits (95% CI: 2.6-3.3), and 4.0 times more skilled nursing facility claims (95% CI: 3.4-4.7).</p> <p>SUMMARY/CONCLUSION: This large retrospective analysis demonstrates a marked elevation in healthcare resource utilization in patients with MG with high corticosteroid toxicity.</p>
74	ASSESSING EFFICACY AND SAFETY OF GEFURULIMAB IN GENERALISED MYASTHENIA GRAVIS: BASELINE CHARACTERISTICS FROM PREVAIL	Francesco Sacca, Kelly Gwathmey, Masayuki Masuda, Ali Habib, Stojan Peric, Sanjay Rakhade, Joachim Scholz, Shailan Shang, James F. Howard Jr.	<p>INTRODUCTION: Complement component 5 (C5) inhibitors are effective treatments for anti-acetylcholine receptor antibody-positive (AChR-Ab+) generalised myasthenia gravis (gMG). Gefurulumab (ALXN1720), designed for weekly subcutaneous (SC) self-injection, is a novel, dual-binding nanobody that blocks C5.</p> <p>OBJECTIVE: The phase 3, multicentre, randomised, double-blind, placebo-controlled PREVAIL study is evaluating the efficacy and safety of gefurulumab in adults with AChR-Ab+ gMG (NCT, NCT05556096; EudraCT, 2023-508284-77-00). Here, we describe summary baseline characteristics of participants in the PREVAIL study.</p> <p>METHODS: Adult patients with AChR-Ab+ gMG were randomised 1:1 to weekly SC self-injection of gefurulumab or placebo. The study consists of an initial screening period (up to 4 weeks), a randomised controlled treatment period (26 weeks), and an open-label extension (up to 105 weeks). Patients may continue previously prescribed allowed therapies, including immunoglobulins. The primary endpoint is change from baseline in Myasthenia Gravis Activities of Daily Living (MG-ADL) total score at week 26. Secondary endpoints include change from baseline in Quantitative Myasthenia Gravis (QMg) total score and Myasthenia Gravis Composite (MGC) total score. Safety, pharmacokinetics, pharmacodynamics, immunogenicity, and quality of life are also assessed.</p> <p>RESULTS: As of 09Dec2024, 260 participants have been enrolled. At baseline (n=259), ~60% of participants were female and mean±SD MG-ADL total score was 9.0±2.2. At first dose of study intervention (n=259), mean±SD age was 52.8±15.8 yrs, and ~83% of patients were using any immunosuppressive therapy.</p> <p>SUMMARY/CONCLUSION: This study examines the potential of gefurulumab as an effective treatment for patients with AChR-Ab+ gMG self-administered once-weekly as a SC injection. Additional baseline characteristics will be presented.</p>
75	EFGARTIGIMOD MONOTHERAPY IN AChR AND MUSK NEGATIVE MG, WITH TITIN ANTIBODY POSITIVITY: A CASE REPORT	Rodrigo Rodriguez	<p>INTRODUCTION: Testing of striational antibodies, such as Titin and RyR, may be informative for diagnosis and treatment of MG. Here we report a case of a MG patient negative for AChR and MuSK, but positive for Titin antibodies, who responds in a clinically meaningful manner to efgartigimod monotherapy.</p> <p>CASE REPORT: 27-year-old female presented with ptosis, fatigue, and minor respiratory dysfunction. Baseline MG-ADL was an 8 (ptosis, intermittent diplopia, difficulty standing from sitting position). She was negative for AChR and MuSK antibodies, but positive for Titin antibodies (tested via immunofluorescence). Repetitive nerve stimulation demonstrated significant decremental response and CT chest showed findings of thymic gland enlargement. Initial treatment was several rounds of IVIg and prednisone. 30 mg daily, which did not offer adequate improvement. Thymectomy was performed in mid-2021 showing thymic hyperplasia but no thymoma. Mycophenolate mofetil 500 mg oral twice daily was initiated and continued for 12 months, but did not improve ptosis or fatigue and was discontinued. Efgartigimod IV was initiated in August 2022 and following 3 cycles of treatment (4 weeks in between cycles) she improved to an MG-ADL of 1 (only mild intermittent ptosis). She was able to return to work as a professional musician after having previously stopped due to her disability and remains on monotherapy with efgartigimod.</p> <p>SUMMARY/CONCLUSION: Efgartigimod may improve symptoms for AChR/MuSK negative MG with Titin positivity. Additional diagnostic modalities may be key for diagnostic confirmation when immunofluorescence gives a negative result. Additionally, striational antibody positivity in AChR/MuSK negative MG may represent immunological cross reactivity with NMJ epitopes.</p>
76	IMPACT OF LONG-TERM INTRAVENOUS EFGARTIGIMOD ON QUALITY OF LIFE, DISEASE SEVERITY, AND SAFETY IN PARTICIPANTS WITH GENERALIZED MYASTHENIA GRAVIS DURING ADAPT NXT	Gregory Sahagian, Ali Habib, Kristi Claeys, Yessir Hussain, Elena Cortés-Vicente, Edward Brauer, Jeffrey Gupta, Li Liu, Rosa H. Jimenez, Delphine Massache, Renato Mantegazza, Andreas Meisel, Arjun Seth, Shahram Attarian	<p>INTRODUCTION: During Part A of ADAPT NXT (Phase 3b, NCT04980495), fixed-cycles or every-other-week (Q2W) dosing of intravenous efgartigimod were well tolerated and efficacious in participants with generalized myasthenia gravis (gMG), with no statistically significant difference in MG-ADL score improvements between dosing arms.</p> <p>OBJECTIVE: To assess long-term impact of efgartigimod on quality of life (QoL), disease severity, and safety.</p> <p>METHODS: Participants with anti-acetylcholine receptor antibody-positive gMG were randomized 3:1 to Q2W or fixed-cycles dosing of 10 mg/kg efgartigimod for 21 weeks in Part A. In Part B, participants (including those who received fixed cycles in Part A) received Q2W dosing during a 105-week extension and could switch to every-third-week (Q3W) dosing, depending on clinical assessment.</p> <p>RESULTS: Sixty-nine participants received ≥1 dose of efgartigimod during Part A; 65 participants continued treatment in Part B. Mean (SE) MG-QoL 15e score (range, 0-30; higher indicates more severe MG-related dysfunction) improved from 16.9 (0.8) at baseline to 12.0 (0.8) at week 4 (n=64). Mean (SE) EQ-5D-5L VAS score (range, 0-100; higher indicates more positive perception of health status) improved from 56.0 (2.3) at baseline to 68.7 (2.2) at week 4. Improvements in both QoL measures were sustained through week 126. MG-ADL improvements occurred as early as week 1, with a mean (SE) -4.4 (0.4) point improvement at week 4, and improvements were sustained through week 126. Efgartigimod was well tolerated across dosing regimens; no new safety signals were observed.</p> <p>SUMMARY/CONCLUSION: Efgartigimod demonstrated sustained QoL and clinical improvements in participants with gMG and was well tolerated across different dosing schedules.</p>
77	EXPLORING ACCURACY AND UTILITY OF ARTIFICIAL INTELLIGENCE IN THE REAL-WORLD MANAGEMENT OF MYASTHENIA GRAVIS	Sara Francesca Santagostino, Matthew Morin, Elle Levit, Michael Hehr	<p>INTRODUCTION: Myasthenia gravis (MG) diagnostic and therapeutic decisions are becoming increasingly complex due to scientific advancements, including six new FDA-approved therapies since 2021 and at least seven novel therapeutic classes under investigation. Timely, evidence-based management is critical to provide optimal care. Given increasing clinical demands and limited specialist access, artificial intelligence (AI) platforms may serve as valuable tools for real-time clinical decision support prior to reaching subspecialist care.</p> <p>This study evaluates whether AI can assist clinicians in navigating MG-related clinical scenarios through structured queries posed to three AI tools.</p> <p>OBJECTIVE: To assess the accuracy, comprehensiveness, consistency, and clinical utility of AI-generated guidance in MG care.</p> <p>METHODS: We examined the performance of three large language models (LLMs), ChatGPT-4o, Gemini 2.5 Flash, and OpenEvidence, in extracting key diagnostic and treatment information on MG from current scientific literature. Five physician-designed query sets were used, each beginning with a general prompt followed by 2–3 prompts with more strict boundaries. Responses were compared across platforms and against expert answers.</p> <p>RESULTS: Despite providing similar answers, there were differences in the sources selected by each AI tool, with Gemini weighing scholarly sources similarly to less rigorous ones. Stricter query constraints did not consistently improve responses. Inaccuracies in cited references were also observed for Chat GPT. A detailed comparative analysis with scoring metrics will be presented.</p> <p>SUMMARY/CONCLUSION: There is an opportunity for AI generated guidance to improve care for MG patients. This study will provide initial comparative analysis among three commonly employed AI tools.</p>

78	OUTCOMES FOR PATIENTS RECEIVING RAVULIZUMAB OR EFGARTIGIMOD TREATMENT WITHIN TWO YEARS OF GENERALIZED MYASTHENIA GRAVIS (gMG) DIAGNOSIS: A RETROSPECTIVE SUBANALYSIS OF US MEDICAL RECORDS	Christopher Scheiner, Samir Macwan, Nicholas Streicher, Karen Yee, Justin Lee, Michael Blackowicz, Emma Weiskopf, Michael Pulley	<p>INTRODUCTION: Ravulizumab (terminal complement inhibitor) and efgartigimod (neonatal Fc receptor blocker) are approved for anti-acetylcholine receptor antibody-positive (AChR-Ab+) gMG. However, real-world data comparing outcomes among patients treated with these therapies are limited.</p> <p>OBJECTIVES: Evaluate outcomes among patients who initiated ravulizumab or efgartigimod as their first targeted immunotherapy within 2 years after gMG diagnosis.</p> <p>METHODS: Physician-abstracted electronic medical records data were included for adults with AChR-Ab+ gMG from Cardinal Health's Neurology Provider Extended Network who initiated their first targeted immunotherapy on/after December 1, 2021, within 2 years after gMG diagnosis. Outcomes included Myasthenia Gravis Activities of Daily Living (MG-ADL), minimal manifestation (MM), and MG exacerbation-associated hospitalizations and were analyzed through 6 months post initiation.</p> <p>RESULTS: Of 45 and 107 patients receiving ravulizumab and efgartigimod, respectively, 18 (40.0%) and 52 (48.6%) initiated treatment within 2 years after diagnosis. MG-ADL scores (mean±SD) for ravulizumab- and efgartigimod-treated patients were 8.9±2.6 and 9.3±4.0 (preinitiation, respectively, and 2.7±2.0 and 4.0±3.0 after 6 months of treatment. MM was achieved in 11/18 (61.1%) and 21/52 (40.4%) patients at first assessment after ravulizumab and efgartigimod initiation, respectively, and 12/17 (70.6%) and 24/50 (48.0%) patients after 6 months of treatment. Total patients hospitalized with MG exacerbations decreased from 4 in the 6 months before initiation to 0 in the 6 months after initiation for ravulizumab-treated patients and from 6 to 1 for efgartigimod-treated patients.</p> <p>SUMMARY/CONCLUSION: Although both treatments improved outcomes in patients initiating treatment within 2 years after gMG diagnosis, these results suggest that ravulizumab-treated patients trended toward greater improvements than those receiving efgartigimod.</p>
79	MEDICALLY REFRACTORY AUTOIMMUNE MYASTHENIA GRAVIS IN A 6-YEAR-OLD GIRL: DRAMATIC RESPONSE TO EFGARTIGIMOD VIA EMERGENCY IND ACCESS	Abigail Schwade, Martha Finch, Bridget McGowan, Tonke van Bragt, Nancy Kuntz	<p>INTRODUCTION: Medically Refractory Autoimmune Myasthenia Gravis in a 6-Year-Old Girl: Dramatic Response to Efgartigimod via Emergency IND</p> <p>Pediatric autoimmune myasthenia gravis (MG) is rare, often presenting with ocular or mild generalized symptoms. Severe bulbar and respiratory involvement is uncommon, and treatment options are limited, with few therapies approved for young children.</p> <p>OBJECTIVE: To describe a case of medically refractory generalized MG in a 6-year-old and her response to efgartigimod accessed via emergency IND.</p> <p>METHODS: A previously healthy 6-year-old girl developed progressive weakness and respiratory failure following a near-drowning event. She was diagnosed with acetylcholine receptor antibody-positive MG. Despite treatment with corticosteroids, IVIG, pyridostigmine, and rituximab, she experienced minimal improvement and required multiple ICU admissions. Plasma exchange (PLEX) offered only transient benefit. Given the severity and refractory nature of her disease, efgartigimod—a neonatal Fc receptor (FcRn) antagonist that reduces pathogenic IgG—was administered under emergency IND. Treatment led to rapid resolution of bulbar, respiratory and generalized weakness. Recurrent symptoms responded to longer term therapy and, over several years of therapy, subject is now in clinical remission. No adverse events occurred.</p> <p>CONCLUSION: This case highlights the complexity of managing refractory pediatric MG and supports the potential role of FcRn inhibition. Efgartigimod was well tolerated and effective in achieving meaningful and sustained clinical improvement. Further research is needed to assess safety and efficacy in the pediatric population.</p>
80	CLINICAL, IMMUNOLOGIC AND THERAPEUTIC CORRELATION BETWEEN MYASTHENIA GRAVIS AND CROHN'S DISEASE: A CASE REPORT AND LITERATURE REVIEW	Keshav Shah, Arada Weerawat, Hiranya Dave	<p>INTRODUCTION: Case report of an adult female with a rare coexistence of autoimmune conditions Crohn's disease and Myasthenia Gravis (anti-MUSK antibody-positive). Myasthenia exacerbations occur with worsening of Crohn's symptoms.</p> <p>OBJECTIVE: Analyze the relationship between immunopathogenesis of both autoimmune conditions. Investigate potential biomarkers that would correlate with either or both conditions. Explore effective therapies in such coexisting diseases.</p> <p>METHODS: Although immunopathogenesis of both diseases are distinct, through illustrations, we demonstrate how a neuromuscular junction disease and its treatment affects Crohn's disease. This review studies gut microbiome testing as an emerging and common biomarker which can be used for diagnostic purposes for Myasthenia whereas used for treatment response purposes in Crohn's. Anticholinergic agents are known to be less effective in anti-MUSK antibody Myasthenia and additionally have gastrointestinal side effects, thus limiting its utility in such coexisting conditions. Corticosteroids are common agents shown to be effective in both conditions, but this review demonstrates effective doses of corticosteroids for both conditions, without severe immunosuppression. Lastly, we analyzed reports demonstrating effects of plasmapheresis and IVIG on Crohn's disease.</p> <p>RESULTS: There is a correlation between the gut microbiome, Myasthenia and Crohn's disease process which needs further statistical analysis. Co-treatment is better individualized based on disease type and severity but should be done with considerable caution of immunosuppression.</p> <p>SUMMARY/CONCLUSION: This case report and literature review highlights the correlation between disease processes and exacerbations of Myasthenia Gravis and Crohn's disease. Gut microbiome can be used as a potential biomarker for both diseases. Coexisting autoimmune conditions like these warrant a cautious approach to immunotherapy.</p>
81	COMBINED USE OF ZILUCLOPLAN AND RITUXIMAB FOR ACETYLCHOLINE RECEPTOR ANTIBODY POSITIVE (AChR+) GENERALIZED MYASTHENIA GRAVIS	Meghana Shownkeen, Arjun Seth	<p>INTRODUCTION: Zilucoplan was developed as therapy for treatment refractory cases of AChR+ generalized myasthenia gravis. There is no data on combined use of zilucoplan (a macrocyclic peptide C5 inhibitor) and rituximab (chimeric anti-CD20 monoclonal antibody). We report a case of refractory generalized myasthenia gravis in which zilucoplan and rituximab were used in combination to better manage myasthenia symptoms.</p> <p>CASE REPORT: A 23-year-old woman with AChR+ myasthenia gravis since age 17 status post thymectomy was on eculizumab every 2 weeks with breakthrough symptoms within 5 days of her infusions. Her initial AChR antibody titer was 475nmol/L. She was trialed on efgartigimod for 2 cycles, but developed myasthenia exacerbations that were responsive to plasma exchange. She received one cycle of rituximab 1g IV x 2. AChR titers dropped to 263.8 nmol/L. She remained symptomatic (MG-ADL > 6), restarted eculizumab with prednisone and mycophenolate mofetil. After 3 months, she transitioned to ravulizumab. 6 weeks later, she had a myasthenia exacerbation that responded to plasma exchange. She resumed eculizumab, continued to have breakthrough symptoms, and 2 months later was started on zilucoplan. However, she continued with intermittent breakthrough weakness, requiring prednisone and mycophenolate. Rituximab 1g IV x 2 every 6 months was initiated, mycophenolate was stopped, and she had more stability in her myasthenia symptoms, less fluctuation, and improvement of MG-ADL to 3.</p> <p>SUMMARY/CONCLUSION: This case demonstrates combination therapy of a C5 peptide inhibitor and a CD20 monoclonal antibody as a possible treatment strategy to better manage the clinical symptoms of refractory myasthenia gravis</p>
82	COMBINING COMPLEMENT INHIBITION AND ANTIBODY REDUCTION IN REFRACTORY AChR GENERALIZED MYASTHENIA GRAVIS	Michael Slama, David Weinberg, Amanda Guidon	<p>INTRODUCTION: Complement inhibition, B-cell depletion and FcRn blockade may each partially benefit patients with refractory AChR generalized myasthenia gravis (gMG). To achieve remission or minimal manifestations, combining complement inhibition with either B-cell depletion or FcRn blockade provides a strategy to simultaneously target two aspects of MG pathophysiology: complement-mediated destruction of the post-synaptic membrane architecture and interaction of pathogenic antibodies with AChR receptors.</p> <p>OBJECTIVE: To describe clinical outcomes and safety following combined therapy with a complement inhibitor and either rituximab or efgartigimod in 5 patients with treatment-refractory AChR gMG.</p> <p>METHODS: We identified 5 patients treated with this strategy by three physicians in outpatient neuromuscular clinics. We assessed gMG symptoms before and during combination treatment, using patient-reported (MG-ADL, MG-QOL15), composite (MGC) and objective (MG-MMT) scales.</p> <p>RESULTS: Clinical improvements were seen in 3 patients (Pt#1: eculizumab+rituximab, Pt#2: ravulizumab+rituximab, Pt#3: zilucoplan+efgartigimod). No definite changes were seen in Pt#4 (ravulizumab+rituximab). Pt#5 initially improved but later worsened (on eculizumab+rituximab, then zilucoplan+rituximab, then zilucoplan+efgartigimod). Responders had early-onset MG (ages 24, 36, 37 at onset) and longer disease duration (17, 30 and 14 years respectively) compared to nonresponders (56, 79 at onset; 6, 1.5 years disease duration respectively). All patients concomitantly received azathioprine, mycophenolate or cyclosporine; 4 also received prednisone. Pt#2 and Pt#3 developed localized herpes zoster infections. Pt#5 had a mild COVID infection.</p> <p>SUMMARY/CONCLUSION: Combination of complement inhibition with either B-cell depletion or FcRn blockade may improve symptom control in some patients with treatment-refractory myasthenia. Additional studies are needed to better understand the safety and efficacy of this approach.</p>

83	AN ANTIGEN-SPECIFIC CHIMERIC AUTOANTIBODY RECEPTOR T CELL STRATEGY FOR THE ELIMINATION OF ANTI-MAIN IMMUNOGENIC REGION ANTIBODY-SECRETING B CELLS	Vu Trinh, David Richman, Lucia Borges, Cristian Musson	<p>INTRODUCTION: 50–70% of the anti-AChR autoAbs in myasthenia gravis (MG) sera and in the sera of its animal model, experimental autoimmune MG, are directed to an immunological hot spot called the main immunogenic region (MIR). Removing the B cells producing these anti-MIR Abs, offers a targeted antigen-specific approach to treating MG. Our objective is to develop MIR chimeric auto-antibody receptor (CAAR)-expressing T cells (MIR CAAR T cells) to specifically target the B cells that secrete the anti-AChR autoAbs, with little or no effect on the rest of the normal functioning of the immune system.</p> <p>OBJECTIVE: To develop a CAAR T cell therapy for MG that removes the MIR producing B-cells.</p> <p>METHODS: As an initial therapeutic, we attached the MIR peptide to the N-termini of an Fcγ1. EAMG rats were treated with IP injections, 2mg/kg, of the soluble MIR-Fc biologic. Autologous rat T cells are being engineered to express a CAAR consisting of our engineered MIR peptide, fused to CD137-CD3ζ T cell receptor signaling domains (MIR CAAR). The MIR CAAR directs the CAAR T cells to bind to and kill anti-MIR Ab-producing B cells.</p> <p>RESULTS: The anti-AChR titers and anti-MIR titers in the control-treated group continued to increase while titers in the treated group remained low. HEK 293 cells were transiently transfected with the lentiviral plasmid expressing MIR CAAR. Anti-AChR sera stained these HEK 293 cells (37.5%), while normal rat sera did not (1.05%).</p> <p>SUMMARY/CONCLUSION: A CAAR T cell therapeutic capable of killing B cells producing anti-MIR autoAbs which has the potential to durably treat MG.</p>
84	RESPONSE TO ROZANOLIXIZUMAB IN PATIENTS WITH GENERALIZED MYASTHENIA GRAVIS: FINAL POOLED ANALYSIS OF MYCARING AND OPEN-LABEL EXTENSION STUDIES	Tuan Vu, Carlo Antozzi, Julian Grosskreutz, Ali Habib, Sabrina Sacconi, Kimiaki Utsumigawa, John Vissing, Fiona Grimson, Thais Tannocin, Vera Bril	<p>INTRODUCTION: One 6-week cycle of rozanolixizumab improved myasthenia gravis (MG)-specific outcomes versus placebo in the Phase 3 MycarinG study (MG0003/NCT03971422). Following MycarinG, patients could enroll in open-label extension studies: MG0004 (NCT04124965) then MG0007 (NCT04650854), or MG0007 directly.</p> <p>OBJECTIVE: Evaluate rozanolixizumab response in patients with generalized MG using MG-Activities of Daily Living (MG-ADL) responder thresholds.</p> <p>METHODS: In MG0004, patients received once-weekly rozanolixizumab (7mg/kg or 10mg/kg) for ≤52 weeks. MG0007 comprised an initial 6-week cycle (7mg/kg or 10mg/kg) with further cycles administered upon symptom worsening. Final data were pooled across MycarinG, MG0004 (first 6 weeks) and MG0007 for patients with ≥2 symptom-driven cycles (efficacy) and MycarinG/MG0007 for patients with ≥1 treatment cycle (safety). Response was prespecified as a ≥2.0-point improvement in MG-ADL score at Day 43 in each cycle; ≥3.0-point and ≥5.0-point thresholds were assessed post hoc.</p> <p>RESULTS: Overall, 129 patients received ≥2 symptom-driven cycles (7mg/kg or 10mg/kg). The proportion achieving a ≥2.0-point improvement in MG-ADL score was 74.4% (n/N=96/129) in Cycle 1 and 88.2% (n/N=15/17) in Cycle 13; lowest proportion in Cycle 3 (63.7%, n/N=72/113) and highest in Cycle 13. A ≥3.0-point improvement was achieved by 62.8% (n/N=81/129 [Cycle 1]) and 82.4% (n/N=14/17 [Cycle 13]); minimum, 54.0% (n/N=6/113 [Cycle 3]); maximum, 83.3% (n/N=20/24 [Cycle 12]). A ≥5.0-point improvement was achieved by 36.4% (n/N=47/129 [Cycle 1]) and 52.9% (n/N=9/17 [Cycle 13]); minimum, 30.6% (n/N=19/62 [Cycle 8]); maximum, 66.7% (n/N=16/24 [Cycle 12]). Treatment-emergent adverse events occurred in 93.1% (n/N=175/188) of patients, most mild/moderate.</p> <p>SUMMARY/CONCLUSION: Rozanolixizumab demonstrated consistent efficacy up to Cycle 13 for each MG-ADL threshold. Funding: UCB.</p> <p>INTRODUCTION: In the Phase 3 MycarinG study (NCT03971422), adults with generalized myasthenia gravis (MG) received one 6-week rozanolixizumab treatment cycle. Following MycarinG, patients could enroll in the open-label extension MG0007 (NCT04650854) for a further cycle, with subsequent cycles based on symptom worsening (investigator's discretion). This needs-based approach led to inter-patient variability in the number of cycles received.</p> <p>OBJECTIVE: Describe rozanolixizumab treatment patterns and assess their associations with baseline patient characteristics.</p> <p>METHODS: Patients with ≥1 treatment cycle across MycarinG and MG0007 (final data) were separated into distinct clusters using K-means cluster analysis based on number of cycles per year. Associations between baseline characteristics (including age, sex, MG-Activities of Daily Living [MG-ADL] score, Quantitative MG score and antibody status) and cycles per year were assessed using multivariate regression models. Treatment-emergent adverse events were evaluated by cluster.</p> <p>RESULTS: Overall, 188 patients received ≥1 treatment cycle (mean: 2.9 cycles per year [standard deviation (SD): 1.8]). The most balanced clustering and optimal goodness-of-fit was achieved using three clusters (low: <2.42 cycles per year; medium: 2.42–4.42; high: >4.42). Mean (SD) cycles per year in each cluster were 1.27 (0.49; n=89), 3.47 (0.61; n=52) and 5.44 (0.63; n=47), respectively. There were no associations between baseline characteristics and number of cycles, except for MG-ADL: scores ≥5 being associated with a mean increase of 1.06 (95% confidence interval: 0.24, 1.89) cycles per year versus <5. Rozanolixizumab was generally well tolerated across the clusters.</p> <p>SUMMARY/CONCLUSION: Physicians and patients adopted an individualized rozanolixizumab treatment approach, resulting in varied cycle cadence between patients. Funding: UCB.</p>
85	ROZANOLIXIZUMAB TREATMENT PATTERNS IN PATIENTS WITH GENERALIZED MYASTHENIA GRAVIS: A POST HOC ANALYSIS OF FINAL POOLED PHASE 3 DATA	Tuan Vu, Ali A. Habib, Sabrina Sacconi, Kimiaki Utsumigawa, John Vissing, Marion Boehllein, Fiona Grimson, Irene Pulido-Valkodolivas, Thais Tannocin, Vera Bril	<p>INTRODUCTION: Nipocalimab is a fully human glycosylated monoclonal antibody with high affinity and specificity for human neonatal fragment crystallizable receptor (FcRn) (dissociation-constant [KD] ≤31.7 pM, pH 6.0; KD ≤57.8 pM, pH 7.4). Nipocalimab blocks the binding of immunoglobulin G (IgG) to FcRn, resulting in decreased circulating IgG levels, including pathogenic IgG. Nipocalimab is being studied in several autoimmune and alloantibody diseases and has been approved for use in anti-AChR and anti-MuSK antibody positive patients with gMG.</p> <p>OBJECTIVE: To compare FcRn blockers, nipocalimab vs efgartigimod, with respect to their structural interactions with FcRn, binding affinities to FcRn, and potency of inhibition of IgG recycling in human aortic endothelial cells (HAECs).</p> <p>METHODS: Nipocalimab binding epitopes on FcRn were determined using X-ray crystallography. Efgartigimod-FcRn complex structure was modeled based on the FcRn-IgG structure. Binding affinities were determined using surface plasmon resonance. Effects on IgG recycling were determined using an HAECs-based assay.</p> <p>RESULTS: Nipocalimab has a larger binding interface with FcRn (1017.5 square angstroms) compared with efgartigimod (651.3 square angstroms). Nipocalimab binds with high affinity (low pM) to FcRn at both neutral and acidic pH. Whereas efgartigimod has a much lower, pH-dependent binding affinity (KD=16 nM, pH 6; KD=208 nM, pH 7.4). In cell-based assays nipocalimab is more potent (≥250 fold) compared with efgartigimod in inhibiting IgG recycling.</p> <p>SUMMARY/CONCLUSION: There are differences in the binding epitopes, binding affinities, and inhibition of IgG recycling between nipocalimab and efgartigimod, with nipocalimab demonstrating a larger binding interface with FcRn, a greater binding affinity and greater in-vitro inhibition of IgG recycling.</p> <p>INTRODUCTION: In 24-week (W) double-blind phase-3 Vivacity-MG3 study (NCT04951622), nipocalimab+standard-of-care (SOC) demonstrated statistically significant and clinically meaningful improvements versus placebo+SOC in patients with generalized myasthenia gravis (gMG).</p> <p>OBJECTIVE: To assess the efficacy in placebo+SOC patients who completed the double-blind phase and transitioned to nipocalimab+SOC in ongoing open-label extension phase (OLE) of Vivacity-MG3.</p> <p>METHODS: In OLE, 98 patients from placebo+SOC transitioned to nipocalimab+SOC. Data were collected up to OLE W24 (cutoff: 23-August-2024). Mean changes in MG-Activities of Daily Living (MG-ADL) and Quantitative MG (QMG) scores from OLE baseline were evaluated. Within-group mean changes were examined using paired t-test. Percentage of patients achieving Meaningful Clinical Improvement (MCI; ≥2-point within-patient improvement versus baseline in MG-ADL) and sustained MCI (for ≥8W), and percentage-of-time spent in MCI were summarized.</p> <p>RESULTS: Mean(SD) MG-ADL and QMG scores at OLE baseline were 6.33(3.37) and 13.47(5.70), respectively. Improvements in MG-ADL score were observed as early as OLE-W2 in placebo+SOC patients transitioned to nipocalimab+SOC: mean (SD) change of -1.33(2.13), n=87, p<0.001), improving to -2.68(3.26), n=59, p<0.001) at OLE-W24. At W24, 63.3% of patients achieved MCI, with 79.5% achieving it at any time during OLE; 51.1% of patients had sustained MCI. Similarly, as early as OLE-W4, QMG improvement was observed with mean(SD) change of -2.65 (3.95), n=79, p<0.001), continuing to -3.24(4.95), n=58, p<0.001) at OLE-W24.</p> <p>SUMMARY/CONCLUSION: Placebo+SOC patients with gMG from Vivacity-MG3 who transitioned to nipocalimab+SOC exhibited improvements in MG-ADL as early as W2 after transition, with continued improvement through W24. This supports the potential of nipocalimab as an effective maintenance treatment option in this gMG population.</p>
86	INSIGHTS INTO NIPOCALIMAB-NEONATAL FRAGMENT CRYSTALLIZABLE RECEPTOR STRUCTURE, BINDING AFFINITY, AND INHIBITION OF IMMUNOGLOBULIN G RECYCLING: COMPARISON WITH EFGARTIGIMOD	Nilufer Seth, Rui Xu, Brian Stoveken, Matthew DuPrie, Samuel Sihapong, Traymon Beavers, Leona F. Ling, Maria Ait-Tihyaty, John J. Sheehan, Tuan Vu	<p>INTRODUCTION: Nipocalimab is a fully human glycosylated monoclonal antibody with high affinity and specificity for human neonatal fragment crystallizable receptor (FcRn) (dissociation-constant [KD] ≤31.7 pM, pH 6.0; KD ≤57.8 pM, pH 7.4). Nipocalimab blocks the binding of immunoglobulin G (IgG) to FcRn, resulting in decreased circulating IgG levels, including pathogenic IgG. Nipocalimab is being studied in several autoimmune and alloantibody diseases and has been approved for use in anti-AChR and anti-MuSK antibody positive patients with gMG.</p> <p>OBJECTIVE: To compare FcRn blockers, nipocalimab vs efgartigimod, with respect to their structural interactions with FcRn, binding affinities to FcRn, and potency of inhibition of IgG recycling in human aortic endothelial cells (HAECs).</p> <p>METHODS: Nipocalimab binding epitopes on FcRn were determined using X-ray crystallography. Efgartigimod-FcRn complex structure was modeled based on the FcRn-IgG structure. Binding affinities were determined using surface plasmon resonance. Effects on IgG recycling were determined using an HAECs-based assay.</p> <p>RESULTS: Nipocalimab has a larger binding interface with FcRn (1017.5 square angstroms) compared with efgartigimod (651.3 square angstroms). Nipocalimab binds with high affinity (low pM) to FcRn at both neutral and acidic pH. Whereas efgartigimod has a much lower, pH-dependent binding affinity (KD=16 nM, pH 6; KD=208 nM, pH 7.4). In cell-based assays nipocalimab is more potent (≥250 fold) compared with efgartigimod in inhibiting IgG recycling.</p> <p>SUMMARY/CONCLUSION: There are differences in the binding epitopes, binding affinities, and inhibition of IgG recycling between nipocalimab and efgartigimod, with nipocalimab demonstrating a larger binding interface with FcRn, a greater binding affinity and greater in-vitro inhibition of IgG recycling.</p> <p>INTRODUCTION: In 24-week (W) double-blind phase-3 Vivacity-MG3 study (NCT04951622), nipocalimab+standard-of-care (SOC) demonstrated statistically significant and clinically meaningful improvements versus placebo+SOC in patients with generalized myasthenia gravis (gMG).</p> <p>OBJECTIVE: To assess the efficacy in placebo+SOC patients who completed the double-blind phase and transitioned to nipocalimab+SOC in ongoing open-label extension phase (OLE) of Vivacity-MG3.</p> <p>METHODS: In OLE, 98 patients from placebo+SOC transitioned to nipocalimab+SOC. Data were collected up to OLE W24 (cutoff: 23-August-2024). Mean changes in MG-Activities of Daily Living (MG-ADL) and Quantitative MG (QMG) scores from OLE baseline were evaluated. Within-group mean changes were examined using paired t-test. Percentage of patients achieving Meaningful Clinical Improvement (MCI; ≥2-point within-patient improvement versus baseline in MG-ADL) and sustained MCI (for ≥8W), and percentage-of-time spent in MCI were summarized.</p> <p>RESULTS: Mean(SD) MG-ADL and QMG scores at OLE baseline were 6.33(3.37) and 13.47(5.70), respectively. Improvements in MG-ADL score were observed as early as OLE-W2 in placebo+SOC patients transitioned to nipocalimab+SOC: mean (SD) change of -1.33(2.13), n=87, p<0.001), improving to -2.68(3.26), n=59, p<0.001) at OLE-W24. At W24, 63.3% of patients achieved MCI, with 79.5% achieving it at any time during OLE; 51.1% of patients had sustained MCI. Similarly, as early as OLE-W4, QMG improvement was observed with mean(SD) change of -2.65 (3.95), n=79, p<0.001), continuing to -3.24(4.95), n=58, p<0.001) at OLE-W24.</p> <p>SUMMARY/CONCLUSION: Placebo+SOC patients with gMG from Vivacity-MG3 who transitioned to nipocalimab+SOC exhibited improvements in MG-ADL as early as W2 after transition, with continued improvement through W24. This supports the potential of nipocalimab as an effective maintenance treatment option in this gMG population.</p>
87	EFFICACY OF NIPOCALIMAB IN OPEN-LABEL EXTENSION IN PATIENTS TRANSITIONED FROM PLACEBO: RESULTS FROM VIVACITY-MG3 TRIAL	Kristl Clacys, Maria Ait-Tihyaty, Kavita Gandhi, Ibrahim Turkoo, Zia Choudhry, Wim Noel, Charlotte Gary, Sindhu Ramchandren, Tuan Vu	<p>INTRODUCTION: Nipocalimab is a fully human glycosylated monoclonal antibody with high affinity and specificity for human neonatal fragment crystallizable receptor (FcRn) (dissociation-constant [KD] ≤31.7 pM, pH 6.0; KD ≤57.8 pM, pH 7.4). Nipocalimab blocks the binding of immunoglobulin G (IgG) to FcRn, resulting in decreased circulating IgG levels, including pathogenic IgG. Nipocalimab is being studied in several autoimmune and alloantibody diseases and has been approved for use in anti-AChR and anti-MuSK antibody positive patients with gMG.</p> <p>OBJECTIVE: To compare FcRn blockers, nipocalimab vs efgartigimod, with respect to their structural interactions with FcRn, binding affinities to FcRn, and potency of inhibition of IgG recycling in human aortic endothelial cells (HAECs).</p> <p>METHODS: Nipocalimab binding epitopes on FcRn were determined using X-ray crystallography. Efgartigimod-FcRn complex structure was modeled based on the FcRn-IgG structure. Binding affinities were determined using surface plasmon resonance. Effects on IgG recycling were determined using an HAECs-based assay.</p> <p>RESULTS: Nipocalimab has a larger binding interface with FcRn (1017.5 square angstroms) compared with efgartigimod (651.3 square angstroms). Nipocalimab binds with high affinity (low pM) to FcRn at both neutral and acidic pH. Whereas efgartigimod has a much lower, pH-dependent binding affinity (KD=16 nM, pH 6; KD=208 nM, pH 7.4). In cell-based assays nipocalimab is more potent (≥250 fold) compared with efgartigimod in inhibiting IgG recycling.</p> <p>SUMMARY/CONCLUSION: There are differences in the binding epitopes, binding affinities, and inhibition of IgG recycling between nipocalimab and efgartigimod, with nipocalimab demonstrating a larger binding interface with FcRn, a greater binding affinity and greater in-vitro inhibition of IgG recycling.</p> <p>INTRODUCTION: In 24-week (W) double-blind phase-3 Vivacity-MG3 study (NCT04951622), nipocalimab+standard-of-care (SOC) demonstrated statistically significant and clinically meaningful improvements versus placebo+SOC in patients with generalized myasthenia gravis (gMG).</p> <p>OBJECTIVE: To assess the efficacy in placebo+SOC patients who completed the double-blind phase and transitioned to nipocalimab+SOC in ongoing open-label extension phase (OLE) of Vivacity-MG3.</p> <p>METHODS: In OLE, 98 patients from placebo+SOC transitioned to nipocalimab+SOC. Data were collected up to OLE W24 (cutoff: 23-August-2024). Mean changes in MG-Activities of Daily Living (MG-ADL) and Quantitative MG (QMG) scores from OLE baseline were evaluated. Within-group mean changes were examined using paired t-test. Percentage of patients achieving Meaningful Clinical Improvement (MCI; ≥2-point within-patient improvement versus baseline in MG-ADL) and sustained MCI (for ≥8W), and percentage-of-time spent in MCI were summarized.</p> <p>RESULTS: Mean(SD) MG-ADL and QMG scores at OLE baseline were 6.33(3.37) and 13.47(5.70), respectively. Improvements in MG-ADL score were observed as early as OLE-W2 in placebo+SOC patients transitioned to nipocalimab+SOC: mean (SD) change of -1.33(2.13), n=87, p<0.001), improving to -2.68(3.26), n=59, p<0.001) at OLE-W24. At W24, 63.3% of patients achieved MCI, with 79.5% achieving it at any time during OLE; 51.1% of patients had sustained MCI. Similarly, as early as OLE-W4, QMG improvement was observed with mean(SD) change of -2.65 (3.95), n=79, p<0.001), continuing to -3.24(4.95), n=58, p<0.001) at OLE-W24.</p> <p>SUMMARY/CONCLUSION: Placebo+SOC patients with gMG from Vivacity-MG3 who transitioned to nipocalimab+SOC exhibited improvements in MG-ADL as early as W2 after transition, with continued improvement through W24. This supports the potential of nipocalimab as an effective maintenance treatment option in this gMG population.</p>

88	EFFECT OF ZILUCOPLAN ON FATIGUE IN PATIENTS WITH GENERALIZED MYASTHENIA GRAVIS: RAISE-XT 120-WEEK FOLLOW-UP	Michael Weiss, Miriam Freimer, Channa Hevamadduma, Angelina Mantaol, Kimiaki Utsumisawa, Jos Bloemers, Babak Borojerdi, Natas Savic, James F. Howard Jr., Marina Marchewcz	<p>INTRODUCTION: Fatigue negatively impacts the quality of life of many patients with generalized myasthenia gravis (gMG). The Phase 3, double-blind, 12-week RAISE study (NCT04115293) demonstrated that treatment with zilucoplan, a complement component 5 (C5) inhibitor, resulted in clinically meaningful and nominally significant improvement in fatigue versus placebo in patients with gMG.</p> <p>OBJECTIVE: This post hoc analysis assessed the long-term effect of zilucoplan on fatigue in patients with gMG in the ongoing open-label extension study RAISE-XT (NCT04225871) up to Week 120.</p> <p>METHODS: Adults with anti-acetylcholine receptor antibody-positive gMG who completed the double-blind RAISE study could opt to enter RAISE-XT and self-administer daily subcutaneous zilucoplan 0.3 mg/kg injections. Here, we report change in Quality of Life in Neurological Disorders (Neuro-QoL) Short Form Fatigue T-scores and fatigue severity levels from RAISE baseline to Week 120.</p> <p>RESULTS: At Week 120, the mean change from baseline in the Neuro-QoL Short Form Fatigue T-score was -12.65 (standard error 1.53, N=166). Of patients with available data at baseline and Week 120, 77.0% (47/61) showed clinically meaningful improvement (≥3.5-point decrease) in their T-score at Week 120 compared with baseline, and 60.7% (37/61) transitioned to a lower fatigue severity level. At Week 120, 60.7% (37/61) of patients had mild or no fatigue compared with 19.7% (12/61) at baseline.</p> <p>SUMMARY/CONCLUSION: Treatment with zilucoplan improved fatigue in patients with gMG, sustained in the long term, up to 120 weeks, with the majority of patients improving by ≥1 fatigue severity level. <i>Funding:</i> UCB</p>
89	INTERPRETING PATIENT QUALITY-OF-LIFE EXPERIENCE WITH ZILUCOPLAN TREATMENT IN GENERALIZED MYASTHENIA GRAVIS IN RAISE AND RAISE-XT	Michael D. Weiss, Miriam Freimer, Channa Hevamadduma, M. Isabel Leite, Angelina Mantaol, Kimiaki Utsumisawa, Tuan Vu, Jos Bloemers, Babak Borojerdi, Shien Guo, Paul Mahoney, Natas Savic, James F. Howard Jr.	<p>INTRODUCTION: In the Phase 3, double-blind RAISE study (NCT04115293), treatment with zilucoplan, a complement component 5 inhibitor, resulted in statistically significant improvements in the Myasthenia Gravis Quality of Life 15-item revised (MG-QoL15r) total score versus placebo in patients with generalized myasthenia gravis (gMG). However, the meaningfulness of these differences for patients can be difficult to interpret.</p> <p>OBJECTIVE: To interpret the meaningfulness of MG-QoL15r improvements, we determined the most likely response to each item of the MG-QoL15r scale before and after zilucoplan treatment in RAISE and the ongoing open-label extension study, RAISE-XT (NCT04225871).</p> <p>METHODS: In RAISE, adults with anti-acetylcholine receptor antibody-positive gMG were randomized to self-administer once-daily placebo or zilucoplan 0.3mg/kg. Adults who completed RAISE received zilucoplan 0.3mg/kg in RAISE-XT. The Rasch Rating Scale Model was used post hoc to determine the probability of item responses for the MG-QoL15r scale.</p> <p>RESULTS: At Week 12, patients on zilucoplan were more likely than those on placebo to answer "not at all" for all items, including for the most severe symptoms (e.g., for "difficulty speaking", placebo: 41.9%; zilucoplan: 63.9%). For the total population, this likelihood increased at Week 60 (e.g., for "difficulty speaking", the likelihood of answering "not at all" was: 18.5% [RAISE baseline]; 80.7% [Week 60]).</p> <p>SUMMARY/CONCLUSION: In RAISE, patients on zilucoplan were more likely to respond favorably to the MG-QoL15r items than patients on placebo, demonstrating they typically no longer experienced the more severe symptoms. This likelihood increased through Week 60. These findings support the interpretation of the meaningfulness of changes in the MG-QoL15r score. <i>Funding:</i> UCB</p>
90	INVISIBLE MARKERS, VISIBLE IMPACT: GREATER DISEASE BURDEN IN SERONEGATIVE MYASTHENIA GRAVIS	Wade Whitt, Emma Ciafaloni, Phillip Mongiovi, Alexis Lizaraga	<p>INTRODUCTION: Controversy exists in the literature regarding disease severity between seronegative and seropositive myasthenia gravis (SNMG and SPMG) with some suggesting a greater symptom burden in SPMG and others suggesting the contrary. Some outcome measures that have been used in the include: Gravis Activities of Daily Living score (MG-ADL) and Myasthenia Gravis Foundation of America (MGFA) class as well as number of hospitalizations for myasthenic crises.</p> <p>OBJECTIVE: To compare disease severity and clinical outcomes between SNMG and SPMG.</p> <p>METHODS: This is a retrospective cohort study of SPMG and SNMG patients in the UPMC neuromuscular disease clinic via medical chart review. Data included demographics, MG-ADL scores, and MGFA class, hospitalizations, and treatment outcomes. Data was analyzed using SPSS using chi-square and t-test.</p> <p>RESULTS: A total 88 patients were identified (56 seropositive and 32 seronegative). SNMG patients had significantly higher MG-ADL scores compared to SPMG patients (initial MG-ADL score: 7.4 vs. 4.28, p<0.001, latest MG-ADL score: 5.48 vs. 3.47 p=0.008). Less SNMG were on immunomodulating therapy than SPMG patients (84% vs. 34%, p=0.049). Hospitalization rate was not significantly different between the two groups (SPMG: 41%; SNMG: 25%, p=0.114). Hospitalization length of stay was significantly longer in SPMG patients (11.6 days vs. 5.07 days, p=0.04).</p> <p>SUMMARY/CONCLUSION: This study shows that SNMG patients have higher average MG-ADL scores and similar rates of hospitalizations for myasthenic crises to SPMG patients. However, SNMG patients are treated using less immunomodulating therapy than SPMG, possibly because there are far fewer options for treating them than SPMG.</p>
91	DEMOGRAPHIC AND CLINICAL PREDICTORS OF PERSISTENT OPHTHALMOPLÉGIA IN JUVENILE MYASTHENIA GRAVIS	Sarah Wright, Simrun Uppal	<p>INTRODUCTION: Juvenile Myasthenia Gravis (JMG) is a rare disease causing muscular weakness, fatigue, and morbidity in children under 18. Ocular Myasthenia Gravis (OMG), involving only eye muscles and ptosis, is more common in pre-pubertal children and may remit spontaneously. However, some children develop persistent ophthalmoplegia leading to permanent visual impairment. While genetic factors may play a role, the impact of demographic and socioeconomic factors on disease progression remains poorly understood, particularly in U.S. populations.</p> <p>OBJECTIVE: This study aims to identify demographic and clinical risk factors associated with persistent ophthalmoplegia in children with OMG and to explore disparities that may inform early biomarkers and equitable management strategies.</p> <p>METHODS: A retrospective chart review was conducted at Children's National Hospital (Washington, D.C.; 2005–present), identifying patients with ICD-10 codes for myasthenia gravis and ophthalmoplegic conditions. Demographics, clinical features, treatments, and ocular outcomes were analyzed descriptively. Fisher's exact test was used to assess odds ratios for individual risk factors.</p> <p>RESULTS: Of 46 identified patients, 20 met criteria for JMG and were included in the final analysis. Mean age of onset was 7.55 years, most pre-pubertal. African American patients comprised 45% of the cohort. AChR antibodies were present in 55%. Persistent ophthalmoplegia was observed in 7 patients (35%) and was more likely in those of African American descent and with OMG.</p> <p>SUMMARY/CONCLUSION: In this small JMG cohort, persistent ophthalmoplegia affected over one-third of patients, with a disproportionate impact observed among African American children. These findings underscore the need for early biomarkers of disease progression and targeted, equitable management.</p>
92	ASSESSING ORAL CORTICOSTEROID TAPERING IN RAVULIZUMAB-TREATED ADULTS WITH ANTI-ACETYLCHOLINE RECEPTOR ANTIBODY-POSITIVE GENERALIZED MYASTHENIA GRAVIS: THE PHASE 4, GLOBAL OCTAGON STUDY DESIGN	Benjamin Yungler, Rasha Aguzzi, Emma Weiskopf, Guido Sabatella	<p>INTRODUCTION: Anti-acetylcholine receptor antibody-positive (AChR-Ab+) generalized myasthenia gravis (gMG) is a rare, chronic, autoimmune neuromuscular disease characterized by fatigable muscle weakness. Although immunosuppressive therapies, including oral corticosteroids (OCS), can be effective in gMG, high-dose and long-term use can cause significant adverse effects. Ravulizumab, a complement component 5 inhibitor, is approved for the treatment of AChR-Ab+ gMG, and treatment has been shown to permit reduction and discontinuation of OCS while maintaining symptom control. Current gMG treatment guidelines recommend steroid-sparing strategies, however, there are limited protocols on OCS tapering in patients with gMG.</p> <p>OBJECTIVES: To evaluate the effectiveness and safety of a rapid steroid reduction algorithm in patients with AChR-Ab+ gMG receiving ravulizumab.</p> <p>METHODS: This phase 4, global study will aim to enroll approximately 75 adults with AChR-Ab+ gMG who have received ravulizumab for a maximum of 6 months and are on a stable OCS dosage of ≤ 7.5 mg/day for ≥ 4 weeks. The planned primary outcome will be the proportion of patients who achieve an OCS dosage of ≤ 5 mg/day without clinical deterioration of gMG if the reason for no further discontinuation was suspected adrenal insufficiency. The proportion of patients with 100% reduction in daily OCS use without clinical deterioration of gMG will also be assessed as a key secondary outcome.</p> <p>RESULTS: Full study design details and estimated enrollment timing will be presented.</p> <p>SUMMARY/CONCLUSION: The OCTAGON study will evaluate the effectiveness and safety of a rapid steroid reduction algorithm in patients with AChR-Ab+ gMG receiving ravulizumab.</p>

			<p>INTRODUCTION: A whole-body physiologically-based-pharmacokinetic (PBPK) model can predict both PK and pharmacodynamic (PD) of FcRn inhibitors (efgartigimod, rozanoliczumab, nipoalimab and batoclimab) in both mother and fetus, and model placental transfer.</p> <p>OBJECTIVE: To refine a previously developed PBPK model for FcRn inhibitors by incorporating published data and applying to predict their transfer and IgG transport across the placenta in pregnancy.</p> <p>METHODS: A whole-body PBPK model was developed for healthy non-pregnant participants. The model was extended to pregnant women population incorporating placental transfer using data from nipoalimab-treated pregnant women at high-risk for early onset severe hemolytic disease of the fetus and newborn.</p> <p>RESULTS: The refined FcRn inhibitor model described the PK and PD (immunoglobulin-G [IgG] profiles) of FcRn inhibitors after optimization of two system-specific parameters and three compound-specific parameters. PK were well described for all drugs and showed a strong target-mediated drug disposition effect. The model captured the observed increase of fetal endogenous IgG with the progression of gestational age. The developed placental transfer model was applied to predict fetal drug exposure and, fetal FcRn blockade, and inhibition of endogenous IgG transfer from the maternal to fetal blood circulation. Predictions showed that these FcRn inhibitors differed substantially with respect to fetal exposure-to-drug and FcRn inhibition of maternal IgG transfer, leading to distinct fetal IgG concentration-time profiles.</p> <p>SUMMARY/CONCLUSION: The developed PBPK model was applied to predict fetal drug-exposure, and the inhibition of FcRn-mediated transport of endogenous maternal IgG across the placenta by FcRn inhibitors. Further validation of the fetal IgG concentrations and the placental transfer model is warranted.</p>
93	DEVELOPMENT OF A WHOLE-BODY PHYSIOLOGICALLY-BASED PHARMACOKINETIC MODEL FOR FCYN INHIBITORS IN PREGNANCY INCORPORATING PLACENTAL TRANSFER	Sophie Fischer-Holzhausen, Wilbert de Witte, Edwin Lain, Eleni Caratzas, Stephan Schaller, Yaowei Zhu, An Vermeulen, Jocelyn Leu	<p>INTRODUCTION: Neonatal Fc Receptor (FcRn), which mediates immunoglobulin G (IgG) recycling, is a promising target for treatment of auto-immune diseases such as generalized myasthenia gravis (gMG).</p> <p>OBJECTIVE: To inform the upcoming EPIC clinical trial (NCT05327114) and clinical practice involving switching FcRn-blockers in gMG, we simulated total serum IgG reductions when switching from efgartigimod to nipoalimab.</p> <p>METHODS: Population pharmacokinetic/pharmacodynamic models for both drugs were combined. The nipoalimab model described IgG turnover using an intrinsic catabolic rate and FcRn-mediated recycling rate. Recycling was inhibited based on the amount of FcRn bound to nipoalimab. Efgartigimod model was reparametrized to align with nipoalimab IgG model. Using nipoalimab-derived IgG systemic parameters, the model also fits IgG data from efgartigimod ADAPT trial.</p>
94	PREDICTING TOTAL IMMUNOGLOBULIN G CHANGE FROM BASELINE WHEN SWITCHING FROM EFGARTIGIMOD TO NIPOCALIMAB	Martine Neyens, Yaowei Zhu, Nolan Campbell, John J. Sheehan, Ruben Faelsens	<p>RESULTS: De novo initiation of nipoalimab IV 30mg/kg loading dose + 15mg/kg every-2-weeks resulted in a rapid initial IgG reduction of 72% at Week(W)2 and subsequently alternated between 80% (odd weeks) and 66% (prior to next dose). Efgartigimod cycles (4W on/4W off) yielded IgG reductions of 65% at W4 and 24% at W8. Switching to nipoalimab after an 8W efgartigimod therapy resulted in robust (>70%) IgG reduction within 1W after switching and reduction of 74% at W2, suggesting no additional reduction in IgG vs de novo initiation. If switching is delayed, for example when IgG levels returned to baseline at 12W after efgartigimod cycle start, the IgG profile post-switching matched that predicted after de novo initiation; robust IgG reduction occurred within 1W after switching.</p> <p>SUMMARY/CONCLUSION: These modeling results support switching to nipoalimab after completing 8W efgartigimod IV cycle.</p> <p>INTRODUCTION: Nipoalimab is a monoclonal antibody that binds to FcRn, inhibiting IgG recycling, and lowering circulating IgG levels, including pathogenic autoantibodies associated with generalized myasthenia gravis (gMG) (anti-AChR [IgG1 and IgG3], anti-MuSK [IgG4], and anti-LRP4 [IgG1 and IgG2]). Clinical studies demonstrated that nipoalimab effectively reduces total serum IgG with favorable efficacy and safety profiles in gMG.</p> <p>OBJECTIVE: To characterize the effects of nipoalimab on IgG subclasses (IgG1-4) and anti-AChR autoantibody levels in patients with gMG.</p> <p>METHODS: Data from phase-2 (N=68) and phase-3 (N=166) studies in patients with gMG were combined to develop a longitudinal pharmacokinetic-pharmacodynamic model. Simulations were conducted to predict changes in IgG subclasses and anti-AChR autoantibody levels over time during steady-state dosing, comparing with profiles reported for efgartigimod and rozanoliczumab.</p>
95	NIPOCALIMAB EFFECT ON IMMUNOGLOBULIN G SUBCLASSES IN PATIENTS WITH GENERALIZED MYASTHENIA GRAVIS	Xia Li, Juan-José Pérez-Ruixo, Yaowei Zhu, Jocelyn H. Leu, John J. Sheehan, Ruben Faelsens	<p>RESULTS: IgG3 exhibited the largest decrease, reaching a maximum 87.3% change from baseline (CFB), and recovered to 54.9% CFB prior to next dose. IgG4 had a maximum 74.7% CFB and recovered to 56.6% CFB. IgG1 and IgG2 had maximum 82.2% and 79.6% CFB, respectively, and recovered to 65.6% and 68.1% CFB, respectively. Anti-AChR autoantibodies decreased to a maximum 88.3% CFB, similar to IgG3. Compared with efgartigimod IV 10mg/kg weekly for 4 weeks and rozanoliczumab SC 7mg/kg and 10mg/kg weekly for 6 weeks, nipoalimab resulted in faster, deeper, and more sustained reductions in IgG (all subclasses) and anti-AChR levels.</p> <p>SUMMARY/CONCLUSION: Nipoalimab significantly inhibits FcRn-mediated IgG recycling, leading to rapid, greater and sustained reductions in all IgG subclasses and pathogenic anti-AChR autoantibodies in gMG compared with efgartigimod and rozanoliczumab, highlighting its therapeutic potential.</p> <p>INTRODUCTION: Nipoalimab is a fully human immunoglobulin (IgG) monoclonal antibody designed to selectively bind and block the IgG binding site on endogenous neonatal Fc receptors (FcRn). An open-label Phase-2/3 study (Vibrance-MG, NCT05265273) of nipoalimab is being conducted in adolescents (aged 12 to <18 years) and children (≥ 2 to ≤ 12 years) with generalized myasthenia gravis (gMG).</p> <p>OBJECTIVE: To confirm the dose regimen for adult patients is also appropriate for adolescents with gMG.</p> <p>METHODS: A previously developed pharmacokinetic (PK)-IgG model for healthy adult participants and adult patients with gMG was adapted to account for expected age- and body weight-related differences in the adolescent gMG population. An external evaluation on the adapted model for adolescents was performed, and the individual post hoc estimates of the PK and pharmacodynamic (PD) parameters for adolescent patients were compared with the corresponding results for adult patients in the phase-3 study (Vivacity-MG3).</p>
96	POPULATION PHARMACOKINETICS AND PHARMACODYNAMICS MODELING OF NIPOCALIMAB IN ADOLESCENTS AGED 12 TO LESS THAN 18 YEARS WITH GENERALIZED MYASTHENIA GRAVIS	Yupeng Ren, Yaowei Zhu, Navin Goyal, Juan-José Pérez-Ruixo, Ruben Faelsens	<p>RESULTS: The external evaluation demonstrated that the adapted model for adolescents could adequately capture both the observed PK and PD data in adolescents. The model-predicted PK (maximum concentration and area under the curve [AUC_{tau}]) and PD (percent change from baseline in pre-dose, average, and nadir total serum IgG) metrics were consistent between adolescent and adult gMG populations.</p> <p>SUMMARY/CONCLUSION: The adult PK-IgG model adapted for adolescents characterized the observed PK and PD profiles in adolescents with gMG. The similar model-predicted PK and PD profiles between adolescents and adults demonstrated that the dose regimen for adults (30 mg/kg intravenous [IV] followed by 15 mg/kg IV every-2-weeks) is also appropriate for adolescents with gMG.</p> <p>INTRODUCTION: Treatment switches in myasthenia gravis (MG) are influenced by efficacy, safety, tolerability, convenience, mechanism of action, and insurance coverage. Understanding drivers of switch and their real-world implementation can inform best practices.</p> <p>OBJECTIVES: This study evaluated neurology healthcare provider (HCP) rationale and methodology for transitioning MG patients from efgartigimod alfa-icab (efgartigimod IV) or efgartigimod alfa and hyaluronidase-qvfc (efgartigimod SC) to ravulizumab, focusing on reasons for discontinuation and outcomes post-switch.</p> <p>METHODS: Retrospective study of MG patients treated with efgartigimod IV or efgartigimod SC who were switched to ravulizumab was conducted using an electronic survey sent to 450 HCPs.</p>
97	RETROSPECTIVE ANALYSIS OF EFGARTIGIMOD ALPHA-FCAB OR EFGARTIGIMOD ALPHA AND HYALURONIDASE-GVFC TO RAVULIZUMAB IN PATIENTS WITH MYASTHENIA GRAVIS (MG)	Christen Kutz, Chloe Sader, Shirali Pandya, Courtney Weir, Sarah Yang	<p>RESULTS: Twenty-one surveys were completed. 62% switched from efgartigimod IV and 43% from efgartigimod SC. Most patients had been on efgartigimod (IV or SC) for under one year before switch (2-6 months: 45%; 6-12 months: 36%). Key drivers for discontinuation included desire to avoid intermittent treatment cycles (33%), worsening symptoms (29%), and perceived lack of efficacy (24%). Ravulizumab was selected for its anticipated efficacy (71%) and convenience of every-8-week dosing (52%). All patients received either 1 or 2 doses of MenACWY and MenB vaccinations before initiating treatment; none received prophylactic antibiotics. HCPs reported 95% of patients improved post-switch. Corticosteroids were discontinued or reduced in 29% patients. At data collection, 90% continued ravulizumab.</p> <p>SUMMARY/CONCLUSION: In real-world practice, transitions from efgartigimod to ravulizumab are often motivated by the limitations of intermittent therapy and inability to meet expectations for improved, sustained disease control. High treatment persistence and reported clinical improvements support ravulizumab's role in MG management.</p>

RETROSPECTIVE ANALYSIS OF INTRAVENOUS IMMUNOGLOBULIN (IVIg) INFUSION SWITCHES TO RAVULIZUMAB (ULTOMIRIS) IN PATIENTS WITH MYASTHENIA GRAVIS (MG) Christen Kutz, Chloe Sader, Shirali Pandya, Jeffrey Tam Sing

INTRODUCTION: Treatment switches in myasthenia gravis (MG) are influenced by factors including efficacy, safety, tolerability, convenience, mechanism of action, and insurance coverage. Better understanding of real-world decision-making and implementation of treatment transitions is critical for optimizing care.

OBJECTIVES: This study evaluated neurology healthcare provider (HCP) rationale and methodology for transitioning MG patients from intravenous immunoglobulin (IVIg) to ravulizumab, focusing on reasons for discontinuation and outcomes post-switch.

METHODS: A retrospective study of MG patients treated with IVIg who were switched to ravulizumab was conducted using an electronic survey sent to 450 HCPs.

RESULTS: Thirty surveys were completed. Patients were 57% male; 74% Caucasian, 14% African American, and 9% Hispanic/Latino. Primary reasons for discontinuing IVIg included worsening MG symptoms (63%), perceived lack of efficacy (51%), and poor tolerability (34%). Ravulizumab was initiated primarily due to hopes for improved efficacy (83%), better tolerability (54%), and provider preference (54%). All patients received either 1 or 2 doses of MenACWY and MenB vaccinations prior to ravulizumab initiation; none received prophylactic antibiotics. 92% patients transitioned ≥4 weeks after their last IVIg dose. Following the switch, 92% experienced clinical improvement; 34% reduced or discontinued corticosteroids. At the time of survey, 84% remained on ravulizumab.

SUMMARY/CONCLUSION: This real-world analysis demonstrates that transitions from IVIg to ravulizumab are driven by unmet needs with prior IVIg therapy and provider confidence in ravulizumab's profile. The high rate of physician-reported improvement and continued treatment supports the clinical value of ravulizumab in MG care.