2019 SCIENTIFIC SESSION
MYASTHENIA GRAVIS
FOUNDATION OF AMERICA
at the 2019 Meeting of the
American Association of Neuromuscular
& Electrodiagnostic Medicine

#MGStrong
www.Myasthenia.org
Welcome Scientists, Clinicians and Guests,

On behalf of the Board of Directors of the Myasthenia Gravis Foundation of America, Inc., welcome to the 2019 Scientific Session of the MGFA Medical and Scientific Advisory Board. We thank you for your interest in MG. This is an exciting time for our community—advances in research and innovative treatments in clinical trials bring us closer to a cure for myasthenia gravis.

Our vision: A World Without MG
Our mission: Create Connections, Enhance Lives and Cure MG

We know we cannot meet our goals without you. Beyond today, we hope that you will be a part of MGFA. There are many ways you can help: speaking at support groups, writing or reviewing an article, forming a Walk team and/or becoming a Medical Walk Ambassador, donating, and joining our Partners in MG Care program to facilitate patient referrals to your practice. You can also simply encourage your patients to enroll in the MG Patient Registry. The Registry, with more than 3,000 participants, has already produced significant finding to advance clinical trial research and advocacy.

If you would like to learn more about the Foundation and its programs, please contact us at 1-800-541-5454 or visit us at myasthenia.org, where you can find information about the MG Patient Registry, information for health professionals, patient resources, and the latest in research. Special thanks to our Session Chairs, Amanda Guidon, MD; Srikanth Muppidi, MD; and Araya Puwanant, MD, and all of our speakers and presenters. Special thanks to our Presenting Sponsors, Alexion Pharmaceuticals, argenx, Momenta Pharmaceuticals, Ra Pharmaceuticals and UCB. Special thanks to our Silver Sponsor, Catalyst Pharmaceuticals. We are grateful for their support!

We hope you find this scientific session to be informative.

Sincerely,

Nancy Law
CEO

Edward Walsh
Board Chair
2019 MGFA Scientific Session

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Department of Neurology  Assistant Professor, Neurology
Massachusetts General Hospital  University of Pittsburgh

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# 2019 MGFA Scientific Session

**AANEM Annual Meeting**  
Wednesday, October 16, 2019 8:00 am to 12:45 pm  
**JW Marriott, Lone Star Ballroom (F-H)**

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Small, G.  EVALUATION OF MEDICATIONS IMPLICATED IN PROMOTING MYASTHENIA GRAVIS (MG) EXACERBATION FACT OR FICTION
PLATFORM PRESENTATIONS
The Adverse Event Unit (AEU): A Novel Metric to Measure the Burden of Treatment Adverse Events

Michael Hehir (South Burlington, VT), Mark Conaway (Charlottesville, VA), Eric Clark (Burlington, VT), Denise Aronzon (Burlington, VT), Noah Kolb (Charlotte, VT), Amanda Kolb (Burlington, VT), Katherine Ruzhansky (Charleston, SC), Reza Sadjadi (Boston, MA). Eduardo De Sousa (Moore, OK), Ted Burns (Charlottesville, VA)

Introduction: There is increasing emphasis on treatment burden in neurology to better understand treatment comparative efficacy. We remain without a practical, easy to administer and interpret metric to measure adverse event (AE) burden that facilitates comparison of medications within and across different classes based on AE burden alone. AEs negatively impact patient quality of life and treatment adherence.

Objective: Design a physician and patient derived tool, the Adverse Event Unit (AEU), to improve AE burden measurement.

Methods: Online survey administered to internal medicine, neurology, and pediatric physicians to assign value to 73 AE categories chosen from the Common Terminology Criteria of Adverse Events (CTCAE) relevant to neurologic disorder treatments. Online forced choice survey of non-physician, potential patients, to weight the severity of the same AE categories. Physician and non-physician data combined to assign value to the AEU.

Results: 363 physicians rated the 73 AE categories derived from CTCAE. 660 non-physicians completed forced choice experiments comparing AEs. The newly created AEU provides 0 – 10, weighted values for the AE categories studied that differ from the ordinal 1-4 CTCAE scale. For example, CTCAE severe diabetes (category 4) is assigned an AEU score of 9. Although non-physician input changed physician assigned AEU values, there was general agreement among physicians and non-physicians about the severity of AEs.

Summary/ Conclusion: The AEU has great promise to be a useful, practical tool to add precision to the measurement of AE burden in the clinic and in comparative efficacy research. AEU utility will be assessed in planned clinical trials.
Survivin is a Negative Regulator of Apoptosis in Myasthenia Gravis: A Human and Animal Model Study

Linda Kusner (Washington, DC), Xiangyang Zhang (Washington, DC), Henry Kaminski (Washington, DC)

Introduction: Myasthenia gravis (MG) is caused by autoantibodies directed against the neuromuscular junction, with the majority of patients expressing antibodies to acetylcholine receptor (AChR). Autoreactive cells that produce the disease evade the immune checkpoints by an unknown mechanism. Survivin is a member of the inhibitor of apoptosis family and known to be expressed in circulating lymphocytes from MG patients. Survivin expression may be part of a mechanism that inhibits the apoptosis of autoreactive B cells in MG.

Objective: To assess the role of survivin in myasthenia gravis

Methods: The peripheral blood mononuclear cells were obtained from MG patients and non-autoimmune controls and stained with anti-human CD45, T cell marker (CD4), B cell marker (CD20), and anti-Survivin. The extracellular or intracellular survivin expression on human CD20+ or CD4+ lymphocytes were viewed by using BD Celesta analyzer followed by FlowJo software. To target survivin, a monoclonal antibody was developed against survivin peptide (SVN53-67/M57). For the animal model, EAMG was induced and mice stratified into three treatment groups (PBS, anti-Survivin 20 mcg and 100 mcg). EAMG mice were assessed for disease severity, AChR-specific antibody production, and expression of survivin in splenocyte population.

Results: Significantly higher percentage of CD4- CD20+ human B cells showed intracellular survivin expression in MG patients compared to controls. In the animal model of MG, antibody to survivin treatment improved disease severity, reduced AChR-specific antibody titers, and decreased survivin expression in CD3- CD19+ splenic B cells compared to PBS controls.

Summary/Conclusion: Targeting survivin–expressing B cells for elimination may be an effective therapeutic approach
Origins and Characteristics of Autoantibody-Producing B Cells in Myasthenia Gravis

Miriam Fichtner (New Haven, CT), Pablo Suarez (New Haven, CT), Panos Stathopoulos (New Haven, CT), Casey Vieni (New York, NY), Damian C. Ekiert (New York, NY), Gira Bhabha (New York, NY), Richard Nowak (New Haven, CT), Kevin O’Connor (New Haven, CT)

Introduction: Pathogenic autoantibodies that recognize muscle-specific tyrosine kinase (MuSK) are present in some patients with myasthenia gravis (MG). The B cells which produce these autoantibodies evade counterselection, which occurs when B cell tolerance checkpoints are not functioning properly. The recent isolation of the rare human B cells, which produce pathogenic autoantibodies, provides a novel opportunity to advance the understanding of MuSK-specific B cell development.

Objective: To gain insight into the details of MuSK MG B cell development.

Methods: We isolated MG patient-derived B cell populations that express MuSK autoantibodies. Human recombinant MuSK monoclonal autoantibodies (mAbs) were then produced from these cells as a means to further investigate both their characteristics and origin.

Results: The mAbs and monomeric antigen-binding fragments (Fabs) bound specifically to MuSK in a live cell-based assay, effectively interrupted agrin-induced clustering of the acetylcholine receptor and altered MuSK phosphorylation patterns. The Fabs bound to their antigen target with exceptionally high affinity. Further empirical evidence was acquired, which suggests that the B cells producing these autoantibodies emerge from a defectively governed developing B cell repertoire.

Summary/Conclusion: This study provides new details regarding the characteristics of human MuSK autoantibodies and how they relate to their pathogenic capacity. The data further provide a speculative mechanism for their development from an aberrantly formed naïve repertoire that materializes in the presence of B cell tolerance checkpoint defects.
In-Depth Immune Profiling of Treatment-Naïve Myasthenia Gravis Patients

Melissa Russo (Durham, NC), James Howard (Chapel Hill, NC), Doug Emmett (Durham, NC) Manisha Chopra (Chapel Hill, NC), Petra Duda (Cambridge, MA), Alonso Ricardo (Cambridge, MA), Simon Read (Cambridge, MA), John Yi (Durham, NC), Jeffrey Guptill (Durham, NC)

Introduction: Biomarkers in myasthenia gravis (MG) are limited and remain a critical unmet need in the field. Electrophysiological studies, such as single-fiber electromyography, are time-consuming, invasive, require specialized expertise, and have limited availability.

Objective: Our goal was to identify an immune signature that discriminated treatment-naïve MG patients from healthy controls and could serve as a baseline prior to immunotherapy. We performed comprehensive immune analysis using high-dimensional flow cytometry and multiplex cytokine assays.

Methods: Peripheral blood mononuclear cells from 24 treatment-naïve MG patients and 23 age- and sex-matched healthy controls were stained using a 28-color flow cytometry panel that identifies and phenotypes immune subsets. Circulating proteins were detected in the plasma using a Th17 Premixed 25-plex magnetic multiplex panel.

Results: CD4 and CD8 T cells in treatment-naïve MG patients exhibited an activated phenotype with increased frequencies of central memory and effector T cells, along with enhanced expression of activation markers ICOS and CTLA-4 for CD4 T cells and EOMES, Tbet, PD-1, and TIGIT for CD8 T cells. Compared to healthy subjects, MG patients had significant increases in peripheral GM-CSF, IL-10, IL-15 (all p<0.01), IFN-gamma, IL-1beta, IL-5, and IL-28A (all p<0.05). In contrast, Th2 related cytokines, including IL-13, IL-9, IL-4, IL-6, IL-25, IL-27, and TNF-beta were decreased in MG patients compared with healthy subjects.

Summary/ Conclusion: Immune profiling revealed a distinct immune signature in treatment-naïve MG patients as compared to healthy controls. Future analyses will focus on immune signature changes in response to immunotherapy to elucidate mechanisms of action and yield predictors of response.
Rituximab in Patients with Moderate to Severe Myasthenia Gravis: A Subgroup Analysis of the BeatMG Study

Richard Nowak (New Haven, CT), Christopher Coffey (Iowa City, IA), Jonathan Goldstein (New York, NY), Jon Yankey (Iowa City, IA), Liz Uribe (Iowa City, IA), Mazen Dimachkie (Kansas City, KS), Michael Benatar (Miami, FL), Gil Wolfe (Buffalo, NY), Ted Burns (Charlottesville, VA), Kevin O’Connor (New Haven, CT), Robin Conwit (Bethesda, MD), John Kissel (Columbus, OH), David Hafler (New Haven, CT), Merit Cudkowicz (Charlestown, MA), Richard Barohn (Kansas City, KS)

Introduction: The objective of the BeatMG study, a randomized, double-blind, placebo-controlled, phase 2 trial, was to determine whether rituximab was safe/beneficial for AChR antibody-positive generalized myasthenia gravis (MG). The primary outcome was a measure of steroid-sparing effect, defined as proportion of participants achieving ≥75% reduction in mean daily prednisone dose and with clinical improvement or no worsening. While rituximab was safe, the primary futility outcome was achieved in a predominately mild disease cohort.

Objective/Methods: In a post-hoc subgroup analysis, we explored the effect of rituximab in 20 patients (10 rituximab, 10 placebo) with moderate-severe disease (MGFA class III-IV) at baseline.

Results: The primary outcome was achieved by 60% vs 50% of participants in the rituximab and placebo groups, respectively. While primary endpoint success was the same in the rituximab group but lower in the placebo group than in BeatMG, this was not significant. The key secondary endpoints were change from baseline to week 52 in Quantitative MG (QMG) and MG Composite (MGC) scores. Mean change in QMG and MGC were -3.9 vs -0.5 and -7.0 vs -4.8 for rituximab and placebo groups, respectively.

Summary/Conclusion: These data suggest that we cannot exclude rituximab treatment effect on successful steroid taper and directionally favorable clinical improvement in moderate-severe AChR antibody-positive generalized MG. Caution is required as this is a post-hoc subgroup analysis without adequate power to make firm conclusions. Further study of the B-cell depletion response is critical for development of patient-tailored treatment paradigms. Additional data on further subgroup analyses will be presented.
PROMISE-MG: A Prospective Multicenter Observational Study of the Comparative Effectiveness of Treatments for Myasthenia Gravis: Preliminary Results

Pushpa Narayanaswami (Boston, MA), Donald Sanders (Durham, NC), Jeffrey Guptill (Durham, NC), Fan Li (Durham, North Carolina), Rishi Desai (Boston, MA), Jurgen Venitz (Richmond, VA), Kathleen Bibeau (Renton, WA), Andrew Krueger (Summerfield, NC), PROMISE-MG Study group (multicenter)

Introduction: PROMISE-MG is an ongoing, multicenter (US/Canada) prospective, real-world, observational comparative effectiveness study of treatments for MG.

Objective: To present baseline data of enrolled subjects.

Methods: All de-identified subject data are entered into a central REDCap database. We analyzed data from the initial study visit of enrolled subjects using descriptive statistics. We used Pearson correlation to measure the relationship between outcome measures.

Results: One hundred and sixty six patients are enrolled (75% of projected); mean age 65 (20-90) years, 61% male, 93% Caucasian. Average age at disease onset was 64±14 years (females 61± 17, males 66±11). Fifty-nine percent were generalized (females 69%, males 53%), MGFA class: 1 (40%), 2 (37%), 3 (21%) 4/5 (2%). Mean disease duration was 1.3± 2.5 years; 76% were AChR-Ab positive, 6% MuSK-Ab positive. Imaging revealed normal/involuted thymus in 87%, thymoma in 7%. At the initial visit, over half were on pyridostigmine and treatment was started/ changed in 74% (77% pyridostigmine, 12% corticosteroids). Mean outcome measure scores at baseline were: MGQOL15r 10.9±8.2, MG Composite (MGC) 8.6±6.3, MG-MMT 8.3±6.8, MG- ADL 5.5± 3.3. Correlations between outcome measures were moderate (0.54 to 0.68, MG-QOL15r vs. MGC, MG-MMT, MG-ADL; MG-ADL vs. MG-MMT) to strong (0.71 to 0.79, MGC vs. MG-ADL, MG-MMT), all p< 0.0001.

Summary/ Conclusion: The subjects in PROMISE-MG are predominantly male with disease onset in the 7th decade; 7% had thymoma. There was moderate correlation between the patient reported measures (MG-ADL, MGQOL15r); MGQOL15r correlated moderately with the other measures. The highest correlation was between MGC and MG-ADL.
Zilucoplan, a Self-Administered Subcutaneous Peptide Inhibitor of Complement Component 5 (C5) for the Treatment of Generalized Myasthenia Gravis: Phase 2 Results

James Howard (Chapel Hill, NC), Richard Nowak (New Haven, CT), Gil Wolfe (Buffalo, NY)
Michael Benatar (Miami, FL), Petra Duda (Cambridge, MA), James MacDougall (Cambridge, MA), Ramin Farzaneh-Far (Cambridge, MA), Henry Kaminski (Washington, DC)

Introduction: In anti-acetylcholine receptor positive (AChR-Ab+) generalized myasthenia gravis (gMG), autoantibodies activate the classical complement pathway and trigger complement-mediated damage to the neuromuscular junction. Zilucoplan inhibits the cleavage of C5, thereby preventing the formation of the terminal complement complex.

Objective: This randomized, double-blind, placebo-controlled Phase 2 study was conducted to evaluate the safety, tolerability, and efficacy of zilucoplan in AChR-Ab+ gMG patients with a Quantitative Myasthenia Gravis (QMG) score ≥12, regardless of prior treatment history.

Methods: The primary and key secondary endpoints were change in QMG and MG Activities of Daily Living (MG-ADL) scores from baseline to week 12. MG Quality of Life and MG Composite scores were also assessed.

Results: Forty-four patients were randomized 1:1:1 to placebo, zilucoplan 0.1 mg/kg, or zilucoplan 0.3 mg/kg self-administered subcutaneously daily over 12 weeks. Clinically meaningful and statistically significant reductions in mean QMG (6 points) and MG-ADL (3.4 points) were observed in the zilucoplan 0.3 mg/kg treatment group (placebo-corrected changes: -2.8; p=0.05 (QMG) and -2.3; p=0.04 (MG-ADL)). Rescue therapy (IVIg or plasma exchange) was required in 3/15 subjects in the placebo arm, 1/15 in the 0.1 mg/kg zilucoplan arm, and 0/14 in the 0.3 mg/kg zilucoplan arm. Zilucoplan was observed to have a favorable safety and tolerability profile, consistent with prior trials. Additional data on Phase 2, including the open-label long-term extension, and the Phase 3 design will be presented.

Summary/Conclusion: These results support the further evaluation of zilucoplan in a registrational Phase 3 trial and its potential therapeutic role in a broader spectrum of patients with gMG.
**Efgartigimod in Myasthenia Gravis: Update on Clinical Development and Phase 3 ADAPT Study**

Peter Ulrichs (Boston, MA), Antonio Guglietta (Ghent, Belgium) Jon Beauchamp (Boston, MA), Hans de Haard (Ghent, Belgium), Wim Parys (Ghent, Belgium)

**Introduction:** Myasthenia Gravis (MG) is mediated by pathogenic IgG autoantibodies causing receptor blockade, accelerated receptor degradation and complement activation. The neonatal Fc receptor (FcRn) recycles IgG, rescuing it from degradation, extending IgG autoantibody half-life. Blocking FcRn function, to reduce IgG autoantibody levels, is a logical potential therapeutic approach for MG. Efgartigimod is a human IgG1 antibody Fc-fragment, a natural ligand of FcRn, engineered for increased, pH-dependent, FcRn affinity. Efgartigimod outcompetes endogenous IgG binding, preventing recycling, increasing IgG degradation.

**Objective:** Present data from efgartigimod phase 1/2 studies.

**Methods:** Efgartigimod at doses ≥10mg/kg IV has been administered to >115 subjects in healthy volunteer and three autoimmune disease studies (prior to phase 3 MG study).

**Results:** Efgartigimod results in targeted reduction of all IgG subtypes without impacting levels of other immunoglobulin isotypes or albumin. It has been well tolerated, with no safety signals or increased risk of infection observed. In a Phase 2 MG study patients received a cycle of 4 weekly IV infusions of 10mg/kg efgartigimod, or placebo (n=24). Efgartigimod resulted in clinically meaningful and sustained improvements in symptoms, consistent across four MG scales. 75% of efgartigimod patients achieved ≥2-point reduction in MG-ADL for ≥6 consecutive weeks versus 25% for placebo (p=0.039). At end of study, 8 weeks after last dose, 6/12 efgartigimod patients maintained clinically meaningful improvement of MG-ADL score, the effect persisting beyond IgG reduction. Additional pharmacodynamic, safety and efficacy data will be presented.

**Summary/ Conclusion:** The global phase 3 ADAPT MG study is ongoing, recruiting 150 AChR, MuSK, LRP4-antibody positive and seronegative patients.
Long-Term Efficacy of Eculizumab in Refractory Generalized Myasthenia Gravis: Responder Analyses

James Howard (Chapel Hill, NC), Chafic Karam (Portland, OR), Marcus Yountz (Boston, MA), Fanny O’Brien (Boston, MA), Tahseen Mozaffar (Irvine, CA)

Introduction: The 6-month double-blind placebo-controlled REGAIN study (NCT01997229) and its open-label extension (OLE; NCT02301624) demonstrated the sustained effectiveness of the terminal complement inhibitor eculizumab in adult patients with anti-acetylcholine receptor antibody-positive refractory generalized myasthenia gravis (gMG).

Objective: To analyze response profiles in REGAIN and its OLE.

Methods: The analysis was conducted using Myasthenia Gravis–Activities of Daily Living (MG-ADL) and Quantitative Myasthenia Gravis (QMG) scores recorded during REGAIN and its OLE. Early/late responses were defined as improvement in MG-ADL score (≥3 points) or QMG score (≥5 points) occurring at ≤12/>12 weeks, respectively, after baseline (eculizumab initiation).

Results: The analysis included 98 patients. By Week 12 and OLE end, MG-ADL response had been achieved at some point by 67.3% and 84.7% of patients, respectively; QMG response by 56.1% and 71.4%, respectively. Response was observed over multiple consecutive assessments for the vast majority of patients. At Week 130, the least-squares mean (LSM) percentage changes from baseline in MG-ADL score were -61.9% and -47.5% in early and late MG-ADL responders, respectively; the LSM percentage changes from baseline in QMG score were -40.8% and -55.5% in early and late QMG responders, respectively (all p<0.0001). Significant baseline differences between early versus late QMG responders were seen for mean duration of MG (10.46 versus 5.46 years, respectively; p=0.0002) and mean QMG score (18.6 versus 15.1, respectively; p=0.0223).

Conclusion: The findings suggest that although most patients with refractory gMG will achieve clinical response (assessed by MG-ADL or QMG scores) by Week 12 of eculizumab treatment, responses can be observed with longer-term treatment.
DATA BLITZ PRESENTATIONS
Multicenter Study of LRP4 and Agrin Antibodies in Myasthenia Gravis (MG)

Michael Rivner (Augusta, GA), Brandy Quarles (Augusta, GA), Jin-Xiu Pan (Cleveland, Ohio), Zheng Yu (Cleveland, OH), James Howard (Chapel Hill, NC), Andrea Corse (Baltimore, MD), Mazen Dimachkie (Kansas City, KS), Carlayne Jackson (San Antonio, TX), Tuan Vu (Tampa, FL), George Small (Pittsburgh, Pennsylvania), Robert Lisak (Detroit, MI), Jerry Belsh (New Brunswick, New Jersey), Ikjae Lee (Birmingham, AL), Richard Nowak (New Haven, CT), Vanessa Baute (Winston Salem, NC), Stephen Scelsa (New York, NY), J. Americo Fernandes (Omaha, NE), Zachary Simmons (Hershey, PA), Andrea Swenson (Iowa City, IA), Richard Barohn (Kansas City, KS), R. Bhavaraju-Sanka (San Antonio, TX), Clifton Gooch (Tampa, FL), Eroboghene Ubogu (Birmingham, AL), James Caress (Winston Salem, NC), Lin Mei (Cleveland, OH), LRP4 Study Group (Augusta, GA)

Introduction: AChR and MuSK antibodies are found in 90% of generalized MG patients. In some double negative MG (DNMG) patients, LRP4 and Agrin antibodies were detected. These antibodies were demonstrated to produce experimental autoimmune MG (EAMG) in mice and have been postulated to cause MG in humans. However, little is known regarding the incidence and clinical features of these patients.

Objective: We studied the prevalence and clinical characteristics of LRP4 and Agrin antibody-positive DNMG.

Methods: DNMG patients at 16 U.S. sites were tested by ELISA for LRP4 and Agrin antibodies, and clinical data was collected. Study patients must have had an abnormal SFEMG or repetitive nerve stimulation study, have clinical symptoms of MG, and not had recent IVIG, rituximab or plasmapheresis.

Results: Of 182 DNMG patients, 12.6% (23) were positive for LRP4 antibodies and 13.7% (25) were positive for Agrin antibodies. Twenty-two of these patients (12.1% of the total) were positive for both LRP4 and Agrin antibodies. LRP4/Agrin antibody positive patients were predominantly female (62%) and had an average age at sample draw of 55 (standard deviation 13.2). Initially 50% (13) had ocular symptoms only. MGFA classification of patients’ worst symptoms were: I (11.5%), II (42.3%), III (30.8%), IV (11.5%) and V (3.9%). Most patients’ symptoms were controlled with standard therapies. With treatment, 92.3% of patients were classified as either Class I or II.

Summary/ Conclusion: Fourteen percent of DNMG patients had antibodies to either LRP4 or Agrin. Eighty-eight percent of patients developed generalized MG. Most patients with therapy attained minimal manifestation status or better.
Sensitivity of Neurophysiologic Tests Regarding the Neuromuscular Junction in Patients with Congenital Myasthenic Syndromes

Vitor Marques Caldas (Brasília, Brazil), Eduardo de Paula Estephan (São Paulo, Brazil), Andre Macedo Serafim da Silva (São Paulo, Brazil), Rodrigo de Holanda Mendonça (São Paulo, Brazil), Carlos Otto Heise (São Paulo, Brazil), Edmar Zanoteli (São Paulo, Brazil)

Introduction: Congenital Myasthenic Syndromes (CMS) are rare inherited disorders of the neuromuscular junction that oftentimes are misdiagnosed for many years. The combination of modern genetic tests and classic neurophysiological techniques are the best scenario for early diagnosis in this group of patients.

Objective: To study the physiology of the neuromuscular junction in patients with CMS with the combination of two neurophysiological techniques: low frequency repetitive stimulation (RS) and jitter analysis using disposable concentric needle electrodes (CNE).

Methods: We selected 18 subjects (mean age 30.1 years) with CMS. The genetic profile of the group was: CHRNE gene mutation was found in eleven patients; RAPSN, COLQ and DOK-7 gene mutation were present in two patients, each; and a new COL13A1 gene mutation was detected in one subject. In all patients, we performed low frequency repetitive stimulation (RS) in at least six different muscles and collected 20 apparent single fiber action potential pairs during minimal voluntary activation of orbicular oculi muscle (OOM) using disposable CNE.

Results: The combined neurophysiological techniques were positive in all patients, with at least one positive test in each of the 18 patients. 15 of them (83.3%) had both tests positive. RS was normal in only one RAPSN and one CHRNE patients (88.8% of sensitivity) and CNE Voluntary Jitter was normal in only one DOK-7 subject (94.4% of sensitivity).
Phenotypic Variation between Early Onset and Late Onset Myasthenia Gravis

Pragma Roy (Essex Junction, VT), Ava Bakhtyari (Burlington, VT), Noah Kolb (Charlotte, VT), Waqar Waheed (Burlington, VT), Rup Tandan (Burlington, VT), Michael Hehir (South Burlington, VT)

Introduction: Myasthenia gravis (MG) phenotype and pattern of progression may be related to age of onset. Differences in phenotype may influence treatment decisions and prognosis. Published series of MG phenotype in late onset MG (LOMG) patients are mixed with reports of both more and less severe phenotypes when compared to patients with early onset MG (EOMG).

Objective: Define the phenotypes of LOMG and EOMG in large cohort of MG patients.

Methods: Retrospective review of all MG patients seen at a tertiary neuromuscular center between 2003 to 2017. Patients with congenital and checkpoint inhibitor MG were excluded from analysis. The primary outcome is the worst median Myasthenia Gravis Foundation of America Clinical Classification (MGFA-CC) in the course of each patients disease course. The MGFA-CC will be compared between EOMG (age ≤ 59 years) and LOMG (age ≥ 60 years) patients. Secondary outcomes include: MGFA Post-intervention status (PIS) at final visit, MG Status and Treatment Intensity Score at final visit, antibody status, thymus pathology, number of myasthenia exacerbations, number and type of immunosuppressant treatments during illness course, and dose of immunosuppressants at time of last visit.

Results: Records from 551 MG patients (EOMG n = 182 and LOMG n = 369) will be reviewed for this study.

Summary/Conclusion: We will report the clinical phenotype of a cohort of MG patients that includes a large percentage of LOMG (67%) patients in an effort to better understand differences between EOMG and LOMG. We believe the final results will have both implications for prognosis and treatment decisions.
Myasthenia Gravis Patient and Physician Opinions about Immunosuppressant Dose Reduction

Michael Hehir (South Burlington, VT), Emma Ciafaloni (Rochester, NY), Anna Punga (Uppsala, Sweden)

Introduction: International MG Consensus Guidelines define successful MG treatments as those that result in minimal disease manifestations or remission with no more than minimal adverse events (AE). In an effort to reduce risk of immunosuppressant (IS) exposure AEs, e.g. opportunistic infections and malignancies 2-9 to reduce burden to patients and the health care system, the guidelines also recommend decreasing IS dose in MG patients with prolonged clinical stability. 1, 10. However, IS dose reduction could also increase patient risk for clinical relapse.

Objective: Define the phenotypes of LOMG and EOMG in large cohort of MG patients.

Methods: Retrospective review of all MG patients seen at a tertiary neuromuscular center between 2003 to 2017. Patients with congenital and checkpoint inhibitor MG were excluded from analysis. The primary outcome is the worst median Myasthenia Gravis Foundation of America Clinical Classification (MGFA-CC) in the course of each patients disease course. The MGFA-CC will be compared between EOMG (age ≤ 59 years) and LOMG (age ≥ 60 years) patients. Secondary outcomes include: MGFA Post-intervention status (PIS) at final visit, MG Status and Treatment Intensity Score at final visit, antibody status, thymus pathology, number of myasthenia exacerbations, number and type of immunosuppressant treatments during illness course, and dose of immunosuppressants at time of last visit.

Results: Records from 551 MG patients (EOMG n = 182 and LOMG n = 369) will be reviewed for this study.

Summary/Conclusion: We will report the clinical phenotype of a cohort of MG patients that includes a large percentage of LOMG (67%) patients in an effort to better understand differences between EOMG and LOMG. We believe the final results will have both implications for prognosis and treatment decisions.
Minimal Manifestations of Status and Prednisone Withdrawal in the MGTX Trial

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Introduction/Objective: To examine whether sustained minimal manifestation status (MMS) with complete withdrawal of prednisone is better achieved in thymectomized myasthenia gravis (MG) patients.

Methods: This study is a post hoc analysis of data from the randomized trial of thymectomy in myasthenia gravis (MGTX). MGTX was a multicenter, randomized, rater-blinded 3-year trial that was followed by a voluntary 2-year extension for patients with acetylcholine receptor (AChR) antibody positive MG without thymoma. Patients were randomized 1:1 to thymectomy plus prednisone versus prednisone alone. Participants were age 18-65 years at enrollment with disease duration less than 5 years. All patients received oral prednisone titrated up to 100mg on alternate-days until they achieved MMS, which prompted a standardized prednisone taper. The achievement rate of sustained MMS (no symptoms of MG for 6 months) with complete withdrawal of prednisone was compared between the thymectomy plus prednisone and prednisone alone groups.

Results: MG patients in the thymectomy plus prednisone group achieved sustained MMS with complete withdrawal of prednisone more frequently (64% vs 38%) and quickly compared to the prednisone alone group (median time 30 months vs not achieved, P<0.001) over the 5-year study period. Prednisone associated adverse symptoms were more frequent in the prednisone alone group and distress level increased with higher doses of prednisone.

Summary/Conclusions: Thymectomy benefits MG patients by increasing the likelihood of achieving sustained MMS with complete withdrawal of prednisone. This study provides Class II evidence that thymectomy plus prednisone is superior to prednisone alone in the treatment of AChR-antibody positive generalized MG patients without thymoma.
A Randomized, Placebo-Controlled, Parallel Group Study to Evaluate the Effect of Amifampridine Phosphate in Patients with MuSK Antibody Positive Myasthenia Gravis

Stanley Iyadurai (Coral Gables, FL), Christian Buettner (Greensboro, NC)

Introduction: Myasthenia gravis (MG) is a rare, debilitating, acquired autoimmune disease of the neuromuscular junction (NMJ). The main proteins affected are acetylcholine receptor (AChR) and muscle-specific receptor tyrosine kinase (MuSK). MuSK-MG is a disease characterized by a predominance in females, earlier onset than other AChR-MG, prominent bulbar involvement, more severe clinical condition, and significant resistance to treatment. Although many patients with MuSK-MG are treated with anticholinesterase inhibitors or immunosuppressants, they do not respond well to such treatments. Hence, MuSK-MG patients may continue to have marked generalized weakness and bulbar signs and symptoms of the disease. In these patients, the search for alternative treatment strategies targeting different pathophysiologic aspects of the disease is a medical need.

Objective: The purpose of this study is to evaluate the safety, tolerability, and efficacy of amifampridine phosphate in patients with MuSK-MG, and a sample of AChR-MG patients.

Methods: This randomized, double-blind, placebo-controlled, parallel group, outpatient study is planned to include approximately 60 MuSK-MG patients and 10 AChR-MG patients. The planned duration of participation for each patient is at least 38 days, excluding the screening period, which can last up to 14 days. After the open-label run-in the patient must show ≥2-point improvement in MG-ADL score to be randomized. The randomization period consists of 10 days of amifampridine or placebo.

Results: Primary endpoint efficacy of the MuSK-MG group will be analyzed as the change in MG-ADL from baseline (Day 0) using the Wilcoxon-Mann-Whitney Rank sum test.

Summary/Conclusion: This study is open with 29 patients enrolled at the time of submission.
POSTER PRESENTATIONS
Retrospective Longitudinal Assessment of MG-ADL Score with Treatment of Myasthenia Gravis

Matthew Varon (Kansas City, KS), Mamatha Pasnoor (Kansas City, KS), Tamara Winden (Kansas City, Kansas), Suzanne Hunt (Kansas City, Kansas), Omar Jawdat (Lenexa, KS), Constantine Farmakidis (Mission Hills, KS), Duaa Jabari (Overland Park, KS), Melanie Glenn (Mission, KS), Jeffrey Statland (Kansas City, KS), Richard Barohn (Kansas City, KS), Mazen Dimachkie (Kansas City, KS)

Introduction: The MG-ADL scale is a validated eight-item questionnaire which assesses the symptoms and correlates well with functional impairment from myasthenia gravis (MG). A two-point change in the MG-ADL is clinically meaningful, with higher scores indicating worse function. Patient reported outcome measures such as MG-ADL have been utilized frequently as an outcome measure in MG research. More recently these have been used in routine clinical care.

Objective: To assess the feasibility of retrospectively extracting the Myasthenia Gravis Activities of Daily Living (MG-ADL) score from the electronic medical record (EMR) for purposes of monitoring clinical status of populations of MG patients.

Methods: MG-ADL scores are routinely obtained and inserted in the documentation flowsheet of the EMR at clinic visits for MG patients in the KUMC neuromuscular clinics. At each clinic visit where MG-ADL scores were obtained we abstracted MG-ADL values, demographics, serology, and interventions received. Descriptive statistics were used to define baseline characteristics and analyze the data.

Results: Data abstraction yielded 845 MG-ADL scores for 334 patients. Data for 61 subjects in which the initial visit was identified was used for analysis. The median age at first encounter was 66 years and 34.4% were female. The median MG-ADL score at the first visit was 7. Median MG-ADL scores at 3, 6, 9, and 12 months were 6, 4, 6, and 4.

Summary/Conclusion: It is feasible to extract MG-ADL scores from our EMR. Median MG-ADL scores suggest that overall most cases of MG remain stable or improve over more than 1 year.
Thymectomy in Seropositive Myasthenia Gravis in a Quaternary Neuromuscular Division Before and After Publication of the MGTX Study

Constantine Farmakidis (Mission Hills, KS), Matthew Varon (Kansas City, KS), Suzanne Hunt (Kansas City, Kansas), Mamatha Pasnoor (Kansas City, KS), Omar Jawdat (Lenexa, KS), Gary Gronseth (Kansas City, KS), Richard Barohn (Kansas City, KS), Mazen Dimachkie (Kansas City, KS)

Introduction: The MGTX study demonstrated that thymectomy leads to reduced disease severity in seropositive (AChRab+) generalized myasthenia gravis (MG). Higher rates of, and earlier thymectomy, may improve outcomes in seropositive MG.

Objective: To determine if there has been increased emphasis in patient counseling and use of thymectomy in seropositive MG patients in our academic practice, as may be expected following publication of MGTX.

Methods: This is a single center retrospective study of generalized seropositive MG patients without thymectomy contraindications. The rates of documented thymectomy counseling, thoracic surgery referral, and thymectomy were compared in new patients seen in the 30-month epochs immediately before and after publication of MGTX in 2016.

Results: 400 adult patients were identified in the HERON database with first encounters including MG codes during both epochs. After excluding patients with contraindications for thymectomy, 41 patients remained. The rate of thymectomy in the before MGTX epoch was 7 per 24 (29.2%). The rate of thymectomy in the after epoch was 9 per 17 (52.9%). This equates to a risk difference of 23.7% (95% CI -5.9% to 49.1%). Rates of documented thymectomy counseling and surgical referral were similar between the two epochs.

Summary/Conclusion: The thymectomy rate was higher in the post MGTX epoch, but the difference did not reach statistical significance. An increase in the use of thymectomy in seropositive MG may or may not be taking place nationally. This is an important question as it could shape future recommendations for optimal care delivery for non-thymomatous MG at the population level.
Exercise Provocation of Stimulated Single Fiber Electromyography, an Attempt to Increase the Diagnostic Yield in Myasthenia Gravis Patients

AyatAllah Farouk Hussein (Cairo, Egypt)

Introduction: Single-fiber EMG provides the most useful test when repetitive nerve stimulation studies are normal in myasthenia gravis suspect patients. Stimulated SFEMG is a useful, easier technique as compared to volitional SFEMG. Here, the intramuscular twigs of the nerve are stimulated near the end plate zone. The jitter is calculated from the stimulus artifact and the single muscle fiber action potential. In an attempt to increase its diagnostic yield, it is done after exercise and detecting the post-exercise exhaustion in the form of increased jitter and neuromuscular blocking.

Objective: To estimate jitter values in myasthenia gravis patients pre- and post-exercise and to detect if any prolongation of the jitter or blocking occur

Methods: Twenty confirmed myasthenia gravis patients, 12 females, 8 males (aged 28.34±10.1 years-old), were studied. All diagnosed by VSFEMG with myasthenia gravis, SSFEMG was carried for the EDC muscle pre- and post-exercise, the parameters for evaluation were jitter expressed as the mean consecutive difference (MCD) and presence of blocking.

Results: SSFEMG prolonged jitter was found in 60% (12 patients) pre-exercise and increased to 80% (18 patients) post-exercise, also 5 patients had blocking pre-exercise, while 10 patients showed blocking post-exercise.

Summary/Conclusion: Exercise provocation for Stimulated SFEMG increased the diagnostic yield in myasthenia gravis diagnosis.
Co-Occurrence of Ocular Myasthenia Gravis and Chronic Inflammatory Demyelinating Polyradiculoneuropathy

Anirudha Rathnam (Detroit, MI), Ritika Suri (Detroit, MI), Naganand Sripathi (Detroit, MI), Kavita Grover (Detroit, MI)

Introduction: The concomitant association of myasthenia gravis (MG) and chronic inflammatory demyelinating polyradiculoneuropathy (CIDP) has been sporadically reported. We present a case of a middle-aged man with this rare association.

Objective: To report a case with co-occurrence of two auto-immune neuromuscular disorders.

Methods: Case report

Results: A 43-year-old man with history of diabetes mellitus, initially presented in 1989 with ocular symptoms of double vision and drooping of eyelids. He had no other reported weakness. His testing was positive for acetylcholine receptor antibodies. He was treated with pyridostigmine without relief, requiring initiation of prednisone and was maintained on 10-15 mg per day. Approximately 16 years later, he began having progressive foot drop on both sides and examination was consistent with absent reflexes and distal motor and sensory deficits. Nerve conductions and electromyography was consistent with acquired sensorimotor peripheral demyelinating polyneuropathy. Monoclonal protein evaluation showed IgG lambda monoclonal protein. He was started on Intravenous Immunoglobulin treatments with subsequent improvement in his CIDP and MG symptoms. In 2015, he had increased weight loss, developed anemia and renal failure and was diagnosed with IgG lambda multiple myeloma. He received chemotherapy with Bortezomib and dexamethasone with improvement in the myeloma, however, chemotherapy was stopped due to painful neuropathy.

Summary/ Conclusion: Myasthenia gravis and CIDP are different autoimmune disorders with distinct immune mechanisms. Given that this association is so rare, physicians need to be cognizant of this co-occurrence as it can influence management in these patients.
Lambert-Eaton Myasthenic Syndrome in the Setting of Immune Checkpoint Inhibitor Treatment of Small Cell Lung Cancer

Nadim Jiwa (Boston, MA), Mary Jane Lim-Fat (Boston, MA), Ugonma Chukwueke (Boston, MA), Jacob Sands (Boston, MA), Christopher Doughty (Boston, MA)

Introduction: Treatment with immune checkpoint inhibitors (ICIs) for cancer may result in neuromuscular immune related adverse events (irAEs). To our knowledge, Lambert-Eaton Myasthenic Syndrome (LEMS) in patients treated with ICIs has been seldom described.

Objective: To report the clinical course of LEMS in the context of treatment with nivolumab.

Case: A 77-year-old man with small cell lung cancer (SCLC) treated with partial lobectomy followed by carboplatin and etoposide noticed progressive leg weakness after completion of chemotherapy. This was associated with new xerostomia. Weakness plateaued after 2 months, at which point he was ambulating independently, so no work-up was performed at that time. He was subsequently found to have metastatic disease and started nivolumab. Within 4 weeks of his first infusion, he developed progressive proximal>distal lower extremity weakness and required a walker. Examination after his fourth infusion demonstrated proximal and distal weakness in his lower extremities, distal sensory loss, and facilitation of reflexes. EDX showed evidence of both an axonal polyneuropathy and a presynaptic disorder of neuromuscular transmission. Antibodies to P/Q-type Voltage-Gated Calcium Channel (VGCC) returned positive, confirming the diagnosis of LEMS. Nivolumab was stopped and pyridostigmine 30mg TID was started. Within 4 weeks, his strength improved, he resumed independent ambulation, and pyridostigmine was stopped.

Summary/ Conclusion: LEMS, a known paraneoplastic consequence of SCLC, may worsen with ICI treatment. Our patient achieved meaningful improvement simply by holding the ICI and starting pyridostigmine. This suggests that patients with mild symptoms may not need immunomodulatory therapy such as corticosteroids and may help guide management of similar patients.
Congenital Myasthenic Syndromes: A Clinical, Electrophysiological, and Genetic Review

Joshua Kaltman (Decatur, GA) Sumit Verma (Johns Creek, GA)

Introduction: The congenital myasthenic syndromes (CMS) are a group of heterogeneous genetic disorders. Clinical, electrophysiological, and genetic studies on pre-synaptic, synaptic, and post-synaptic subtypes of CMS are limited.

Objective: To study a clinical, electrophysiological, and genetic profile of CMS.

Methods: Retrospective study of genetically confirmed CMS patients (CHRNE n=5, CHAT deficiency, n=4, PLEC n=3, Dok7 n=1) at a tertiary care Children’s hospital from 2013 to 2019. Demographic data, signs, symptoms, electrophysiological data, and treatment strategy were recorded. Descriptive statistics were used.

Results: Thirteen children (7 girls) mean age 7.6±5.5 years (range 1.5-21 years) with symptom onset at mean age 10.5±11.8 months (range 0-36 months). Genetic confirmation mean age 33.3±37.3 months (range 3 months-11.5 years) with 69% (n=9) post- and 31% (n=4) pre-synaptic defects. Mean delay of diagnosis 22.7±30.0 months (range 1 month-8.5 years). All children (n=13) had fatigue. Ninety two percent (n=12) had proximal muscle weakness, ptosis, and feeding difficulty. Seventy seven percent (n=10) had cognitive/behavioral problems and 70% (n=9) had apnea episodes. Thirty percent (n=4) underwent gastrostomy and 23% (n=3) had tracheostomy. Orbicularis oculi stimulated jitter analysis in twelve children showed mean mean jitter 50.4±19.2 µs (range 25-86 µs, normal ≤26 µs) and 30.3±26.2 % blocking (range 0-77%). All patients received pyridostigmine, 54% (n=7) 3,4-Diaminopyridine (3,4-DAP), 38% (n=5) liquid albuterol, and 8% (n=1) ephedrine.

Summary/ Conclusion: CMS is rare with no sex predilection. Delay in diagnosis was common. CHRNE and CHAT deficiency were the two most common genetic defects in our cohort. Majority of subjects required polypharmacy, with pyridostigmine and 3,4-DAP most commonly used.
Natural Course and Treatment of ACHR-MG Converted to MuSK-MG or DP-MG in Children: 2 Case Studies and Literature Review

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Introduction: Anti-muscle-specific tyrosine kinase antibody (MuSK-Ab) is the second most common autoantibody in myasthenia gravis. MuSK-Ab and anti-acetylcholine receptor antibody (AChR-Ab) can coexist in a few patients.

Objective: To explore the clinical features and possible mechanism of conversion of AChR-MG to MuSK-MG or DP-MG.

Methods: We report two children MG patients with AChR-Ab (ACHR-MG) who converted to double antibody positive MG (DP-MG) or MuSK-Ab positive MG (MuSK-MG). We also conducted a literature review to find similar cases.

Results: We found six similar cases through literature searches via online databases. Including our two patients, there were a total of eight patients in this study. Six female and two male. The average age of onset was 7.25±5.95 years, and the median age of onset was 5.5 years. Four AChR-MG patients converted to DP-MG in their natural course of disease, and one of them converted to MuSK-MG after converting to DP-MG; two AChR-MG patients converted to MuSK-MG after thymectomy. Two AChR-MG patients converted to DP-MG after thymectomy.

Summary/ Conclusion: AChR-MG can convert to MuSK-MG or DP-MG under certain conditions. We speculate that this conversion is the two courses of MG. These two courses may occur in succession or overlap. Female AChR-MG patients are prone to conversion. After appearing with MuSK-Ab, they show the characteristics of MuSK-MG. These patients respond poorly to cholinesterase inhibitors and well to corticosteroids. During myasthenic crisis, plasma exchange can bring satisfactory effects.
Hand Myasthenia in a Patient with Positive Acetylcholine Receptor Antibodies

Ricardo Maselli (Davis, CA), Victor Saunders (Sacramento, CA), Ge Xiong (Davis, CA), David Richman (Sacramento, CA)

Introduction: A small percentage of patients with a confirmed diagnosis of myasthenia gravis (MG) have distal extremity weakness exceeding proximal weakness. However, unilateral distal weakness as a sole manifestation of MG is very rare.

Objective: To describe a patient with mild ocular and generalized seropositive MG, who developed an unusual focal recurrence of MG involving only flexor muscles of the right hand.

Methods: Clinical and electrodiagnostic evaluation.

Results: A 56-year old man, who achieved a complete remission of MG after thymectomy and treatment with prednisone at high dose followed by slow tapering, developed weakness and fatigability of intrinsic muscles of the right hand three years after the original treatment. Symptoms were initially attributed to a carpal tunnel syndrome. However, an EMG study showed that the motor distal latency in the right median nerve was not prolonged and repetitive stimulation of the median nerve at 2 Hz was abnormal indicating failure of neuromuscular transmission. The recording from the abductor pollicis brevis muscle showed a decrement of compound muscle action potential amplitudes of 16% during the period of post-exercise exhaustion. Repetitive stimulation of the right spinal accessory nerve was normal.

Summary/Conclusion: Unilateral hand weakness can present several years after the onset of MG in spite of a successful treatment of the presenting symptoms. It is unclear whether this constitutes an exacerbation of MG or represents a separate manifestation of the original disease with a different pathogenic mechanism.
LRP4 and Agrin Antibodies in Myasthenia Gravis (MG): Augusta University (AU) Experience

Michael Rivner (Augusta, GA), Brandy Quarles (Augusta, GA), Jin-Xiu Pan (Cleveland, OH), Zheng Yu (Cleveland, OH), Kristy Bouchard (Augusta, GA), Lin Mei (Cleveland, OH)

Introduction: Ten percent of MG patients are ACHR and MuSK antibody-negative (DNMG). LRP4/Agrin autoantibodies occur in DNMG patients and are pathogenic in animals. We present data on MG patients from AU who are LRP4 or Agrin antibody-positive.

Objective: We describe the clinical characteristics of LRP4/Agrin positive MG patients.

Methods: MG patients seen from February 2014 through February 2019 were tested by ELISA for LRP4 and Agrin autoantibodies. All patient had symptoms of MG with either abnormal repetitive nerve stimulation or single fiber EMG. Most subjects were double negative, but some had other antibodies.

Results: 61 MG patients were tested for LRP4 and 52 for Agrin antibodies. Fifteen were ACHR positive and 7 MuSK positive. No subjects were positive for both ACHR and MuSK. Six subjects (9.8%) were LRP4 positive and 5 (9.6%) Agrin positive. Four (7.5%) were positive for both. One was LRP4 and ACHR positive, 1 was Agrin and ACHR positive, 1 was LRP4 and MuSK positive and none were Agrin and MuSK positive. Two patients required ventilatory support, one of whom was Agrin/ACHR positive the other LRP4/Agrin positive. A third LRP4/Agrin positive patient had significant swallowing symptoms requiring hospitalization. 43 additional subjects were tested for LRP4 and 38 for Agrin. In our total group of 103 subjects, 13 (12.6%) were LRP4 antibody-positive while 8 of 89 tested (9.0%) were Agrin antibody-positive.

Summary/Conclusion: Approximately 10% of tested MG patients had LRP4/Agrin antibodies. These patients’ symptoms were Class III or greater at their worst. Most responded to standard treatments. Several representative cases will be presented.
Evaluation of Medications Implicated in Promoting Myasthenia Gravis (MG) Exacerbation Fact or Fiction

George Small (Pittsburgh, PA), Mohammad Ali (Wexford, PA), Carol Schramke (Pittsburgh, USA)

Introduction: The Myasthenia Gravis Foundation of America (MGFA) lists multiple medications as relatively contraindicated (RCMS) in MG patients. Evidence of increased exacerbation risk associated with using these medications may be unclear. If only questionably associated with exacerbation, treatment delay with such therapies may promote unnecessary morbidity and mortality.

Objective/Methods: 100 clinical records of electrically or serologically confirmed MG patients over a 3 year period were reviewed. We defined an exacerbation as MGFA stage worsening by 2 grades, defined in patients who did and did not report receiving RCMS. Chi Square tests were utilized to examine increased exacerbation risk in patients treated with any RCM, particularly beta blockers (BBs) and certain antibiotics.

Results/Conclusions: 67 patients reported taking a RCM. 25 (37%) had exacerbations, compared to 15/33 (46%) who did not take a RCM. Of the 36 on BBs, 16 (44%) suffered an exacerbation, compared to 24/64 (38%) not taking BBs. Of 14 patients on contraindicated antibiotics, 3 (21%) suffered an exacerbation, compared to 37/86 experiencing an exacerbation not taking contraindicated antibiotics. None of the differences were statistically significant. Human case reporting and animal studies have resulted in recommendations for caution in exposing MG patients to medications thought to promote MG exacerbation. Unfortunately, withholding useful medications for other conditions in MG patients may have severe negative consequences. Our data suggest the risk of exacerbation may not be higher when using these medications.
mgregistry.org

The Myasthenia Gravis Patient Registry is an active database of persons with myasthenia gravis (MG), developed for the purposes of research, treatment, and patient information. The Registry is managed by the Coordinating Center of the University of Alabama at Birmingham (UAB) with oversight by the MGFA Patient Registry Committee.

The MG Patient Registry is:

- For myasthenia gravis research
- Participant-driven
- Free to enroll
- Confidential
- Open to Adults 18+ in the U.S.

What types of questions are in the enrollment survey?

- General contact information
- Demographic information, for example, education, employment status, income, insurance
- Year, month, and place of birth of parents and grandparents
- Information on places where you lived when you were under 25 years old
- MG medical history; including tests and diagnoses, treatments, other conditions, family’s MG history
- Information on quality of life and lifestyle
- Future, follow-up surveys will contain a subset of the enrollment questions as well as one new section of questions

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Q: Want Better Doctor-Patient Communication?

A: Encourage your patients to use the MGFA myMG app.

The Myasthenia Gravis Foundation of America, Inc. (MGFA) has created “myMG,” an innovative mobile application (or “app”) that enables people with Myasthenia Gravis (MG) to track their MG. “myMG” is a software application designed to run on computers, smartphones, tablets and other mobile devices for daily use.

When patients use the “myMG” app to record MG symptoms and other information, it empowers them to have a more interactive dialogue with their physicians. Symptoms, and their impact on daily life, can be recorded on the “Survey Tab”. Patients can log any notes that they believe may explain changes in symptoms (change in meds, forgotten meds, strenuous exercise).

Patients can also view cumulative survey results on the “Charts Tab” to monitor symptoms over time. Patients can also print the “Charts Tab” to share with their doctor during their appointment.
PARTNERS IN MG CARE PROGRAM

Partners in MG Care is a program that recognizes and supports quality MG care.

The Partners in MG Care program is designed to:
- Create a network of quality MG healthcare providers for patients
- Strengthen healthcare provider connections through information, services and support for MG patients and caregivers
- Empower MG patients with a robust resource of healthcare providers and support
- Enhance knowledge-sharing, professional education and networking opportunities about MG for healthcare providers

Partners in MG Care Approach:
- Partners in MG Care have: interest in treating MG patients, experience treating MG, involvement in MG community, ability to refer patients to MGFA as well as promote and support MGFA activities.
- A Partner in MG Care receives a recognition certificate and is publicly acknowledged on the MGFA website. Information about their practice will be shared with local MG patients seeking referrals. MGFA will provide literature, information about educational opportunities, research and advocacy updates, clinical trial notifications, and other important information to participants of the program. Partners are also invited to be a part of the MG Clinical Network.

How to Get Started:
- The Partners in MG Care program launched in fall 2017. Current M/SAB members who are U.S.-based and seeking patients will be invited to become a Partner in MG Care and asked to complete an agreement and information form.

To learn more about how to become a Partner in MG Care, please contact us at: MGFA@myasthenia.org
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