AAN Conference 2024 - Summary of MG-Focused Sessions

Session 1: NMJ Disorders: MG, Ocular, and MUSK Myasthenia

- **Date:** April 16
- **Presenters:** Srikanth Muppidi, MD, FAAN & Neelam Goyal, MD
- **Key Points:**
  - RINOMAX trial showed a steroid sparing effect in early-stage patients.
  - Hope expressed for positive outcomes from the MINT trial.
  - Ocular MG lacks approved therapies due to exclusion from trials based on MGFA scores.
  - MUSK MG identified in 2001; B-cell depleting therapies found effective in 2017.
  - MUSK MG patients respond well to rituximab, often requiring three cycles.
  - MG-ADL is the preferred scale in the US for patient self-reporting.
  - Complement inhibitors like eculizumab show better outcomes when started early.
  - Challenges include vaccination and antibiotic prophylaxis when using complement inhibitors.
  - FcRn receptor inhibitors, like efgartigimod, approved in 2021 showing favorable long-term tolerability.

Session 2: The Role of B Cells in gMG Pathogenesis

- **Date:** April 13, 2024
- **Presenters:** Kevin O'Connor, Pushpa Narayanaswami, MBBS, MD, FAAN, Richard J. Nowak, MD
- **Key Points:**
  - Discussion on CD20 vs. CD19 expression affecting efficacy of B-cell depletion in AChR+ vs. MuSK+ gMG.
  - BEAT-MG study overview showed no significant differences in steroid sparing or clinical outcomes.
  - RINOMAX study targeted new onset AChR+ gMG patients, showing primary outcomes with less steroid reduction needed.
  - Effective CD20 therapy in MuSK+ gMG presented as best data.
  - Role of thymectomy debated; less beneficial in MuSK+ MG.
  - Insights into long-term remissions in MuSK MG patients who responded to CD20 depletion.

Session 3: Clinically Meaningful Improvement in Physical Fatigue and Muscle Weakness Fatigability with Rozanolixizumab

- **Date:** April 15, 2024
- **Background:** MycarinG study Phase 3 trial investigated rozanolixizumab.
- **Objective:** Post-hoc analysis of MG Symptoms PRO scales in patients treated with rozanolixizumab.
- **Results:**
  - Significant percentage of patients treated showed clinically meaningful improvements at Day 43.
  - Rozanolixizumab-treated patients showed greater improvements in muscle weakness fatigability and physical fatigue compared to placebo.

Session 4: Cost-effectiveness Analysis of Efgartigimod Versus Chronic Intravenous Immunoglobulin (IVIg)

- **Date:** April 15, 2024
- **Objective:** Evaluate the cost-effectiveness of efgartigimod compared to IVIg in Canada.
- **Results:**
  - Efgartigimod showed an increase in quality-adjusted life years (QALYs) and lower total costs compared to IVIg.
  - Indicated dominance of efgartigimod over IVIg with cost savings.

Session 5: Eculizumab Versus Rituximab for Acetylcholine Receptor-positive Generalized Myasthenia Gravis

- **Date:** April 15, 2024
- **Objective:** Compare treatment efficacy and safety outcomes between eculizumab and rituximab.
- **Results:**
  - Eculizumab led to significant improvement in MGADL scores and greater steroid-sparing effect.
  - Patients previously on rituximab without adequate symptom control improved significantly after switching to eculizumab.

Session 6: Comorbidity Burden and Steroid Use in Generalized Myasthenia Gravis

- **Date:** April 15, 2024
- **Objective:** Analyze the burden of comorbid conditions and patterns of steroid treatment among gMG patients under Medicare.
- **Results:**
  - High comorbidity load among gMG patients, with significant long-term steroid use.
  - Highlighted the need for careful consideration when prescribing treatments to minimize worsening of comorbid conditions.

Session 7: Incidence and Prevalence of Myasthenia Gravis

- **Date:** April 15, 2024
- **Objective:** Quantify the prevalence and incidence rates of MG in the US.
- **Results:**
  - Provided robust evidence of the prevalence and incidence of MG, supporting ongoing research in this medical field.

Session 8: Baseline Characteristics in LUMINESCE Study

- **Date:** April 17, 2024
- **Objective:** Describe baseline characteristics of patients enrolled in a Phase 3 study of satralizumab for gMG.
- **Results:**
  - Majority of participants were female with a broad representation of gMG population.
  - Study will evaluate the efficacy, safety, and PK/PD profiles of satralizumab in gMG.

Session 9: Long-term Safety, Tolerability, and Efficacy of Subcutaneous Efgartigimod PH20

- **Date:** April 17, 2024
- **Objective:** Evaluate long-term safety, tolerability, and efficacy of subcutaneous efgartigimod PH20 in gMG.
- **Results:**
  - Well tolerated and efficacious, consistent with intravenous efgartigimod in previous studies.
  - Improvements in MG-ADL and MG-QoL scores across multiple cycles.

Session 10: Drivers of New Rozanolixizumab Treatment Cycles

- **Date:** April 17, 2024
- **Objective:** Understand factors leading to initiation of new rozanolixizumab treatment cycles in gMG.
- **Results:**
  - Initiation of new cycles generally driven by worsening in MG-ADL and/or QMG scores.
  - Physicians personalized treatment based on individual patient needs.

Session 11: Eculizumab and Ravulizumab Treatment Timing

- **Date:** April 15, 2024
- **Objective:** Assess MG-ADL total score changes from baseline to week 26 in patients initiated on eculizumab before or after 2 years of gMG diagnosis.
- **Results:**
  - Significantly greater improvements for early compared with late eculizumab initiators.
  - Retrospective data analysis supports better outcomes for early treatment initiation.

Session 12: Immunopathogenesis of AChR Autoantibody-positive MG

- **Date:** April 15, 2024
- **Objective:** Examine differences in immunopathogenesis between early onset and late-onset MG.
- **Results:**
  - Distinct immunopathogenic mechanisms suggested for late-onset MG.
  - Identification of differentially expressed proteins could help tailor therapeutic approaches.

Session 13: Long-term Safety and Efficacy of Zilucoplan

- **Date:** April 15, 2024
- **Objective:** Evaluate long-term safety and efficacy of zilucoplan up to 96 weeks in gMG.
- **Results:**
  - Favorable long-term safety profile demonstrated.
  - Efficacy sustained through to Week 96 in patients previously receiving zilucoplan.

Session 14: Evaluation of DNTH103 in Preclinical MG Model

- **Date:** April 15, 2024
- **Objective:** Describe the evaluation of DNTH103, a monoclonal antibody targeting the classical pathway, in a preclinical MG model.
- **Results:**
  - DNTH103 improved neurotransmission impairment in the model, supporting its potential use in MG.

Session 15: Shared Decision-Making in gMG Care

- **Date:** April 15, 2024
- **Objective:** Discuss the importance of shared decision-making in the era of new gMG targeted therapies.
- **Key Points:**
  - Exploration of data and real-world cases to balance individual patient needs and treatment options.
  - Importance of optionality in gMG care highlighted.

Session 16: COVID-19 and MG Severity

- **Date:** April 14, 2024
- **Objective:** Investigate if MG severity predicts the outcome of COVID-19 infection.
- **Results:**
  - No significant association found between MG severity and COVID-19 severity.

Session 17: Post-hoc Analysis of BeatMG and RINOMAX Trials

- **Date:** April 15, 2024
- **Objective:** Perform a post-hoc analysis of the BeatMG study with RINOMAX trial criteria.
- **Results:**
  - Analysis suggested a beneficial effect of rituximab in a subgroup of patients with AChR-Ab+ MG.
  - Highlighted the impact of clinical trial design on study outcomes.

Session 18: Validation of a Quantitative Assay for MuSK Antibodies

- **Date:** April 15, 2024
- **Objective:** Validate a quantitative assay for measuring MuSK antibodies in seronegative MG.
- **Results:**
  - Demonstrated a simple and sensitive assay for detecting MuSK antibodies.
  - Provided data on MuSK antibody positivity rates and titers.

Session 19: Chronic Weekly Rozanolixizumab in gMG

- **Date:** April 15, 2024
- **Objective:** Evaluate the safety, tolerability, and efficacy of chronic weekly rozanolixizumab in gMG.
- **Results:**
  - Well tolerated with a safety profile similar to repeated cycles of rozanolixizumab treatment.
  - Clinically relevant improvements maintained across MG-specific outcomes.

Session 20: Real-world Reduction in Oral Corticosteroid Utilization Following Efgartigimod Initiation

- **Date:** April 17, 2024
- **Objective:** Evaluate real-world utilization of oral corticosteroids among gMG patients post-efgartigimod initiation.
- **Results:**
  - Substantial proportion of patients experienced reduction in corticosteroid usage over the first 6 months post-efgartigimod initiation.

Session 21: Myasthenic Crisis, Exacerbations, and Hospitalizations in gMG Treated with Eculizumab

- **Date:** April 17, 2024
- **Objective:** Assess rates of myasthenic crisis, exacerbations, and hospitalizations among gMG patients treated with eculizumab.
- **Results:**
  - Decreased rates of myasthenic crisis, exacerbations, and hospitalizations observed following eculizumab initiation.

Session 22: Steroid Use and Outcomes in gMG Patients Receiving C5 Inhibitor Therapy

- **Date:** April 17, 2024
- **Objective:** Assess disease outcomes and changes in corticosteroid use in gMG patients receiving C5 inhibitor therapy.
- **Results:**
  - Statistically significant reductions in prednisone average daily dose and gMG exacerbations and crises observed.

Session 23: Eligibility of AchR+ gMG Patients for Phase III Trials

- **Date:** April 17, 2024
- **Objective:** Analyze the eligibility of AchR+ gMG patients treated in routine care for phase III clinical trials.
- **Results:**
  - Majority of patients treated in routine care would not have met clinical trials criteria.
  - Broader inclusion criteria suggested to increase patient eligibility and generalizability of trial results.

Session 24: Achievement of Minimal Symptom Expression in gMG Treated with Efgartigimod

- **Date:** April 17, 2024
- **Objective:** Assess incidence, characteristics, and changes in gMG-specific scales in participants achieving minimal symptom expression with efgartigimod.
- **Results:**
  - Substantial improvement across multiple disease measures and quality of life comparable to healthy populations observed in participants achieving MSE.

Session 25: Transitioning to Ravulizumab from Eculizumab in gMG

- **Date:** April 17, 2024
- **Objective:** Assess the effectiveness and safety of transitioning gMG patients from eculizumab to ravulizumab.
- **Results:**
  - Transitioning was safe and effective, with stable MG-ADL total scores and MGFA classifications observed.

Session 26: "Wearing Off" Phenomenon in gMG Patients Treated with Ravulizumab

- **Date:** April 17, 2024
- **Objective:** Describe a "wearing off" phenomenon between standard maintenance infusions of ravulizumab in gMG.
- **Results:**
  - One-third of the patients reported wearing off prior to their next scheduled dose.
  - No specific factors identified to explain this fluctuation.