

# May 13 – 15, 2025 Postillion Convention Center The Hague, The Netherlands

The MGFA International Conference is curated as a three-day journey to the future of MG. Each day features a renowned plenary keynote speaker and a variety of sessions, talks, panels, roundtable discussions, and interactive debates.

The first day will encompass a nod to the "past," with a fresh lens on the historical knowledge and progress that has been made in MG treatment and care; an interactive panel with several patients; and a deep dive into special populations within the MG community.

This leads into our second day, an exploration of the current landscape and a direct confrontation of its shortcomings through lively debates, a full session on congenital myasthenic syndromes, and parallel sessions to dive deeply into basic and clinical topics.

The event culminates with a bold invitation to envision and take aim toward a future "World Without MG" through instigative debates, lively discussion, and presentations of the most promising work from the premier thought and change leaders around the globe.

More information about the conference, including registration, is available at <u>myasthenia.org/internationalconference</u>.

CONFERENCE DAY 1 Tuesday, May 13 8:00am - 5:00pm

## SESSION 1 (8:00 am - 10:10am) Overview of Autoimmunity

#### **Session Chairs:**

- Rozen Le Panse, PhD, Pierre and Marie Curie University; France
- Pilar Martinez, PhD, Maastricht University; The Netherlands

#### **Welcome and Opening Remarks**

- Samantha Masterson, President and CEO, MGFA

#### **Keynote: An overview of autoimmunity**

- Sonia Berrih-Aknin, PhD, INSERM/Sorbonne University; France

#### Microbiota and the antibody/microbiota interface

- Delphine Sterlin, PharmD, PhD, Sorbonne University; France

#### B cells orchestrate tolerance to the neuromyelitis optica autoantigen AQP44

- Thomas Korn, MD, Technical University of Munich School of Medicine and Health, Munich; Germany

# Differences in IgG autoantibody Fab glycosylation across autoimmune diseases

- Theo Rispens, PhD, Sanguin; The Netherlands

# Thymic cell populations in establishment of self-tolerance

-Marte Heimli, PhD, Oslo University Hospital; Norway

BREAK (10:10 am-10:45am) Explore Posters, Exhibit Hall, and Experience Rooms

SESSION 2 (10:45am-12:15pm)
The Social Impact of MG with Guest Patient Panel

#### The social impacts of MG

 Dong Dong, PhD, The Chinese University of Hong Kong Centre for Bioethics; Hong Kong

## Patient panel

- Facilitated by Dong Dong, PhD, The Chinese University of Hong Kong Centre for Bioethics; Hong Kong and Carolina Barnett-Tapia, MD, PhD, University Health Network - Toronto General Hospital; Canada

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- Lutgarde Allard, Patient Advocate, ABMM/EuMGA; Belgium
- Juliana Magnusdottir, Patient Advocate, MG-Fèlag Islands; Iceland
- Liselotte Schirakow, Patient Advocate, MG Group of Muskelvindfonden; Denmark
- Maya Uccheddu, Patient Advocate, Associazione Italiana Miastenia AIM; Italy

#### ON-SITE LUNCH (12:15pm-1:30pm) Lunch provided

## SESSION 3 (1:30pm-3:00pm) Etiology of MG

#### Session Chairs:

- Rozen Le Panse, PhD, Pierre and Marie Curie University; France
- Pilar Martinez, PhD, Maastricht University; The Netherlands

# Identification of genetic risk loci and prioritization of genes and pathways for myasthenia gravis: a genome-wide association study

- Bryan Traynor, MD, PhD, National Institute on Aging; USA In honor of the late Dan Drachman, the co-principal investigator on myasthenia gravis work

#### Myasthenia gravis-specific aberrant neuromuscular gene expression by medullary thymic epithelial cells in thymoma

- Yoshiaki Yasumizu, MD, PhD, Yale University School of Medicine; USA

# IL17/Th17/thymoma

- Güher Saruhan Direskeneli, PhD, Istanbul University; Turkey

#### **Subclinical MG in thymomas**

- Florit Marcusse, PhD, Maastricht University Medical Center (MUMC); The Netherlands

# Perturbations in circulatory NK cells suggest their pathogenic activity in AChR-IgG+ MG

-Soumya Yandamuri, MSE, PhD, Yale University; USA

# Single-cell transcriptomics identifies a prominent role for the MIF-CD74 axis in myasthenia gravis thymus

-Paula Terroba Navajas, Doctoral Student, University Hospital Münster, Germany

#### BREAK (3:00-3:30pm) Explore Posters, Exhibit Hall, and Experience Rooms

#### SESSION 4 (3:30-5:00pm) Special Populations

#### **Session Chairs:**

- Valentina Damato, MD, University of Florence; Italy
- Ali Habib, MD, University of California, Irvine; USA

#### **Ocular MG**

- Jeanine Heckmann, MB, ChB, PhD, University of Cape Town; South Africa

#### Women and pregnancy-related issues in MG

- Amelia Evoli, MD, The University of Cattolica del Sacro Cuore; Italy

#### Neonatal and juvenile MG

- Sithara Ramdas, MD, Oxford Children's Hospital; England

#### **General overview of LEMS**

- Jan Verschuuren, MD, PhD, Leiden University Medical Center; The Netherlands

#### WELCOME RECEPTION AND POSTER SESSION 1 (5:00-7:00pm)

Hors d'oeuvres and cocktails provided

# CONFERENCE DAY 2 Wednesday, May 14 (8:00am- 5:00pm)

#### **WELCOME**

### MGFA Lifetime Achievement Award (8:00am- 8:15 am) Recipient Announcement and Talk

The MGFA Lifetime Achievement Award recognizes a distinguished scientist, scholar, or clinician who has made major contributions in the field of myasthenia gravis. Individuals considered for this award will have spent 25+ years of their career dedicated to the area of neurology, immunology, or genetics, leading to impactful advancement.

SESSION 5 (8:15 am-10:15 am)

#### **Controversies in MG**

#### Session Chairs:

- Pushpa Narayanaswami, MD, Beth Israel Deaconess Medical Center; USA
- Ali Habib, MD, University of California, Irvine; USA

#### **Keynote: Controversies in MG-"Novel Therapeutics vs the Real World"**

- James (Chip) F. Howard, Jr., MD, University of North Carolina at Chapel Hill; USA

#### Debate 1:

New targeted therapies if available should be started in all patients at diagnosis

- Carolina Barnett-Tapia, MD, PhD, University Health Network; Canada

vs

New Targeted therapies should be used after conventional therapies are tried

- Pushpa Narayanaswami, MD, Beth Israel Deaconess Medical Center; USA

#### Debate 2:

Thymectomy remains an early treatment option in Achr ab + MG despite the of targeted therapies

- Henry Kaminski, MD, George Washington University; USA

vs

Thymectomy should be reserved for patients with thymoma associated MG

 Gil Wolfe, MD, University at Buffalo Jacobs School of Medicine and Biomedical Sciences; USA

BREAK (10:15 am-10:45am) Explore Posters, Exhibit Hall, and Experience Rooms

SESSION 6 (10:45am-12:15pm) Congenital Myasthenic Syndromes (CMS)

Session Chair: Ricardo A. Maselli, MD, University of California Davis; USA

#### Genetic analysis of CMS

- Kinji Ohno, MD, PhD, Nagoya University Graduate School of Medicine; Japan

#### Postsynaptic/Dok7 CMS

- David Beeson, MA, PhD, University of Oxford Nuffield Department of Clinical Neuroscience; United Kingdom

#### **Presynaptic CMS**

- Ricardo A. Maselli, MD, University of California Davis; USA

#### Agrin/LRP4/MuSK CMS

- Stéphanie Bauché, PhD, Sorbonne University; France

#### **Targeted therapies of CMS**

- Hanns Lochmuller, MD, University of Ottawa; Canada

#### **Moderated Q&A**

- Margherita Milone, MD, PhD, Mayo Clinic; USA

ON-SITE LUNCH (12:15pm-1:30pm) Lunch Provided

SESSION 7 (1:30pm-3:00pm)
Parallel Tracks

### SESSION 7a MG Monitoring & Adverse Events

#### **Session Chairs:**

- Carolina Barnett-Tapia, MD, PhD, University Health Network; Canada
- Stephen Reddel, MBBS, PhD, Sydney Neurology; Australia

#### **International Consensus Guidance management update**

- Pushpa Narayanaswami, MD, Beth Israel Deaconess Medical Center; USA

#### Adverse event monitoring and management

- Michael Hehir, MD, The University of Vermont Medical Center; USA

# Guidelines for managing steroidal therapy complications

- Charles Kassardjian, MD, University of Toronto; Canada

# Managing exacerbations and crisis, and the role of novel treatments

- Heinz Wiendl, University of Freiburg; Germany

# Patient preferences and shared decision making

- Carolina Barnett-Tapia, MD, PhD, University Health Network; Canada

## SESSION 7b Antibodies (Basic Science Perspective)

#### **Session Chairs:**

- Miriam Fichtner, Dr. med, PhD, Charité Universitätsmedizin; Germany
- Kevin O'Connor, PhD, Yale University School of Medicine; USA

#### Mechanisms underlying MG immunopathology - MuSK RTX

- Miriam Fichtner, Dr. med, PhD, Charité Universitätsmedizin; Germany

#### Mechanisms of AChR autoantibody effector functions

- Kevin O'Connor, PhD, Yale University School of Medicine; USA

#### Novel mechanisms associated with MuSK autoantibody-mediated pathology

- Gianvito Masi, MD, Yale University School of Medicine; USA

## Structure function studies of human autoantibodies targeting NMJ receptors

- Colleen Noviello, PhD, University of California, San Diego; USA

# Complement deposition, IgG subtyping and assessment of postsynaptic destruction at the neuromuscular junction in LRP4-antibody-positive generalized myasthenia gravis

- Sarah Hoffmann, M.Sc, Charité Universitätsmedizin; Germany

## BREAK (3:00-3:30pm) Explore Posters, Exhibit Hall, and Experience Rooms

SESSION 8 (3:30pm-5:00pm)
Parallel Tracks

#### **SESSION 8a**

**Non-Pharmacologic Interventions** 

#### **Session Chairs:**

- Ali Habib, MD, University of California, Irvine; USA
- Anna Punga, MD, PhD, Uppsala University; Sweden

## Balancing MG with exercise and sleep: DIG-MG trial

- Anna Punga, MD, PhD, Uppsala University; Sweden

#### Mindfulness for MG

- Sui Wong, MD, King's College; United Kingdom

#### Mental fatigue and associated factors

- Sarah Hoffmann, M.Sc, Charité Universitätsmedizin; Germany

# Comprehensive care in MG: Unmasking the Masqueraders

- Miriam Freimer, MD, Ohio State University Wexner Medical Center; USA

#### **SESSION 8b**

**Models for MG and Translational Research** 

#### Session Chairs:

- Maartje Huijbers, PhD, Leiden University Medical Center; The Netherlands
- Pilar Martinez, PhD, Maastricht University; The Netherlands

# Thymic epithelial organoids from mice

- Sangho Lim, PhD, Hubrecht Institute; The Netherlands

Mechanism of action of the acetylcholine receptor at subunit extracellular domain in inducing antigen-specific immune tolerance in the experimental autoimmune myasthenia gravis model

- Konstantinos Lazaridis, PhD, Hellenic Pasteur Institute; Greece

Induction of acetylcholine receptor-specific, autoreactive B cells in a passive transfer myasthenia gravis rat model

- Jie Luo, PhD, The George Washington University; USA

Skeletal muscle targeted ClC-1 ion channel inhibitor improves skeletal muscle function and respiratory function in a rat model of MuSK-MG

- Marianne Gerberg Skals, PhD, NMD Pharma, Denmark

Immunoregulatory effects of neonatal Fc receptor inhibition by efgartigimod in myasthenia gravis: unraveling a new mechanism of action beyond IgG reduction

- Maria Cristina Tarasco, PhD Student, Fondazione IRCCS Istituto Neurologico "Carlo Besta"; Italy

POSTER SESSION (5:20pm-6:20pm)

**EVENING EVENT Offsite Dinner** 

**Shuttles to Dinner** 

(7:00pm-8:30pm)
Dinner Provided

# **CONFERENCE DAY 3**

Thursday, May 15 (8:00am-5:15pm)

**SESSION 9 (8:00am-10:15am)** 

The Future of MG: Can We Cure MG?

Session Chairs: Stephen Reddel, MBBS, PhD, Sydney Neurology; Australia Jan Verschuuren, MD, PhD, Lieden University Medical Center; The Netherlands

#### Welcome

The Need for a Cure in MG

Stephen Reddel, MBBS, PhD, Sydney Neurology; Australia and Jan Verschuuren, MD, PhD, Lieden University Medical Center; The Netherlands

CAR-T CD19 approaches in autoimmunity

- Aiden Haghikia, MD, Medizinische Hochschule Hannover; Germany

#### CAAR-T anti-AChR

- Aimee Payne, MD, PhD, Columbia University Irving Medical Center; USA

#### MuSK agonist

- Maartje Huijbers, PhD, Leiden University Medical Center; The Netherlands

#### Complement inhibitors in AChR model: What's new?

- Pilar Martinez, PhD, Maastricht University; The Netherlands

# Efficacy and safety of autologous BCMA-directed mRNA CAR T-cell therapy in generalized myasthenia gravis: Results from a phase 2b randomized placebo-controlled trial

- James (Chip) F. Howard, Jr., MD, University of North Carolina at Chapel Hill; USA

#### DOK7-AAV9 gene therapy in a novel mouse model for Congenital Myasthenic Syndrome caused by mutations in CHRND

- Setareh Alabaf, MD, PhD Student, Cambridge University Hospitals; England

BREAK (10:15am-10:45am) Explore Posters, Exhibit Hall, and Experience Rooms

#### SESSION 10 (10:45am-12:15pm) Platforms

#### **Session Chairs:**

- Maartje Huijbers, PhD, Leiden University Medical Center; The Netherlands
- Carolina Barnett-Tapia, MD, PhD, University Health Network; Canada

#### Selective depletion of AChR specific B cells by Chimeric Autoantibody Receptor T cells

- Gregorio Spagni, MD, PhD, University of Florence; Italy

#### Thymectomy in juvenile myasthenia gravis is safe and effective: results of a 15year follow up study

- Michelangelo Maestri, MD, PhD, Neurological Institute, Azienda Ospedaliera Universitaria Pisana; Italy

#### Identification of a Novel Metabolically Active B Cell Cluster in Refractory Myasthenia Gravis: Insights into Plasma Cell Precursors and Therapy Resistance Biomarkers

- Jacopo Morroni, PhD, Fondazione Policlinico Universitario Agostino Gemelli IRCCS ; Italy

Clinical Outcome of Myasthenic Crisis: A Multicenter Prospective Study

- Xiao Huan, Doctorate Student, Huashan Hospital, China

# Effects of IL-4Ra shRNA Lentiviral Particles Treatment in Experimental Autoimmune MuSK Myasthenia Gravis Model

Gizem Koral, PhD Student, Istanbul University; Turkey

#### Risk of Death in Korean Patients with Myasthenia Gravis

- Hye Yoon Chung, Graduate Student, Yonsei University College of Medicine; South Korea

# Muscle-specific tyrosine kinase IgG2-4 and IgA autoantibodies in Myasthenia Gravis

- Sofia-Natsuko Gkotzamani, Graduate Student, Tzartos Neurodiagnostics; Greece

# MiRNA-mRNA-lncRNA networks modulating T and B cell proliferation contribute to unresponsiveness to immunosuppressive drugs in myasthenia gravis

- Paola Cavalcante, PhD, Fondazione IRCCS Istituto Neurologico "Carlo Besta"; Italy

# Facilitators and barriers to exercise in autoimmune MG: a cross-sectional consumer survey study

- Simone Birnbaum, PhD, Institute of Myology; France

ON-SITE LUNCH (12:15pm-1:30pm) Lunch Provided

SESSION 11 (1:30pm- 3:00pm) Personalized Care

#### Session Chairs:

- Anna Punga, MD, PhD, Uppsala University; Sweden
- Jan Verschuuren, MD, PhD, Lieden University Medical Center; The Netherlands

# Objective biomarkers for MG

- Anna Punga, MD, PhD, Uppsala University; Sweden

# Digital endpoints and AI application to MG

- Henry Kaminski, MD, George Washington University; USA

# Home monitoring and apps

- Sophie Lehnerer, MD, PhD, Charité, University Medicine Berlin; Germany

# Artificial intelligence: video or photo analysis

- Martijn Tannemaat, MD, Leiden University Medical Center; The Netherlands

### BREAK (3:00-3:30pm) Explore Posters, Exhibit Hall, and Experience Rooms

#### SESSION 12 (3:30pm-5:00pm) What I Don't Know About MG Roundtable

#### Moderated By:

- Pushpa Narayanaswami, MD, Beth Israel Deaconess Medical Center; USA
- Ali Habib, MD, University of California, Irvine; USA

#### Seronegative MG

- Miriam Freimer, MD, Ohio State University Wexner Medical Center; USA
- Maria Isabel Leite, MD, Dphil, FRCP, Oxford University; England
- Jan Verschuuren, MD, PhD, Lieden University Medical Center; The Netherlands

#### **B-Cell Targeting in MG**

- Sophie Lehnerer, MD, PhD, Charité, University Medicine Berlin; Germany
- Richard Nowak, MD, MS, Yale University School of Medicine; USA
- Sabrina Sacconi, MD, PhD, Nice University Hospital, France

#### **Prognosis in MG**

- James (Chip) F. Howard, Jr., MD, University of North Carolina at Chapel Hill; USA
- Anna Punga, MD, PhD, Uppsala University; Sweden
- Andreas Meisel, Charité, University Medicine Berlin; Germany

#### CLOSING REMARKS (5:00pm-5:15pm)

- Samantha Masterson, President and CEO, MGFA