MGFA 15TH INTERNATIONAL CONFERENCE ON MYASTHENIA AND RELATED DISORDERS

MAY 13-15, 2025 POSTILLION CONVENTION CENTER THE HAGUE, THE NETHERLANDS PROGRAM AGENDA



WELCOME TO THE 15TH INTERNATIONAL CONFERENCE ON MYASTHENIA GRAVIS AND RELATED DISORDERS

Dear Friends,

On behalf of the MGFA Board of Directors, professional staff, our steering committee, and the MGFA Medical & Scientific Advisory Council, welcome to our 15th MGFA International Conference, and our very first program hosted outside of the United States. We are excited and honored to be here with you all in The Hague in The Netherlands. We are nearly 700 strong... What an outstanding reflection of the movement and progress in our MG space!

As you know, this is an incredibly innovative and impactful time in myasthenia gravis research, as evidenced by the sheer number of researchers and clinicians in attendance, as well as the unprecedented number of submitted research abstracts. This year, we have over 200 accepted abstracts and posters – the most we have ever accepted after peer review. It speaks to the extraordinary innovation, technology, and sheer dedication of the global research community in the MG and rare disease space. You will experience the full impact of this groundbreaking work during our oral sessions and throughout the conference.

During the last two years, the MGFA Medical & Scientific Advisory Council has expanded and deepened its involvement across the global landscape. Thanks to the council's leadership, the MGFA has transitioned our research grant awards program to model the US National Institutes of Health (NIH) standards and policies, and we have expanded eligibility to receive grant submissions from international investigators. Our goal is to fund the best and most impactful research projects/programs. We are so pleased to share that our first grant awarded to an investigator outside of the United States occurred in 2024.

We anticipate continued excitement around our grant awards program in the coming years and are committed to maintaining the rigor that will drive so many new innovations and positive outcomes in the MG space. We ask that you look for our upcoming grant opportunities at myasthenia.org/grants.

I hope you have a memorable and inspirational experience here at the MGFA International Conference, and we hope you have fun here in The Hague as well. Thank you for your commitment to the MG community, and thank you for working to make life better for those impacted by myasthenia gravis and other rare diseases.

Sincerely,



Samantha Masterson President and CEO Myasthenia Gravis Foundation of America

Dear Colleagues,

It's a pleasure to welcome you all to the 15th MGFA International Conference on Myasthenia Gravis and Related Disorders! With nearly 700 registered participants representing more than 35 countries and a diversity of backgrounds as basic scientists, translational experts, patients, biotech and commercial parties as well as doctors joining this event, we are confident the conference will provide inspiration for your future and that of MG patients.

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 care, as well as 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.

Throughout the conference, you will enjoy time for cross-pollination of ideas and discussions to build relationships throughout the neuromuscular field. Join us for a special dinner on Wednesday evening offering an opportunity for connection and celebration of the progress made in understanding and treating MG.

Sincerely,



Maartje Huijbers, PhD Steering Committee Co-Chair



Carolina Barnett-Tapia, MD, PhD Steering Committee Co-Chair

STEERING COMMITTEE

Thank you to our steering committee for their leadership.



Maartje Huijbers, PhD Co-chair Leiden University Medical Center, The Netherlands



Carolina Barnett-Tapia, MD, PhD Co-chair University Health Network – Toronto General Hospital, Canada



Valentina Damato, MD University of Florence, Italy



Pushpa Narayanaswami, MD Beth Israel Deaconess Medical Center / Harvard Medical School, United States



Miriam Fichtner, Dr. med, PhD Charité – Universitätsmedizin Berlin, Germany



Richard Nowak, MD, MS Yale School of Medicine, United States



Ali Habib, MD University of California Irvine, United States



Kevin O'Connor, PhD Yale School of Medicine, United States



Rozen Le Panse, PhD Pierre and Marie Curie University, France



Anna Punga, MD, PhD Uppsala University, Sweden



Pilar Martinez-Martinez, PhD Maastricht University, The Netherlands



Stephen Reddel, MBBS, PhD Sydney Neurology, Australia



Ricardo Maselli, MD University of California Davis Health, United States



Jan J.G.M. Verschuuren, MD, PhD Leiden University Medical Center, The Netherlands

AGENDA SUMMARY

MONDAY, MAY 12

14:00 –18:00 Pre-Registration / Check-In

POSTER SESSION ABSTRACTS



Download and view the abstracts and poster locations on the MGFA conference webpage.

TUESDAY, MAY 13 • 08:00 - 17:00

All sessions take place in Congress 2/3

06:30 – 07:45 Registration/Check-In

07:00 – 07:45 Breakfast Foyer

08:00 – 08:15 Welcome and Opening Remarks

Session 1 – 08:15 – 10:15 Overview of Autoimmunity

10:15 - 10:45

Break: Explore Posters, Exhibitor Hall, and UCB Experience Room Congress 2/3, Congress 4 (Exhibitor Hall), and 5th Floor (Experience Room)

Sponsored by Immunovant

Session 2 – 10:45 – 12:15 The Social Impact of MG with Guest Patient Panel **12:15 – 13:30 Luncheon** Foyer

Session 3 – 13:30 – 15:00 Etiology of MG

15:00 - 15:30

Break: Explore Posters, Exhibitor Hall, and UCB Experience Room Congress 2/3, Congress 4 (Exhibitor Hall), and 5th Floor (Experience Room)

Sponsored by Immunovant

Session 4 – 15:30 – 17:00 Special Populations

17:00 - 19:00

Welcome Reception and Poster Session Congress 2, 3, & 4

Hors d'oeuvres and refreshments provided in Congress 2/3 Sponsored by Johnson & Johnson Innovative Medicine

AGENDA SUMMARY

WEDNESDAY, MAY 14 • 08:00 - 18:20

All sessions take place in Congress 2/3

07:00 – 07:45 Breakfast

08:00 – 08:10 Welcome and Opening Remarks

Session 5 – 08:10 – 10:15 Controversies in MG

10:15 - 10:45

Break: Explore Posters, Exhibitor Hall, and UCB Experience Room Congress 2/3, Congress 4 (Exhibitor Hall), and 5th Floor (Experience Room)

Sponsored by Immunovant

Session 6 – 10:45 – 12:15 Congenital Myasthenic Syndromes (CMS)

12:15 – 13:30 Luncheon Foyer

13:30 – 15:00 Parallel Session Tracks: Session 7A – Antibodies (Basic Science Perspective) Congress 2

Session 7B – MG Monitoring & Adverse Events Congress 3

15:00 - 15:30

Break: Explore Posters, Exhibitor Hall, and UCB Experience Room Congress 2/3, Congress 4 (Exhibitor Hall), and 5th Floor (Experience Room) Sponsored by Immunovant

15:30 - 17:00

Parallel Session Tracks: Session 8A – Non-Pharmacologic Interventions Congress 2

Session 8B – Models for MG and Translational Research Congress 3

17:20 - 18:20

Poster Sessions Congress 2, 3, & 4

19:30 - 21:30

Celebratory Gathering and Dinner The Atrium – City Hall of the City of Den Haag, Spui 70, Den Haag Shuttles provided from select hotels starting at 19:00

Sponsored by argenx

THURSDAY, MAY 15 • 08:00 - 17:15

All sessions take place in Congress 2/3

07:00 – 07:45 Breakfast

08:00 – 08:25 MGFA Lifetime Achievement Award: Presentation and Remarks from Honored Recipient

Session 9 – 08:25 – 10:20 The Future of MG: Can We Cure MG?

10:15 – 10:45 Break: Explore Posters, Exhibitor Hall, and UCB Experience Room Congress 2 & 3, Congress 4 (Exhibitor Hall), and 5th Floor (Experience Room)

Sponsored by Immunovant

Session 10 – 10:45 – 12:15 Platform **12:15 – 13:30** Luncheon Foyer

Session 11 – 13:30 – 15:00 Personalized Care

15:00 - 15:30

Break: Explore Posters, Exhibitor Hall, and UCB Experience Room Congress 2/3, Congress 4 (Exhibitor Hall), and 5th Floor (Experience Room)

Sponsored by Immunovant

Session 12 – 15:30 – 17:00 What I Don't Know About MG Roundtable

17:00 – 17:15 Closing Remarks

Visit the Exhibitor Hall and UCB Experience Room

Learn about our partners in the Exhibitor Hall in Congress 4 and in the UCB Experience Room on the 5th floor. These spaces are open during breaks from conference sessions.

Meeting Rooms

If you are meeting with an industry partner, these will take place on the 5th and 6th floors of the Postillion. There will be someone to greet and direct you to the appropriate room(s), as well as signage.

FULL AGENDA

All sessions take place in Congress 2/3

08:00 - 08:10 • Welcome and Opening Remarks



Samantha Masterson, President and CEO Myasthenia Gravis Foundation of America



Maartje Huijbers, PhD, Leiden University Medical Center; The Netherlands Steering Committee Co-Chair



Carolina Barnett-Tapia, MD, PhD, University Health Network; Canada Steering Committee Co-Chair

Session 1 • 08:15 – 10:15 • Overview of Autoimmunity

SESSION CHAIRS:



Rozen Le Panse, PhD, Pierre and Marie Curie University; France



Pilar Martinez-Martinez, PhD, Maastricht University; The Netherlands

Keynote: The future of autoimmunity: the place for precision medicine



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

Dr. Berrih-Aknin is a leading immunologist specializing in autoimmunity. She joined INSERM (Institut national de la santé et de la recherche médicale) in 1985. For over 40 years, her research has centered on myasthenia gravis, investigating both the muscle (target organ) and thymus (effector organ). She has explored the autoimmune antiacetylcholine receptor response, identified key molecular and cellular mechanisms, and uncovered immunoregulatory defects in MG patients. Her work has also contributed to understanding sex-based differences in autoimmunity. She has published approximately 200 research articles on these topics. Even after retirement, she continues her work on autoimmunity mechanisms and precision medicine innovations.

Gut microbiome and the antibody-microbiota interface in autoimmunity



Delphine Sterlin, PharmD, PhD, Sorbonne University; France

Dr. Sterlin is an associate professor of immunology at Sorbonne University in Paris. She is also affiliated with the Department of Immunology at La Pitié-Salpêtrière Hospital in Paris. Her current translational research aims to create a substitute therapy using intestinal IgA for patients with IgA deficiency. Additionally, she works on autoimmune diseases for elucidating how the gut contributes to auto-antibody maturation.

B cells orchestrate tolerance to the neuromyelitis optica autoantigen AQP44



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

Dr. Korn is a full professor of neurology, director of the Institute for Experimental Neuroimmunology, and deputy director of the Department of Neurology. He is a member of several research consortia funded by the German Research Council (TRR274, TRR355, SyNergy Excellence Cluster) and held an ERC consolidator grant. For his work, Dr. Korn was awarded a series of prizes, including the Sobek Young Investigator Award, the Heinrich-Pette-Preis of the German Neurologic Society, and the Sobek Award for MS research. His major interest remains the molecular characterization of fate decisions of T cells in development and inflammation.

Differences in IgG autoantibody Fab glycosylation across autoimmune diseases



Theo Rispens, PhD, Sanquin and Vrije Universiteit Amsterdam; The Netherlands Since 2006 Dr. Rispin has worked as a principal investigator at the Department of Research at Sanquin. In 2024 he was appointed as a professor of molecular immunology and biotherapeutics at the Department of Molecular Cell Biology and Immunology at VU. His work involves basic and translational research toward understanding the role of the humoral immune response in disease to optimize biopharmaceutical therapies. To achieve this, his team develops biophysical and immunochemical methodology to investigate structure-function relationships of immunoglobulins in a basic and clinical setting.

Thymic cell populations in establishment of self-tolerance



Marte Heimli, PhD, Oslo University Hospital; Norway

Dr. Heimli is a postdoctoral fellow at the Department of Medical Genetics at the University of Oslo and Oslo University Hospital. She holds a PhD from the University of Oslo, and her research focus includes the use of multi-modal single-cell and spatial technologies for studying the thymus in health and in myasthenia gravis.

10:15 - 10:45 • Explore Posters, Exhibitor Hall, and UCB Experience Room

Congress 2/3, Congress 4 (Exhibitor Hall), and 5th Floor (Experience Room)

Coffee breaks sponsored by Immunovant

Session 2 • 10:45 - 12:15 • The Social Impact of MG with Patient Panel

PATIENT PANEL:



Lutgarde Allard, Patient Advocate, ABMM/EuMGA; Belgium

Ms. Allard was diagnosed with myasthenia gravis in March 1994 and has been a patient advocate since 1998. She was educated to be a nurse and worked as a civil servant from 1980 to 2010. She was a member of the Flemish Myasthenia Gravis organization from 1994 to 2023. Since May 2023, she has served as Board treasurer for the European MG Association. In 2023, she attended an in-depth training on patient advocacy organized by Patvocates and took an active role in the development of the EURO-NMD patient journey on myasthenia gravis. She is a patient representative in the Neuromuscular Junction Defects Group.



Júlíana Magnúsdóttir, Patient Advocate, MG-Fèlag Islands; Iceland

Ms. Magnúsdóttir is a nurse BSc and lactation consultant IBCLC. She was diagnosed with myasthenia gravis in 2016 and serves as a board member of MG-Fèlag Islands, taking an active role in building the organization up from dormancy. This year she is doing a diploma on disability studies at the University of Iceland and planning on extending those studies into a master's degree focusing on the aspects of disability connected to MG.



Liselotte Schau Schirakow, Patient Advocate, MG Group of Muskelvindfonden; Denmark

Ms. Schirakow has lived with generalized myasthenia gravis since 2006. She works as a midwifery specialist at the university hospital of Odense, Denmark. Liselotte has been a patient advocate since 2006, advocating at national and international levels. In 2022, she attended an in-depth training on patient advocacy organized by Patvocates. Since May 2024, she has been a member of the board of the European MG Association.



Maria Bonaria (Maya) Uccheddu, Patient Advocate, Associazione Italiana Miastenia (AIM); Italy

Ms. Uccheddu is a patient living with myasthenia gravis, a pharmacist, and a freelance digital artist with a master's degree in pharmaceutical chemistry and technology. She co-owns and manages a para-pharmacy in Italy. Maya serves as the chairperson for the MG patient advocacy committee in the European MG Association. She successfully completed the "MG Advocate Development Program" and is attending the Eurordis "School on Medicines Research and Development." She is a patient representative in the Neuromuscular Junction Defects Group.

SESSION MODERATORS:



Dong Dong, PhD, JC School of Public Health and Primary Care, Chinese University of Hong Kong; Hong Kong

Dr. Dong is an assistant professor specializing in interdisciplinary health services research with a strong focus on patient-centered care for underserved populations. Since 2014, she has conducted repeated national cross-sectional studies to assess the multifaceted impact of rare diseases, while also developing and validating measurement tools tailored to capture the health-related quality of life of rare disease patients. She is deeply engaged in rare disease advocacy and policy, serving on the boards of the Asia Pacific Alliance of Rare Disease Organizations, the China Alliance for Rare Diseases, the Illness Challenge Foundation, and the Beijing Aili Myasthenia Gravis Care Center.



Carolina Barnett-Tapia, MD, PhD, University Health Network – Toronto General Hospital: Canada

Dr. Barnett-Tapia is an active clinician-scientist as well as an associate professor in the Department of Medicine, Division of Neurology and the Institute of Health Policy, Management and Evaluation, at the University of Toronto. Her main research interest is patient-centered outcomes and patient preference elicitation in patients with neuromuscular disorders and neurofibromatosis. She developed the Myasthenia Gravis Impairment Index (MGII) a novel, patient-centered measure of disease severity. She received the 2020 Surinderjit Singh Young Lectureship Award from AANEM and the 2023 MGFA Medical Partner of the Year Award.

12:15 - 13:30 • Luncheon

Foyer

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Share your conference experience with the MGFA's community. Tag us on Instagram or LinkedIn.



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(in) @myastheniagravisfoundationofamericainc

TUESDAY, MAY 13, 2025

Session 3 • 13:30 – 15:00 • Etiology of MG

SESSION CHAIRS:



Rozen Le Panse, PhD, Pierre and Marie Curie University; France

Pilar Martinez-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

In honor of the late Dan Drachman, the co-principal investigator on myasthenia gravis work



Bryan Traynor, MD, PhD, National Institute on Aging; USA

Dr. Traynor is a neurologist and a senior investigator at the National Institute on Aging. He is best known for his work unraveling the genetic causes of amyotrophic lateral sclerosis (ALS) and frontotemporal dementia (FTD). Dr. Traynor has over 200 publications in professional journals, including Neuron, New England Journal of Medicine, Nature Genetics, and Nature Neuroscience. He sits on the editorial boards of JAMA Neurology (2017–2021), JNNP, and Neurobiology of Aging and is an associate editor for Brain. He has received numerous awards for his work, including the NIH Director's award, the Sheila Essey Award, and the Potamkin Prize, and has been elected fellow of the Royal College of Physicians of Ireland, the Royal College of Physicians (London), and the Association of American Physicians.

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



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

Dr. Yasumizu is an associate research scientist in Yale's Department of Neurology. He earned his MD in 2019 and PhD in immunology in 2024 from Osaka University, Japan. His research interests lie at the intersection of neuroimmunology and autoimmune diseases, with a particular focus on elucidating the molecular mechanisms driving conditions such as myasthenia gravis and neuromyelitis optica through cutting-edge omics approaches and computational biology.

IL17/Th17/thymoma



Güher Saruhan-Direskeneli, PhD, Istanbul University; Turkey

Dr. Saruhan-Direskeneli is a professor of physiology at the Department of Physiology at Istanbul University, Istanbul Medical Faculty. Her main research interests are immune mechanisms and genetics of myasthenia gravis and vasculitides such as Behcet's disease and Takayasu arteritis.

Subclinical MG in thymomas



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

Dr.Marcuse, is a pulmonologist in training at the MUMC. She has been involved with MUMC's thymic team since 2013, with a special affinity for rare thoracic cancers, paraneoplastic syndromes and thymic diseases. She is the founder of the Dutch National Thymic Tumor Board and chair of the committee for communication, education, and symposia at the Dutch Rare Cancer Platform.

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



Soumya S. Yandamuri, MSE, PhD, University of Colorado Anschutz Medical Campus; USA

Dr. Yandamuri recently joined the faculty of the Anschutz Medical Campus as an assistant professor in the neurology department. Her laboratory investigates novel neuropathogenic mechanisms of innate cellular immunity, with a focus on neurologic autoantibodies and natural killer (NK) cells in demyelinating diseases. Dr. Yandamuri started her neuroimmunology training in Professor Thomas Lane's laboratory, then at the University of Utah, and conducted her post-doctoral training in Professor Kevin O'Connor's laboratory at Yale, where she investigated the pathogenic properties of neurologic autoantibodies and potential disease biomarkers.

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



Paula Terroba-Navajas, PhD Student, University Hospital Münster; Germany

Ms. Terroba-Navajas is a third-year PhD student in neuroimmunology at the University of Münster. She completed her undergraduate studies in biochemistry and molecular biology at the University of the Basque Country (Spain) and specialized in neuroscience with a master's degree at the University of Science and Technology, in Trondheim (Norway). Currently, her research focuses on the characterization of antibody effector responses and the exploration of predictive signatures in various autoimmune diseases, as well as the application of single-cell transcriptomics to dissect cellular pathways in disease-specific contexts.

TUESDAY, MAY 13, 2025

15:00 - 15:30 • Explore Posters, Exhibitor Hall, and UCB Experience Room

Congress 2/3, Congress 4 (Exhibitor Hall), and 5th Floor (Experience Room)

Session 4 • 15:30 - 17:00 • Special Populations

SESSION CHAIRS:



Valentina Damato, MD University of Florence; Italy

Ali Habib, MD University of California, Irvine; USA

Ocular MG – notes and updates



Jeanine Heckmann, MBChB, PhD, University of Cape Town; South Africa

Dr. Heckmann is a consultant neurologist and professor of neurology at UCT and Groote Schuur Hospital. She heads the neurology research group in the UCT Neurosciences Institute, with a research focus on neuromuscular diseases in Africans, particularly myasthenia gravis, motor neuron disease/amyotrophic lateral sclerosis, and inherited neuromuscular disease.

Women and pregnancy-related issues in MG



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

Dr. Evoli is an associate professor of neurology. Her clinical and research interests are mainly focused on neuroimmunology, particularly myasthenia gravis, other disorders of neuromuscular transmission, paraneoplastic neurological diseases, and autoimmune disorders of the central nervous system. She is a member of the Italian Society of Neurology and the Italian Myology Association.

Neonatal and juvenile MG



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

Dr. Ramdas is a consultant pediatric neurologist as well as an honorary senior lecturer at University of Oxford. She leads the regional Paediatric Neuromuscular Service and Highly Specialised Paediatric Multiple Sclerosis Service. She is the pediatric lead for the Highly Specialised Services for Congenital Myasthenia Syndromes and Neuromyelitis Optica. She leads the NHS England Rare Disease Collaborative Network (RDCN) for Juvenile Myasthenia Gravis.

Is LEMS a myasthenic disorder?



Jan J.G.M. Verschuuren, MD, PhD, Leiden University Medical Center; The Netherlands

At LUMC, Dr. Verschuuren heads the clinical neuromuscular section, the Department of Neurology, and the board of the Neuroscience Research Theme. He has been involved in translational research on several forms of autoimmune myasthenia, including AChR and MuSK myasthenia gravis; Lambert-Eaton myasthenic syndrome; and Duchenne and Becker muscular dystrophy. His group was the first to describe the HLA association of MuSK MG, studying the special and unexpected role of IgG4 antibodies in this disease. Currently, the group is working on the development of a targeted therapy using the results of studies with human monoclonal antibodies derived from MuSK MG patients.

17:00 - 19:00 • Welcome Reception and Poster Session

Congress 2, 3, 4; Hors d'oeuvres and refreshments provided in Congress 2/3

Thank you to our generous sponsor Johnson & Johnson Innovative Medicine.

WEDNESDAY, MAY 14, 2025

All sessions take place in Congress 2/3 unless otherwise noted.

Session 5 • 08:00 - 10:15 • Controversies in MG

SESSION CHAIRS:



Pushpa Narayanaswami, MD Beth Israel Deaconess Medical Center / Harvard Medical School; USA



Richard Nowak, MD, MS Yale School of Medicine; USA

Keynote: Controversies in MG - Evolving Novel Therapeutics in MG Treatment – Boon or Bust?



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

Dr. Howard is a professor of neurology, medicine, and allied health in the Department of Neurology at UNC-Chapel Hill School of Medicine. He has practiced neurology for over 43 years with a focus on myasthenia gravis and EMG. He currently directs the Myasthenia Gravis Clinical Trials and Translational Research Unit. He is the former James F. Howard Distinguished Professor of Neuromuscular Disease and the prior chief of the Neuromuscular Disorders Section at UNC. Dr. Howard also holds an appointment as adjunct professor of clinical sciences (neurology) at North Carolina State University College of Veterinary Medicine.

MGFA Funding Opportunities

Through our grant program, the MGFA funds the most promising research. Consider applying for one of two upcoming grant opportunities in 2025. These grants are open to investigators globally.

- MGFA High Impact Clinical Research and Scientific Pilot Projects on Myasthenia Gravis and Related Neuromuscular Junction Disorders grants up to \$110,000, over two years, to support high-impact clinical research and scientific pilot studies that are focused and innovative. Letters of Intent to apply will be accepted between May 15 and June 15, 2025.
- The Clinician Scientist Development Award in Myasthenia Gravis, in collaboration with the American Brain Foundation, will provide up to \$160,000 to early career clinicians/scientists with the purpose of developing the next generation of researchers working to better understand and alleviate the effects of myasthenia gravis through innovative research. Applications will be accepted June 2, 2025 through September 9, 2025, via the American Brain Foundation.

Learn more by searching Myasthenia Gravis Foundation of America on ProposalCentral.com, or visit myasthenia.org.



Debate 1: Novel Therapies



Novel therapies should not be used as first-line treatment in MG

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

Dr. Barnett-Tapia is an active clinicianscientist as well as an associate professor in the Department of Medicine, Division of Neurology and the Institute of Health Policy, Management and Evaluation, at the University of Toronto. Her main research interest is patient-centered outcomes and patient preference elicitation in patients with neuromuscular disorders and neurofibromatosis. She developed the Myasthenia Gravis Impairment Index (MGII) a novel, patient-centered measure of disease severity. She received the 2020 Surinderjit Singh Young Lectureship Award from AANEM and the 2023 MGFA Medical Partner of the Year Award.



Novel therapies should be used as first-line treatment in MG

Pushpa Narayanaswami, MD, Beth Israel Deaconess Medical Center / Harvard Medical School; USA

Dr. Narayanaswami is a professor of clinical neurology at Harvard Medical School and the vice-chair of clinical operations for Beth Israel's Department of Neurology. She is a fellow of the American Academy of Neurology and co-editor of the book Principles and Practice of the Muscular Dystrophies. She serves as an associate editor for Muscle and Nerve and the associate editor for classification of evidence for Neurology. Dr. Narayanaswami has received research funding from AHRQ, PCORI AND NIH. She is the recipient of the 2019 AANEM Jun Kimura Outstanding Educator Award.

Debate 2: Thymectomy

Thymectomy remains an early treatment option in AChR ab+ MG

Henry Kaminski, MD, George Washington University; USA

Dr. Kaminski is the Meta A. Neumann Professor of Neurology. He has performed clinical, translational, and basic investigations related to myasthenia gravis for over 25 years with continuous support of the federal and nonprofit organizations. In 2019, Dr. Kaminski established, as principal investigator, the NIH Rare Disease Clinical Research Network site dedicated to myasthenia gravis, MGNet. Recently, he has started work towards objective quantitation of the neuromuscular examination via telemedicine using artificial intelligence to enhance clinical outcome measures and clinical trial performance.



Thymectomy should be pushed aside as an early treatment option in AChR ab+ MG and be reserved for thymomatous MG

Gil Wolfe, MD, University at Buffalo Jacobs School of Medicine and Biomedical Sciences, State University of New York; USA Dr. Wolfe is a SUNY Distinguished Professor and immediate past chairman of the Department of Neurology. His clinical activity and academic investigation focus on neuromuscular disorders, particularly on myasthenia gravis and peripheral neuropathies. He has authored or co-authored over 160 papers and 25 chapters on neuromuscular disorders. He is a fellow of the American Neurological Association and American Academy of Neurology and recipient of the 2018 SUNY Chancellor's Award for Excellence in Scholarship and Creative Activities.

10:15 - 10:45 • Explore Posters, Exhibitor Hall, and UCB Experience Room

Congress 2/3, Congress 4 (Exhibitor Hall), and 5th Floor (Experience Room)

Coffee breaks sponsored by Immunovant

Session 6 • 10:45 - 12:15 • Congenital Myasthenic Syndromes (CMS)

SESSION CHAIR:

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

Genetic analysis of CMS



Kinji Ohno, MD, PhD, Nagoya University of Arts and Sciences; Japan

Dr. Ohno is a professor in the Graduate School of Nutritional Sciences and the vice dean of School of Liberal Arts. He previously served as a university vice president and as division leader for the Division of Neurogenetics, Nagoya University Graduate School of Medicine, and spent a decade at Mayo Clinic. His research interests include (i) physiology and pathology of the neuromuscular junction, (ii) development of AI tools for biomedical data sciences, (iii) RNA metabolisms in physiology and pathology, (iv) development of therapeutic strategies for musculoskeletal disorders, (v) gut microbiota in neurodegenerative diseases, (vi) molecular mechanisms of molecular hydrogen, and (vii) quantal effects of extremely weak electromagnetic field on human diseases.

Postsynaptic/Dok7 CMS



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

Dr. Beeson is a professor of neuroscience and a fellow of the Academy of Medical Sciences. He has spent more than three decades studying disorders of neuromuscular transmission. In early studies he was the first to clone many of the genes encoding key proteins at the human neuromuscular junction, and subsequently his research focused on identifying and understanding the genes and molecular mechanisms that underlie congenital myasthenic syndromes.

Presynaptic CMS



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

Dr. Maselli is the director of UC Davis' Clinical Neurophysiology-EMG Laboratory and a professor of neurology. He is a translational neurologist whose main research interests are CMS and gene therapy for CMS.

Agrin/LRP4/MuSK CMS



Stéphanie Bauché, PhD, Sorbonne University; France

Dr. Bauché received her PhD in neurosciences in 2013 and joined the Myology Research Center at Sorbonne University in 2019. She has a robust expertise in the human neuromuscular junction, which has allowed her to publish over 40 scientific articles in her field. She has become a key figure in pathophysiological studies of mutations identified in CMS.

Targeted therapies of CMS



Hanns Lochmuller, MD, University of Ottawa Faculty of Medicine / The Ottawa Hospital Department of Medicine; Canada

Dr. Lochmuller is a neurologist and clinical academic specializing in genetic neuromuscular disorders and rare disease. He is a senior scientist at the Children's Hospital of Eastern Ontario (CHEO) Research Institute. He also holds an appointment as a professor of neurology at University of Ottawa. From 2007 to 2017, he held the chair of experimental myology at the Institute of Genetic Medicine at Newcastle University in the UK.

Moderated Q&A



Margherita Milone, MD, PhD, Mayo Clinic; USA

Dr. Milone is a professor of neurology and consultant neurologist with expertise in neuromuscular disease. She serves as director of Mayo Clinic's Muscle Pathology Laboratory and Muscular Dystrophy Association Care Center. Her current research focuses on mechanisms of inherited muscle disorders, especially those with rimmed vacuoles, and correlation genotype-phenotype.

12:15 - 13:30 • Luncheon

Foyer

WEDNESDAY, MAY 14, 2025

13:30 - 15:00 • Parallel Session Tracks

SESSION A • Antibodies (Basic Science Perspective) • Congress 2 SESSION B • MG Monitoring & Adverse Events • Congress 3

SESSION 7A Antibodies (Basic Science Perspective)

Congress 2

SESSION CHAIRS:



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

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

Mechanisms underlying MG immunopathology – MuSK RTX



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

Dr. Fichtner is a trained medical doctor with a degree from the Institute of Clinical Neuroimmunology (AG Meinl) at Ludwig-Maximilian University of Munich. She received a PhD in 2023 from University of Maastricht. As a postdoctoral fellow at Yale School of Medicine, she received several awards, including the Eberhard Pfleiderer Preis and the 1st Felix Jerusalem Preis. She leads an independent group focusing on B cells in autoimmune diseases and serves as visiting faculty at Koç University in Istanbul.

Novel therapeutic targeting of AChR autoantibody effector functions



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

Dr. O'Connor is a professor of neurology and immunobiology. His investigative interests are focused on human translational immunology and neurology. He and his group are specifically interested in defining the mechanisms by which B cells—and the autoantibodies they produce—contribute to the pathology of human autoimmune neurological disease. Dr. O'Connor serves as chief scientific advisor to the MGFA.



Alexandra Bayer Wildberger, PhD Yale School of Medicine; USA

Structure function studies of human autoantibodies targeting the muscle AChR



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

Dr. Noviello is a project scientist and founding member of Ryan Hibbs' laboratory at UC San Diego. The lab's focus is on structure-function studies of the Cys-loop superfamily. She has worked on the $\alpha 4\beta 2$, $\alpha 7$, $\alpha 3\beta 4$, and the muscle-type of nicotinic acetylcholine receptors, as well as on the GABA-A receptor. Dr. Noviello leads the lab's autoimmune branch, with a focus on autoimmune autonomic ganglionopathy (targeting the $\alpha 3\beta 4$ nAChR), GABA-A autoimmune encephalitis, and myasthenia gravis.

Novel insights into the MuSK-specific autoantibody repertoire



Gianvito Masi, MD, Yale School of Medicine; USA

Dr. Masi obtained his medical degree at Catholic University of the Sacred Heart in Rome, Italy. He then completed the Neurology Residency Program at Agostino Gemelli University Hospital in Rome. In 2021 Dr. Masi joined Professor Kevin O'Connor's laboratory at Yale, where he is currently working as an associate research scientist. Dr. Masi's research and clinical interests span the field of autoimmune neurology, with a focus on myasthenia gravis.

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

Dr. Hoffmann is a professor of neuromuscular research, senior neurologist, and deputy of the Integrated Myasthenia Gravis Center (IMZ) at Charité. Her work focuses on both translational and clinical aspects of myasthenia gravis, including the investigation of antibody-specific pathomechanisms and addressing unmet clinical needs such as fatigue and seronegative MG. She received the Pfleiderer Myasthenia Gravis Research Award for her research on complement-mediated pathomechanisms in seronegative MG and serves on the Medical Advisory Board of the German Myasthenia Gravis Society.

WEDNESDAY, MAY 14, 2025

SESSION 7B

MG Monitoring & Adverse Events Congress 3

SESSION CHAIRS:



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



Stephen Reddel, MBBS, PhD Sydney Neurology; Australia

Adverse event monitoring and management



Michael Hehir, MD, Robert Larner, MD College of Medicine at the University of Vermont; USA

Dr. Michael Hehir is the James F. Howard Jr. M.D. Professor of Neuromuscular Disorders, vice chair of clinical affairs, and division chief of neuromuscular medicine in the Department of Neurological Sciences. He serves on the board of the Neuromuscular Study Group and is a long-standing advisor to the MGFA. Through MGFA funding, his team developed a patient and physician-weighted consensus unit called the Adverse Event Unit (AEU) that can quantify and compare side effect burden among any classes of medications for neurologic conditions. In an effort to improve treatment decisions, Dr. Hehir's current research is focused on applying the AEU and other tools to measure the comparative side effect burden among the treatments employed for myasthenia gravis patients and other neurological patients.

Guidelines for managing steroidal therapy complications



Charles Kassardjian, MD, MSc, FRCPC, University of Toronto; Canada

Dr. Kassardjian is a neuromuscular neurologist and assistant professor with a clinical focus in the area of peripheral neurological diseases and neurophysiology. His research and educational focus is on quality improvement, patient safety, and innovation. He has a particular interest in medication safety and standardization of practice for neuromuscular patients treated with immunosuppressive medications. Dr. Kassardijian co-founded NQIL (the Neurology Quality and Innovation Lab) to foster collaboration among neurologists looking to work on quality and safety-related projects.

Managing exacerbations and crisis, and the role of novel treatments



Heinz Wiendl, MD, University of Freiburg; Germany

Dr. Wiendl is the head of the university's Department of Neurology and Neurophysiology. His research focuses on inflammatory neurodegeneration and immune regulation and protection as well as monitoring multiple sclerosis and its therapy. Dr. Wiendl is a member of numerous scientific and academic advisory boards and expert panels, and he is the founder and principal investigator of the "Body and Brain Institute" Muenster, a prestigious research building funded by the German Federal Ministry. His achievements have been recognized by both Sobek awards of the German Society for MS.

Patient preferences and shared decision making



Carolina Barnett-Tapia, MD, PhD, University Health Network; Canada Dr. Barnett-Tapia is an active clinician-scientist as well as an associate professor in

Dr. Barnett-Tapia is an active clinician-scientist as well as an associate professor in the Department of Medicine, Division of Neurology and the Institute of Health Policy, Management and Evaluation, at the University of Toronto. Her main research interest is patient-centered outcomes and patient preference elicitation in patients with neuromuscular disorders and neurofibromatosis. She developed the Myasthenia Gravis Impairment Index (MGII) a novel, patient-centered measure of disease severity. She received the 2020 Surinderjit Singh Young Lectureship Award from AANEM and the 2023 MGFA Medical Partner of the Year Award.

How do I do all this in a 30-minute visit?



Pushpa Narayanaswami, MD, Beth Israel Deaconess Medical Center / Harvard Medical School; USA

Dr. Narayanaswami is a professor of clinical neurology at Harvard Medical School and the vice-chair of clinical operations for Beth Israel's Department of Neurology. She is a fellow of the American Academy of Neurology and co-editor of the book Principles and Practice of the Muscular Dystrophies. She serves as an associate editor for Muscle and Nerve and the associate editor for classification of evidence for Neurology. Dr. Narayanaswami has received research funding from AHRQ, PCORI AND NIH. She is the recipient of the 2019 AANEM Jun Kimura Outstanding Educator Award.

Q&A

15:00 - 15:30 • Explore Posters, Exhibitor Hall, and UCB Experience Room

Congress 2/3, Congress 4 (Exhibitor Hall), and 5th Floor (Experience Room)

Coffee breaks sponsored by Immunovant

WEDNESDAY, MAY 14, 2025

15:30 - 17:00 • Parallel Session Tracks

SESSION A • Non-Pharmacologic Interventions • Congress 2

SESSION B • Models for MG and Translational Research • Congress 3

SESSION 8A

Non-Pharmacologic Interventions Congress 2

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

Dr. Punga is a professor of clinical neurophysiology and a consultant physician at Uppsala University Hospital. Her postdoctoral studies in Basel, Switzerland, focusing on animal models of MG earned her the Eberhardt Pfleiderer Prize from the German Myasthenia Gravis Foundation. In 2014, she started her independent translational research group on MG at Uppsala University. Her translational research focuses on improving diagnostic and monitoring tools and care in MG, including biomarkers. She holds multiple highly competitive national research grants and was awarded the Göran Gustafsson large prize in medicine for her studies in MG.

Support the MGFA's Research Mission

Myasthenia Gravis Foundation of America has a long history of fighting for people with MG – improving access to care, funding research, educating the community, and supporting patients and their families. Our donors help ensure this work continues. Make a difference by making a gift to support our most critical work.

Make a Gift



Mindfulness for MG



Sui H. Wong, MBBS, MD, FRCP, Moofields Eye Hospital, Guys and St Thomas' Hospitals, KCL and UCL; United Kingdom

Dr. Wong is a consultant neurologist and neuro-ophthalmologist with affiliate academic institutions of King's College London and University College London. She leads one of the largest dedicated ocular myasthenia gravis services internationally. Her research on mindfulness-based interventions in neurological disorders, particularly her work on visual snow syndrome, earned the North American Neuro-Ophthalmology Society's J. Lawton Smith Award for most impactful research. She led the development of the first validated rating scale for ocular myasthenia gravis, and her current research explores evidence-based lifestyle interventions for neurological conditions.

Fatigue in MG: Understanding More, Doing Better



Sarah Hoffmann, M.Sc, Charité Universitätsmedizin; Germany

Dr. Hoffmann is a professor of neuromuscular research, senior neurologist, and deputy of the Integrated Myasthenia Gravis Center (IMZ) at Charité. Her work focuses on both translational and clinical aspects of myasthenia gravis, including the investigation of antibody-specific pathomechanisms and addressing unmet clinical needs such as fatigue and seronegative MG. She received the Pfleiderer Myasthenia Gravis Research Award for her research on complement-mediated pathomechanisms in seronegative MG and serves on the Medical Advisory Board of the German Myasthenia Gravis Society.

Comprehensive care in MG: Unmasking the Masqueraders



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

Dr. Freimer is a clinical professor of neurology at The Ohio State University. Until recently she was the director of the Neuromuscular Division and is currently the director of the Myasthenia Gravis Clinic and the director of the Electromyography Laboratory. Dr. Freimer has been active in both the care of patients with neuromuscular junction disorders and in clinical research of these disorders. She is the author or coauthor of over 75 peer-reviewed journal articles and numerous abstracts. She is and has been the site principal investigator for multiple clinical research trials in both myasthenia gravis and other neuromuscular disorders.

WEDNESDAY, MAY 14, 2025

SESSION 8B

Models for MG and Translational Research Congress 3

SESSION CHAIRS:



Maartje Huijbers, PhD Leiden University Medical Center; The Netherlands

Pilar Martinez-Martinez, PhD Maastricht University; The Netherlands

Thymic epithelial organoids from mice



Sangho Lim, PhD, Hubrecht Institute; The Netherlands

Since 2019, Dr. Lim has been a postdoctoral researcher in the Organoid group led by Dr. Hans Clevers at the Hubrecht Institute. He has been pioneering the development of novel organoid models to study thymic epithelial cells from both mice and humans, focusing on their role in T cell education. He is also investigating the antigen presentation function of epithelial cells under inflammatory conditions and exploring innovative cancer treatment strategies through neoantigen identification and CAR-T/TCR-T cell therapies using organoid-immune cell co-culture models. He earned his Ph.D. in immunobiology from Hanyang University, South Korea.

Mechanism of action of the acetylcholine receptor a1 subunit extracellular domain in inducing antigen-specific immune tolerance in the experimental autoimmune myasthenia gravis model



Konstantinos Lazaridis, PhD, Hellenic Pasteur Institute; Greece

Dr. Lazaridis is a researcher and group leader at the Department of Immunology of the HPI and head of the institute's Biotechnology Unit. Dr. Lazaridis studied molecular biology at the University of Surrey, in Guildford, UK and obtained his PhD at the University of East Anglia, in Norwich, UK, studying TGF-b cell signalling in normal and pathological conditions. He started working in the field of autoimmunity and specifically myasthenia gravis in 2006. Currently, his main efforts are directed towards the development and characterization of novel therapeutic strategies for autoimmune diseases, especially targeted antigen specific approaches.

Building an induced pluripotent stem cell-based 3D neuromuscular junction on a chip: a novel model to study neuromuscular disease



Robyn Verpalen, PhD Student, Leiden University Medical Center; The Netherlands Since 2021, Ms. Verpalen has been a graduate student in the NeuroImmunology group led by Dr. Maartje Huijbers. She has been developing a novel human pluripotent stem cell derived 3D neuromuscular junction on a chip model, where functional outcome measures like contraction are a central point of focus. Ultimately the goal is to model MG and other neuromuscular diseases within the neuromuscular junction on a chip. She holds a master's degree in biomedical sciences from Leiden University.

Skeletal muscle targeted CIC-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

Dr. Skals is a trained physiologist. One of her focus areas is myasthenia gravis animal models, both for generalized MG and MuSK MG. During her PhD program and a five-year postdoc, she focused on cell membrane signaling in relation to bacterial toxins and the translation to in vivo animal models of sepsis and urinary tract infections. In 2018 she made the transition from academia to industry. Since then, she has focused on a growing expertise within pre-clinical animal studies in neuromuscular diseases and drug development.

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

Ms. Tarasco is a third-year graduate student in experimental neuroscience at the University of Milano-Biococca. She is also a senior fellow at the FINCB. She holds a bachelor's degree in biology from the University of Bari (Italy) and a master's degree in molecular biology from the University of Parma (Italy). Her PhD project aims to identify non-coding RNAs involved in MG pathogenesis and uncover their potential as targets of innovative RNA-based therapies.

Q&A

17:20 - 18:20 • Poster Session

Congress 2, 3, 4

19:30 – 21:30 • Celebratory Gathering

The Atrium – City Hall of the City of Den Haag

Thank you to our strategic sponsor argenx.

Join conference attendees at an offsite dinner to connect and celebrate the progress made in the study of myasthenia gravis and related disorders.

Dress: Business Casual / Shuttles will be provided to and from the following hotels: Hotel Indigo, Voco, Hotel Novotel, Hotel Hilton, Hotel Ibis, Hotel Leonardo

Shuttles depart hotels starting at 19:00 and return at the conclusion of the event.

Attendees are also welcome to walk, use the tram, or use a ride share / taxi service.

Partners in Care Program

The MGFA Partners in MG Care cohort is comprised of providers who have an interest in treating MG patients and are committed to making life better for those living with MG. All Partners are listed on the MGFA website to assist patients and their families.

All our Partners in MG Care go through an evaluation process and are committed to:

- Providing quality care to MG patients
- Empowering patients and caregivers by providing accessible education and resources
- Participating in knowledge-sharing, professional education, and networking opportunities in the MG space
- Engaging with and staying informed about the work of the MGFA

Learn more and apply to be a Partner in MG care:



All Sessions take place in Congress 2/3

8:00 - 08:25 • MGFA Lifetime Achievement Award Presentation

PRESENTED BY:



Samantha Masterson President and CEO, MGFA



Kevin O'Connor, PhD Yale School of Medicine; Chief Scientific Officer, MGFA

The MGFA Lifetime Achievement Award recognizes a distinguished scientist, scholar, or clinician who has made significant 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 9 • 8:25 - 10:20 • The Future of MG: Can We Cure MG?

SESSION CHAIRS:



Stephen Reddel, MBBS, PhD Sydney Neurology; Australia

Jan J.G.M. Verschuuren, MD, PhD Lieden University Medical Center; The Netherlands

The Need for a Cure in MG



Stephen Reddel, MBBS, PhD, Sydney Neurology; Australia

Dr. Reddel is a staff specialist neurologist at Concord Repatriation & General Hospital Sydney, where he leads the neuroimmunology clinic, and consultant neurologist at the Brain & Mind Research Institute, University of Sydney. He has a longstanding interest in clinical safety, including the Alemtuzumab in MS Safety Study (AMS3), which led to the development of RxPx, a multinational medical IT safety company. His research interests include examining the function of anti-MuSK antibodies and the homeostasis of the neuromuscular junction; neuroimmunology and MS; and neurogenetics, including the muscular dystrophies and inherited neuropathies.

CAR-T CD19 approaches in autoimmunity



Aiden Haghikia, MD, Medizinische Hochschule Hannover; Germany

Dr. Haghikia is a clinical neurologist and neuroimmunologist, serving as professor and chair of the Department of Neurology and Clinical Neurophysiology at MHH. He trained at the Ruhr-University Bochum, where he also started his translational research on the role of the gut microbiome and diet on autoimmunity of the central nervous system. He went on to continue his research on the genetics of neuroimmunological diseases as well as cell-based therapies in myasthenia gravis and other neuroimmunological disorders.

CAAR-T antigen-specific immunotherapy approaches in autoimmunity



Aimee Payne, MD, PhD, Columbia University Irving Medical Center; USA Dr. Payne is the Herbert and Florence Irving Professor and Chair of Dermatology. Her research laboratory focuses on mechanisms and treatments of autoimmunity, with particular focus on B cell-mediated autoimmune diseases, including pemphigus and myasthenia gravis. Her laboratory invented a targeted immunotherapy approach for antigen-specific B cell depletion known as chimeric autoantibody receptor T-cell (CAAR-T) therapy and performed key studies leading to FDA clearance of two Investigational New Drug Applications for CAAR-T technology in mucosal pemphigus vulgaris and MuSK myasthenia gravis, alongside preclinical validation of CAAR-T approaches for AChR myasthenia gravis and other autoantibody-mediated diseases.

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, FAAN, University of North Carolina at Chapel Hill; USA

Dr. Howard is a professor of neurology, medicine, and allied health in the Department of Neurology at UNC-Chapel Hill School of Medicine. He has practiced neurology for over 43 years with a focus on myasthenia gravis and EMG. He currently directs the Myasthenia Gravis Clinical Trials and Translational Research Unit. He is the former James F. Howard Distinguished Professor of Neuromuscular Disease and the prior chief of the Neuromuscular Disorders Section at UNC. Dr. Howard also holds an appointment as adjunct professor of clinical sciences (neurology) at North Carolina State University College of Veterinary Medicine.

Complement inhibitors in AChR model: What's new?



Pilar Martinez-Martinez, PhD, Maastricht University; The Netherlands

Dr. Martinez-Martinez is a leading professor in neuroinflammation of neuropsychiatric disorders, renowned for her extensive contributions to the fields of neuroinflammation, neuropsychiatric disorders, and autoimmunity. Her research focuses on understanding the molecular mechanisms underlying autoimmune and neuroinflammatory diseases such as myasthenia gravis, Alzheimer's disease, psychotic disorders, and on developing novel diagnostic and therapeutic strategies.

MuSK agonist antibodies to treat neuromuscular diseases



Maartje Huijbers, PhD, Leiden University Medical Center; The Netherlands Dr. Huijbers is an associate professor and research group leader at the Department of Human Genetics and the Department of Neurology at LUMC. Currently, the translational research in her group, in close collaboration with clinicians, focuses on understanding the cause and consequences of (IgG4) autoantibodies and B cells and their characteristics in autoimmune neuromuscular diseases. Together with a pharmaceutical partner, a therapeutic (MuSK agonist ARGX-119) stemming from her research is now being tested in phase 2 clinical trials.

DOK7-AAV9 gene therapy in a novel mouse model for congenital myasthenic syndrome caused by mutations in CHRND



Setareh Alabaf, MD, DPhil, University of Oxford; UK

Dr. Alabaf studied medicine at University of Gothenburg in Sweden and completed a DPhil at University of Oxford in the lab of Prof. David Beeson and Dr. Yin Dong. During her doctoral studies, she worked on DOK7 AAV9 gene therapy in mouse models of CMS. She is currently in her first year of neurology residency and has a special interest in neuromuscular disorders, in particular CMS.

10:20 - 10:45 • Explore Posters, Exhibitor Hall, and UCB Experience Room

Congress 2/3, Congress 4 (Exhibitor Hall), and 5th Floor (Experience Room)

Coffee breaks sponsored by Immunovant

THURSDAY, MAY 15, 2025

Session 10 • 10:45 - 12:15 • 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; Charité - Universitätsmedizin Berlin, Germany

Dr. Spagni is a consultant neurologist and early career clinician-scientist with a strong interest in myasthenia gravis. He completed his residency in neurology and PhD in neuroscience at the Catholic University of Rome (Italy) and spent two years as a research fellow at the Charité University of Berlin (Germany). Dr. Spagni's clinical and research interests focus on myasthenia gravis and other antibody-mediated nervous system disorders, such as autoimmune encephalitis, neuromyotonia, and neuromyelitis optica spectrum disorders. His research interests include optimizing antibody diagnostics, identifying biomarkers of treatment response, and developing novel therapeutic approaches to target disease-driving B cells.

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



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

Dr. Tassoni is a neurologist at the university's Neurological Clinic. Since 2005, he has dedicated most of his clinical and research activity to treating patients with myasthenia gravis, in the group led by Dr. Roberta Ricciardi. After her retirement, he became director of the Myasthenia Gravis Group at the Azienda Ospedaliero Universitaria Pisana and University of Pisa. He has run several clinical trials on innovative therapies in MG and has a particular focus and collaborations on thymoma and neurological-related diseases and on MuSK-MG.

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

Dr. Morroni is a postdoctoral fellow at the university's Experimental Neurology Lab. His research focuses on the role of B cells in autoimmune neurological diseases, with a particular emphasis on myasthenia gravis. His work delves into the intricate characterization of thymic and peripheral B cell subpopulations, exploring their correlation with therapy response in different MG patient cohorts.

Clinical Outcome of Myasthenic Crisis: A Multicenter Prospective Study



Xiao Huan, MD, PhD, Huashan Hospital, Fudan University; China

Dr. Xiao Huan is a neurology resident specializing in neuromuscular disorders with a translational research focus on myasthenia gravis pathophysiology and immune dysregulation. Her current work focuses on myasthenic crisis and refractory MG cohort studies, integrating longitudinal clinical assessments with multi-platform immune profiling to identify predictive biomarkers and disease progression patterns. Her research characterizes CD4+T cell immune signatures in MG subtypes through flow cytometry, scRNA-seq, TCR analysis, and cytokine profiling, elucidating their clinical correlations and therapeutic implications.

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



Gizem Koral, PhD Student, Istanbul University; Turkey

Ms. Koral is currently pursuing a PhD in advanced neurological sciences through the university's Aziz Sancar Institute of Experimental Medicine. Her research focuses on autoimmune neurological diseases, particularly through the experimental autoimmune myasthenia gravis (EAMG) model. Her current work involves the preclinical evaluation of RNA interference-based therapies to modulate pathogenic immune responses in myasthenia gravis.

Risk of Death in Korean Patients with Myasthenia Gravis



Hye Yoon Chung, MD, Yonsei University College of Medicine; South Korea

Dr. Chung is a clinical assistant professor of the Department of Neurology at Yongin Severance Hospital. She specializes in neuromuscular diseases, with a primary research interest in myasthenia gravis. She completed both residency and fellowship training at Severance Hospital, Yonsei University College of Medicine.

THURSDAY, MAY 15, 2025

Muscle-specific tyrosine kinase IgG1-4 and IgA autoantibodies in myasthenia gravis



Sofia-Natsuko Gkotzamani, PhD Student, Tzartos Neurodiagnostics; Greece After obtaining her master's degree in human biology, Ms. Gkotzamani worked as a research associate in protein crystallography at Proteros Biostructure GmbH in Germany. Since September 2024, she has been pursuing a PhD as part of the IgG4-TREAT Consortium, a Marie Skłodowska-Curie EU-funded initiative focused on advancing research in IgG4 autoimmune diseases.

MiRNA-mRNA-IncRNA 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 Dr. Cavalcante has spent two decades studying immunology. Currently she leads a team at the institute focused on myasthenia gravis. She is coordinator of a project dedicated to the development of RNS-based advanced therapies to achieve immunomodulation in MG, as well as the European project "MG-PerMed," which aims to develop biomarker-based artificial intelligence models to promote implementation of personalized medicine in MG clinical practice. In 2018, she received the Mosaic of Autoimmunity (MAI) Award for outstanding contribution to the field of autoimmunity.

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



Simone Birnbaum, PhD, Institute of Myology; France

Dr. Birnbaum is a research physiotherapist in the Neuromuscular Physiology and Evaluation Laboratory. She completed her physiotherapy degree at the University of Melbourne, Australia. Her doctoral studies focused on exercise training in autoimmune myasthenia gravis. Dr. Birnbaum is passionate about improving patient care and autonomy, research translation, and the role of exercise in chronic disease. Current areas of research include patient-reported measures, the evaluation and implementation of exercise interventions, and fatigability and dyspnoea in autoimmune and inherited neuromuscular disorders.

12:15 - 13:30 • Luncheon

Foyer

Session 11 • 13:30 – 15:00 • Personalized Care

SESSION CHAIRS:



Anna Punga, MD, PhD Uppsala University; Sweden

Jan J.G.M. Verschuuren, MD, PhD Lieden University Medical Center; The Netherlands

Objective biomarkers for MG



Anna Punga, MD, PhD, Uppsala University; Sweden

Dr. Punga is a professor of clinical neurophysiology and a consultant physician at Uppsala University Hospital. Her postdoctoral studies in Basel, Switzerland, focusing on animal models of MG earned her the Eberhardt Pfleiderer Prize from the German Myasthenia Gravis Foundation. In 2014, she started her independent translational research group on MG at Uppsala University. Her translational research focuses on improving diagnostic and monitoring tools and care in MG, including biomarkers. She holds multiple highly competitive national research grants and was awarded the Göran Gustafsson large prize in medicine for her studies in MG.

Digital endpoints and AI application to MG



Henry Kaminski, MD, George Washington University; USA

Dr. Kaminski is the Meta A. Neumann Professor of Neurology. He has performed clinical, translational, and basic investigations related to myasthenia gravis for over 25 years, with continuous support of federal and nonprofit organizations. In 2019, Dr. Kaminski established, as principal investigator, the NIH Rare Disease Clinical Research Network site dedicated to myasthenia gravis, MGNet. Recently, he started work towards objective quantitation of the neuromuscular examination via telemedicine using artificial intelligence to enhance clinical outcome measures and clinical trial performance.

Home monitoring and apps



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

Dr. Lehnerer is a board-certified neurologist at the certified Integrated Myasthenia Center. Her research focuses on the burden of disease in MG as well as the development and scientific evaluation of digital solutions for telemonitoring and telemedical treatment of myasthenia patients. For her innovative work in this field, she was awarded the Health-i Award by Handelsblatt and Techniker Krankenkasse. Dr. Lehnerer has led multiple studies on myasthenia, including research on the use of complement inhibitors in myasthenic crisis. Her commitment to patient collaboration in research has been recognized with the Patient and Stakeholder Award from the Berlin Institute of Health.

Artificial intelligence: video or photo analysis



Martijn Tannemaat, MD, Leiden University Medical Center; The Netherlands Dr. Tannemaat is a neurologist and clinical neurophisyologist. He is a co-author of the national guideline on MG and acts as a scientific advisor, both to the patient advocacy organization Spierziekten Nederland and to several pharmaceutical and biotech companies. His research focuses on clinical aspects of MG, including the development of novel diagnostic strategies such as quantitative MRI, evoked potentials, and artificial intelligence. In a recently started collaboration with Huma Therapeutics, he aims to develop an AI-based smartphone tool to allow MG patients to monitor their symptoms.

15:00 - 15:30 • Explore Posters, Exhibitor Hall, and UCB Experience Room

Congress 2/3, Congress 4 (Exhibitor Hall), and 5th Floor (Experience Room)

Coffee breaks sponsored by Immunovant

Session 12 • 15:30 - 17:00 • What I Don't Know About MG Roundtable

MODERATED BY:

Pushpa Narayanaswami, MD Beth Israel Deaconess Medical Center / Harvard Medical School; USA



Ali Habib, MD University of California, Irvine; USA

Seronegative MG



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

Dr. Freimer is a clinical professor of neurology at The Ohio State University. Until recently she was the director of the Neuromuscular Division and is currently the director of the Myasthenia Gravis Clinic and the director of the Electromyography Laboratory. Dr. Freimer has been active in both the care of patients with neuromuscular junction disorders and in clinical research of these disorders. She is the author or coauthor of over 75 peer-reviewed journal articles and numerous abstracts. She is and has been the site principal investigator for multiple clinical research trials in both myasthenia gravis and other neuromuscular disorders.



Maria Isabel Leite, MD, Dphil, FRCP, Oxford University; England

Dr. Leite has developed her clinical and research career on autoimmune neurology within the Nuffield Department of Clinical Neurosciences. While her clinical expertise, research interest and educational activities are broad, her main focus is on autoimmune disorders of the neuromuscular junction, in particular myasthenia gravis and Lambert-Eaton myasthenic syndrome, including those associated with malignancy (e.g. thymoma and lung cancer) as well as associated with immunotherapies. She is principal investigator in several research projects and has been awarded research grants, including from Myaware and Muscular Dystrophy UK. She was made associate professor of neurology in 2019.



Jan J.G.M. Verschuuren, MD, PhD, Lieden University Medical Center; The Netherlands At LUMC, Dr. Verschuuren heads the clinical neuromuscular section, the Department of Neurology, and the board of the Neuroscience Research Theme. He has been involved in translational research on several forms of autoimmune myasthenia, including AChR and MuSK myasthenia gravis; Lambert-Eaton myasthenic syndrome; and Duchenne and Becker muscular dystrophy. His group was the first to describe the HLA association of MuSK MG, studying the special and unexpected role of IgG4 antibodies in this disease. Currently, the group is working on the development of a targeted therapy using the results of studies with human monoclonal antibodies derived from MuSK MG patients.

B-Cell Targeting in MG



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

Dr. Lehnerer is a board-certified neurologist at the certified Integrated Myasthenia Center. Her research focuses on the burden of disease in MG as well as the development and scientific evaluation of digital solutions for telemonitoring and telemedical treatment of myasthenia patients. For her innovative work in this field, she was awarded the Health-i Award by Handelsblatt and Techniker Krankenkasse. Dr. Lehnerer has led multiple studies on myasthenia, including research on the use of complement inhibitors in myasthenic crisis. Her commitment to patient collaboration in research has been recognized with the Patient and Stakeholder Award from the Berlin Institute of Health.

THURSDAY, MAY 15, 2025



Richard Nowak, MD, MS, Yale School of Medicine; USA

Dr. Nowak currently serves as chief medical advisor to the MGFA and is the chair of the MGFA Research Committee. He is a neuromuscular-fellowship-trained neurologist and a faculty member in the Department of Neurology at Yale. He is the founding director of both the Program for Clinical & Translational Neuromuscular Research (CTNR) and the Yale Myasthenia Gravis Clinic. In this leadership capacity, he has successfully established and built a translational neuromuscular immunology program. Since formation of the CTNR program, he has initiated over a dozen studies focused on immune-mediated neuromuscular conditions.



Sabrina Sacconi, MD, PhD, Nice University Hospital; France

Prof. Sacconi is a neurologist and head of the Department of Peripheral Nervous System and Muscle. She leads multiple initiatives, including the CCMR Neurogenetics and CRMR Neuromuscular centers, and has published extensively in neurology, with a focus on MG. She is the principal investigator for four ongoing studies and has three upcoming studies related to MG. Additionally, she organized the 278th European Neuromuscular Centre International Workshop, held in September 2024. Her research also encompasses other neuromuscular diseases, and she is actively involved in various national and international projects.

Prognosis in MG



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

Dr. Howard is a professor of neurology, medicine, and allied health in the Department of Neurology at UNC-Chapel Hill School of Medicine. He has practiced neurology for over 43 years with a focus on myasthenia gravis and EMG. He currently directs the Myasthenia Gravis Clinical Trials and Translational Research Unit. He is the former James F. Howard Distinguished Professor of Neuromuscular Disease and the prior chief of the Neuromuscular Disorders Section at UNC. Dr. Howard also holds an appointment as adjunct professor of clinical sciences (neurology) at North Carolina State University College of Veterinary Medicine.



Anna Punga, MD, PhD, Uppsala University; Sweden

Dr. Punga is a professor of clinical neurophysiology and a consultant physician at Uppsala University Hospital. Her postdoctoral studies in Basel, Switzerland, focusing on animal models of MG earned her the Eberhardt Pfleiderer Prize from the German Myasthenia Gravis Foundation. In 2014, she started her independent translational research group on MG at Uppsala University. Her translational research focuses on improving diagnostic and monitoring tools and care in MG, including biomarkers. She holds multiple highly competitive national research grants and was awarded the Göran Gustafsson large prize in medicine for her studies in MG.



Andreas Meisel, Dr. med, Charité Universitätsmedizin Berlin; Germany

Dr. Andreas Meisel is a professor of neurology and consultant neurologist at the Department of Neurology with Experimental Neurology and Neuroscience Clinical Research Center. He serves as director of the Centre for Stroke Research Berlin and head of the Integrated Myasthenia Gravis Center at the Charité Universitätsmedizin. His group is engaged in experimental and clinical research in the fields of neuromuscular and cerebrovascular disorders. The aim is to develop and establish biomarker guided treatment strategies for individualized patient care.

17:00 - 17:15 • Closing Remarks



Samantha Masterson President and CEO, MGFA

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THANK YOU TO OUR SPONSORS FOR THEIR VITAL SUPPORT OF THIS EVENT.





Johnson &Johnson Innovative Medicine



Trainee and Early Career Investigator Scholarships

Engaging with the brightest minds in neuromuscular research from around the world can help launch a career and spark ideas for the next generation of young scientists. We are grateful to Johnson & Johnson Innovative Medicine for their support of scholarships to bring young and emerging investigators to the 15th International Conference.

A Special Acknowledgement

With Sincere Gratitude...

- Our Steering Committee for their exceptional work guiding an impactful, exciting, and important gathering.
- The MGFA Medical and Scientific Advisory Council for their leadership and guidance.
- Our event management company in Europe, DDMC.

Emergency / Healthcare Needs

Pharmacies

Drogisterij Parfumerie Van der Gaag

Dagelijkse Groenmarkt 27, 2513 AL Den Haag Monday: 11:00 – 18:00 Tuesday: 09:00 – 18:00 Wednesday: 09:00 – 18:00 Thursday: 09:00 – 20:00 Friday: 09:00 – 18:00

Etos

Wagenstraat 2, 2511 BD Den Haag Monday: 11:00 – 18:00 Tuesday: 10:00 – 18:00 Wednesday: 10:00 – 18:00 Thursday: 10:00 – 21:00 Friday: 10:00 – 18:00

Apotheek Hoefkade Hoefkade 203, 2526 BR Den Haag 08,30 - 18,00

Huisartsenpost (Urgent Care Center)

Huisartsenpraktijk Arts en Zorg Goudenregenhof

Goudenregenstraat 32C, 2565 EX Den Haag www.artsenzorg.nl/vind-uw-praktijk/ goudenregenhof-den-haag 08:00 – 17:00

Huisartsenpost HagaZiekenhuis

Charlotte Jacobslaan 10, 2545 AB Den Haag Open 24 Hrs

Hospital

Haga Hospital location Leyweg Els Borst-Eilersplein 275, 2545 AA Den Haag

NOTES

FOR A WORLD WITHOUT MYASTHENIA GRAVIS

