

# 14<sup>TH</sup> MGFA

INTERNATIONAL CONFERENCE on Myasthenia and related disorders

# WELCOME TO MANIE

Hyatt Regency Miami May 10<sup>th</sup> - 12<sup>th</sup> 2022

Conference Program

for more information, visit myasthenia.org

# Welcome to Miami and the 14th MGFA International Conference on Myasthenia and Related Disorders

#### Dear Friends.

Welcome to the 14th MGFA International Conference on Myasthenia and Related Disorders! We are so thrilled to welcome you, and we want to thank you for your support and participation. We cannot think of a better way to come together after two long years of being apart than to join together in sunny Miami for three days of superb science in a field that we all highly regard!

The MGFA International Conference is the premiere global conference solely focused on MG and related disorders, and the caliber of researchers and medical professionals who come together to share cutting-edge research, progress, and new discoveries is second to none. The sheer expertise and talent is absolutely superb! The MGFA aims to bring together leading academic scientists, researchers, and research scholars to exchange and share their experiences and research results on all aspects of myasthenia gravis. It also provides a premier interdisciplinary platform for researchers, practitioners, and educators to present and discuss the most recent innovations, trends, and concerns, as well as practical challenges encountered and solutions adopted in the field of myasthenia gravis.

Over the course of the next three days, you will listen and learn from both basic and clinical researchers from around the globe, participating from more than 23 countries, covering topics from bench to bedside. We want to extend our most sincere gratitude to these speakers for sharing their expertise and knowledge, and certainly for the very important work that they do to advance the field of myasthenia gravis. We extend our highest appreciation to the global Steering Committee that has worked tirelessly for the better part of the last year to develop the program for this conference, and who assisted in engaging the speakers, selecting the topics, and ensuring that we developed and presented a comprehensive forum on myasthenia gravis and related disorders. We are so very grateful for our volunteer partners and their desire to collaborate with us. Finally, we would like to thank our sponsors. MGFA is extremely fortunate to have industry partners who support all of our efforts and work closely with the organization to improve the quality of life for those living with myasthenia gravis and to advance treatments. Together, and only together, will we move closer to a world without myasthenia gravis.

We wish you a great conference and encourage you to take advantage of your time here... ask questions, introduce yourself and make new friends! Again, we are thrilled that you have joined us, and we thank you for your support.

Sincerely,



Brian Gladden Chair of the MGFA Board of Directors



Samantha Masterson
President and CEO
MGFA



We Deeply Thank Our Generous Conference Industry
Partners and Sponsors. We Appreciate Your Commitment
to the Myasthenia Population.

# **NATIONAL PRESENTING PARTNERS**









# **NATIONAL SPONSORS**















# 14th MGFA International Conference Program at a Glance

# Tuesday, May 10, 2022

#### 7:00 - 8:00 Breakfast (Riverwalk)

8:00 - 8:15 Welcome - Samantha Masterson, President, and CEO, MGFA

Brian Gladden, Chair, Board of Directors, MGFA (Regency Ballroom)

#### 8:15 – 9:05 Session 1: Rationale for Therapeutics in Development (Regency Ballroom)

Chair: Nils Erik Gilhus, MD, PhD

Complement Inhibition: From Basics to RCT Data, Saiju Jacob, MD, PhD

FcRn: Mechanism and RCT Data, Sally Ward, MD

#### 9:05 - 10:00 Keynote Speaker (Regency Ballroom)

Introduction: Carolina Barnett-Tapia, MD, PhD

Keynote Speaker: Angela Vincent, MBBS (Hon PHD Bergen), FRCPath, FMedSci, FRS,

10:00 - 10:30 Coffee Break (Riverwalk)

#### 10:30 - 12:00 Session 2: Other Therapeutics (Regency Ballroom)

Chair: Volkan Granit, MD

B Cell Depletion: Mechanisms and RCT Data, Richard Nowak, MD, MS

CAR-T Therapy for AChRAb positive MG, Volkan Granit, MD

Hinge-Deleted IgG4 Blocker Therapy for AChR MG, Mario Losen, PhD

MuSK CAAR-T, Aimee Payne, MD, PhD

12:00 - 13:30 Lunch Break (Riverwalk)

#### 13:30 - 15:00 Session 3: Update in MG Immunopathogenesis (Regency Ballroom)

Chair: Kevin O'Connor, PhD

Complement and Autoimmune Disease, Jeffrey Bennett, MD, PhD

MuSK B Cells (MGNet), Kevin O'Connor, PhD

Single-cell Profiling in Myasthenia Gravis, Bettina Schreiner, MD, PhD

Targeting IL-23 Amerliorates Tymic and Neuromuscular Defects in MG, Nadine Dragin, PhD

15:00 - 15:30 Coffee Break (Riverwalk)

#### 15:30 - 16:40 Session 4: Autoantibodies (Regency Ballroom)

Chair: Maartje Huijbers, PhD

MuSK IqG4 Pathogenicity, Glycosylation, Maartje Huijbers, PhD

Identification of Rare Membrane Antigen Specific Human B Cells, Nicholas Sanderson, PhD Heterogeneity Of Acetylcholine Receptor Autoantibody–Mediated Complement Activity In

Patients With Myasthenia Gravis, Abeer Obaid, PhD Candidate

A bioassay for neuromuscular junction-restricted complement activation by myasthenia gravis

acetylcholine receptor antibodies, JJ Plomp, PhD

16:45 Optional Session – Luc Truyen, Chief Medical Officer, argenx (Orchid ABC)

18:00 - 19:30 Poster Session 1 (Regency Ballroom)

19:30 Welcome Reception (Riverwalk)

#### 7:00 - 8:00 Breakfast (Riverwalk) Basic Science Trainee Breakfast (Hibiscus A)

8:00 Welcome: Carolina Barnett-Tapia, MD, PhD for Clinical Sessions (Orchid ABC) Kevin O'Connor for Basic Science Sessions (Regency Ballroom)

#### 8:05 - 10:00 Parallel Session 1A: International views/Challenging Cases (Orchid ABC)

Chair: Carolina Barnett-Tapia, MD, PhD

Panelists: Ha Young Shin, MD, PhD (Korea), Janine Heckmann, MD (So Africa),

Valeria Salutto, MD (Argentina), Dong Dong, PhD (Hong Kong), Fatmah Al-Zahmi, MD (Middle East)11-May

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#### 8:05 – 10:00 Parallel Session 1B: Biomarkers (Regency Ballroom)

Chair: Linda Kusner, PhD

Biomarker Development, Methodological Challenges, Gary Cutter, PhD

Survivin, Linda Kusner, PhD

Treatment Predictive Metabolomic Biomarkers for Myasthenia Gravis, Henry Kaminski, MD

Circulating microRNA in MG, Anna Punga, MD, PhD Biomarker Response to THX, Sonia Berrih-Aknin, PhD

10:00 - 10:30 Coffee Break (Riverwalk)

#### 10:30 - 12:10 Parallel Session 2A: Updates in MG Management (Orchid ABC)

Chair: Erika Greene, MD

Diagnostic Challenges, Robert De Meel, MD

Management Issues, Refractory Myasthenia, Ali Habib, MD

Updates in LEMS and Paraneoplastic NMJ disorders, Jan Verschuuren, MD

Ocular MG, Sui Wong, MD

#### 10:30 – 12:10 Parallel Session 2B: MG Pathophysiology and Thymus (Regency Ballroom)

Chair: Rozen Le Panse, PhD

Role of Endogenous Nucleic Acids in Interferon Type 1 Signature in EOMG, Rozen Le Panse, PhD

NMJ Function and Safety Factor, William Phillips, PhD

miR-146a in MG Thymus, Paola Cavalcante, PhD MuSK is a BMP Co-Receptor, Justin Fallon, PhD

12:10 - 13:30 Lunch (Riverwalk)

#### 13:30 – 15:10 Session 3: Congenital MG/Pediatric MG (Regency Ballroom)

Chair: David Beeson, MA, PhD

Updates on CMS, Ricardo Maselli, MD

Mechanism of Disease and Therapeutic Rescue of DOK7 in CMS, Steven Burden, PhD

Pediatric MG, Emmanuelle Tiongson, MD

B-adrenergic Treatment, David Beeson, MA, PhD

15:10 - 15:40 Coffee Break (Riverwalk)

#### 15:40 - 16:20 Session 4: Tolerance Induction (Regency Ballroom)

Chair: Amelia Evoli, MD

Preclinical Study of an Antigen-Specific Therapy for the Treatment of MG, Konstantinos Lazaridis, PhD Oral Tolerance of MuSK EAMG, Debby Reuveni, MD

16:20 Closing: Carolina Barnett-Tapia, MD, PhD (Regency Ballroom)

16:45 - 17:45 Special Session: MGNet Outcome Measure Training (Regency Ballroom)

18:00 - 19:30 Poster Session 2 (Regency Ballroom)

#### 7:00 - 8:00 Breakfast (Riverwalk) Clinical Trainee Breakfast (Hibiscus B)

8:00 – 8:05 Welcome Kevin O'Connor, PhD (Regency Ballroom)

#### 8:05 - 10:00 Session 1: Abstract Submissions - Platform Presentations (Regency Ballroom)

Chairs: Carolina Barnett-Tapia, MD, PhD and Kevin O'Connor, PhD

- B Cell Related Predictive Biomarkers Of Treatment Response In Myasthenia Gravis, Valentina Damato, MD
- Dysgammaglobulinemia In Musk Myasthenia Gravis Patients, Dana Vergoossen, PhD Candidate
- Introducing The Pediatric Myasthenia Gravis Consortium, Jonathan Strober, MD
- Conditioned Mesenchymal Stromal Cells As Tools For Immunomodulation In Myasthenia Gravis, Alexandra Bayer Wildberger, PhD Candidate
- Dok7-Aav Improved Ácetylcholine Deficiency Cms In A Mouse Model And Enhanced The Effectiveness Of Pyridostigmine Treatment, Yin Yao Dong, PhD
- Five Decades Of Epidemiology In Myasthenia Gravis: Data From Two Italian Mg Referral Centers, Silvia Falso, PhD
- B Cells From Muscle-Specific Tyrosine Kinase Antibody Positive Myasthenia Gravis Patients Show Increased Frequencies Of Cd20 Low And Cxcr5 Negative Populations, Patricia Sikorski, PhD
- A Population-Based Cohort Study Of Pregnancy Outcomes In Women With Myasthenia Gravis, Carolina Barnett-Tapia, MD, PhD
- Mri Of The Extra-Ocular Muscles In Myasthenia Gravis Show Small Volume And Fat Fraction Increases, Kevin Keene, PhD Candidate
- Effects of IgG1-MuSK Antibodies on the Agrin-Induced AChR Clustering Pathway, Michelangelo Cao, MD, PhD

10:00 - 10:30 Break (Riverwalk)

#### 10:30 - 12:00 Session 2: Hot Topics I (Regency Ballroom)

Chair: James Howard Jr., MD

PROMISE MG, Pushpa Narayanaswami, MD, FAAN

Drug Pricing, A.Gordon Smith, MD

ICER - Health Economic Assessment of New Drugs for MG, Foluso Agboola, MBBS, MPH

12:00 - 13:30 Lunch (Riverwalk)

#### 13:30 – 15:20 Session 3: Hot Topics II (Regency Ballroom)

Chair: Anna Punga, MD, PhD CARE MG, Srikanth Muppidi, MD

Exercise in MG RCT, Simone Birnbaum, PhD Prednisone Taper RCT, Tarek Sharshar, MD, PhD

Checkpoint Inhibitor NMJ Disease, Amanda Guidon, MD

15:20 Closing: Samantha Masterson, President and CEO, MGFA

Safe travels home!

#### MG Research

Incredible Progress and Momentum Against Myasthenia Gravis

Since our inception in 1952, the MGFA has led the charge to support the most promising scientific endeavors—funding research, engaging young scientists and clinicians, and spearheading a comprehensive patient registry. Research has led to significant improvements in diagnostic techniques, treatments and therapies, and improved disease management. Understanding myasthenia gravis (MG), its causes, treatments, and prognoses, is possible through ongoing research. This is an extraordinary time in the MG Community for progress and momentum against myasthenia. There are many new scientific discoveries that could lead to better treatments and a cure for MG.

In addition, new treatments and medicines are coming online soon, and other treatment technology is evolving and improving. People with MG have every reason to be hopeful - new treatment choices will become a reality in the next few years. The following Website resources reflect the power of MG Research.

# Applying for Participation in MG Clinical Trials

MGFA is dedicated to driving research to better understand, treat and cure myasthenia gravis for good. To achieve this goal, we are committed to creating awareness about clinical trials for those with myasthenia gravis and related neuromuscular joint disorders.

Visit myasthenia.org/Research/Apply-to-Clinical-Trials

# MGFA Research Grants and Funding

We support the most promising and impactful research that will improve the lives of patients with myasthenia gravis and related neuromuscular junction disorders. The committee has identified five broad research priorities: Biomarkers, Disease Mechanisms, Targeted Therapies, Patient Outcomes and Pediatric Treatment.

Visit myasthenia.org/Professionals/Research-Grants

### Clinical Overview of MG

What was once a relatively obscure condition of interest primarily to neurologists is now the best characterized and understood autoimmune disease. A wide range of potentially effective treatments are available, many of which have implications for the treatment of other autoimmune disorders.

Visit myasthenia.org/Professionals/Clinical-Overview-of-MG



#### Hot Research News

Articles and coverage that calls out key new developments in MG research and clinical trials. Visit myasthenia.org/Research/Hot-Research-News

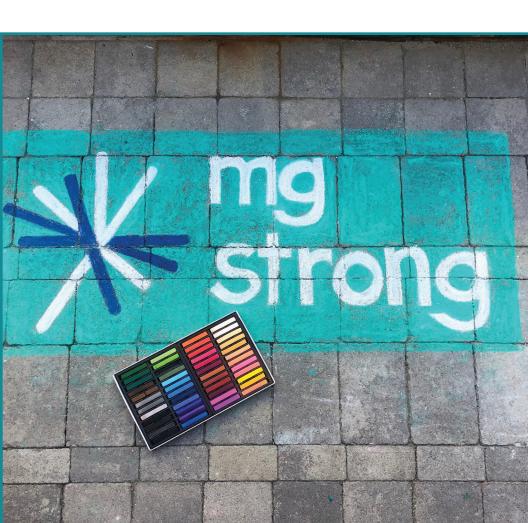
# What's New in MG Research Webinars

You're invited to join our "What's New in MG Research" webinar to learn about the latest research results, key clinical trial phases, and current outcomes from top research trials taking place right now. Visit myasthenia.org/Webinars/Whats-New-in-MG-Research

# Resources for Professionals

We offer a variety of materials for health professionals, such as webinars, manuals, diagnostic tools and other resources to become more familiar with MG. Materials are geared towards physicians, researchers, scientists, nurses, emergency medical professionals and others in the health care industry.

Visit myasthenia.org/Professionals/Resources-for-Professionals





# For a World Without Myasthenia Gravis

MGFA Programs Help Patients and Provide Guidance and Education about MG These programs and offerings showcase research and new developments in MG that educate people across the MG Community, but they are also a strong set of resources for medical professionals and healthcare providers as well.

#### MGFA Scientific Session at AANEM Conference

Join us in Nashville, Tennessee, September 21–24, 2022 and experience the MGFA 2022 Scientific Session. The MGFA Session is scheduled for Wednesday, September 21, 2022, 8:00 am – 11:30am at the AANEM meeting in Nashville, TN at the Gaylord Opryland Resort & Conference Center.

#### MGFA Wellness Webinars

MGFA has responded to the need for MG-based wellness strategies by responding with our MG Wellness Webinar Series to connect, educate, and empower MG patients, care partners, and medical professionals. Visit myasthenia.org/Webinars/Wellness-Series

# MyMG Mobile App

Recently launched to Help MG Patients Manage MG from the Palm of Their Hands MGFA recently introduced its completely brand new MyMG Mobile App for your Apple iPhone or Android phone. This state-of-the-art mobile app provides a host of new features that will enable patients to help manage and track their myasthenia symptoms and treatments while helping anyone across our community access critical MGFA resources including webinars, brochures, research information, events and blogs, and MG assistance and guidance. Visit myasthenia.org/Newly-Diagnosed/MyMG-Mobile-App

# MGFA Support Groups

Support Groups can offer MG patients and caregivers support, resources, educational programming as well as social and recreational activities.

 $Visit\ myasthenia.org/MG-Community/Find-MG-Support-Groups$ 

# MGFA Events and Conferences

MGFA events serve to bring all of us across the MG Community together – whether online or in person. We are proud to offer conferences, seminars, and wellness and research webinars as well as fundraising events such as our MG Walks. Visit myasthenia.org/Events

#### MG Friends

Our free peer-to-peer phone support program, MG Friends, helps to ensure that everyone in the community gets the information they need, and know they are not alone in living with myasthenia gravis. While Support Groups are critical to service delivery, there are many people who are not able to access this service or who feel more comfortable talking one-on-one than sharing in a group setting. MG Friends provides the opportunity for a person with questions and concerns to connect to someone else with MG—no matter where they live in the United States. Visit myasthenia.org/MG-Community/MG-Friends





