



# MG Quarterly

The e-newsletter of the MGFA

**October 31, 2018**



Through November 2 (tomorrow!), [AmazonSmile](#) is donating 5% (ten times the usual amount) to MGFA when you shop.

Make sure to select “Myasthenia Gravis Foundation National Office” as your organization to help support our work.

## RESEARCH

### **MGFA-Funded Research Projects Have Promising Impact on Future Treatment for MG**

Dr. Andrew Engel, Professor of Neurology at the Mayo Clinic, and 2018 recipient of MGFA’s High Impact Pilot Project Grant Award, will begin work on his research project, “*Genetic Basis of Unsolved Congenital Myasthenic Syndromes and Role of the AchR-CYS Loop Length in AChR Activation*,” in January 2019. His work will focus on better understanding, diagnosis and treatment of congenital myasthenic syndromes (CMS).

His research will use the candidate gene approach combined with whole exome sequencing to find the cause of different CMS, determine the mechanism by which the mutant gene causes the CMS, and then use this information to generate structure-function correlations and devise strategies for therapy.

Dr. Ruksana Huda, Assistant Professor at the University of Texas Medical Branch, provides an update on her groundbreaking MG research, funded by MGFA. The results of her study, “*Therapeutic efficacy of BAFF receptor specific mAb-siRNA conjugate in experimental autoimmune myasthenia gravis*,” have been presented at the American Association of

Immunologist (AAI) meetings in Seattle (2016) and Washington (2017). View the abstract [here](#), published in the Journal of Immunology.

An article regarding her study titled, “*High-dose BAFF receptor specific mAb-siRNA conjugate generates Fas-expressing B cells in lymph nodes and high-affinity serum autoantibody in a myasthenia mouse model*” has been published in Clinical Immunology, view the abstract [here](#).

Dr. Huda and team have also filed a patent on the novel mAb-siRNA conjugate on January 11, 2018. Research results obtained as a result of MGFA funding have led to additional multi-year support of her work from The French Muscular Dystrophy Association, [AFM-Telethon](#). The promising work of this project thus far has the potential to lead to a new therapy for MG.

## **UCB Accelerates Rozanolixizumab in MG Into Confirmatory Development Phase**

[Click here for full story](#)

UCB, a Belgium-based biopharmaceutical company, announced positive results from a phase 2 study with a subcutaneous FcRn (neonatal Fc receptor) monoclonal antibody, *rozanolixizumab*, in patients with myasthenia gravis (MG). Based on these results, UCB intends to accelerate the development of *rozanolixizumab* with a confirmatory study in MG in the second half of 2019. The full data will be presented at a medical congress in the near future and submitted for publication in a peer-reviewed journal.

## **Argenx Begins Dosing in Phase III MG Trial**

[Click here for full story](#)

Biotechnology company Argenx has started dosing patients in a Phase III clinical trial of its drug candidate *efgartigimod* (ARGX-113) to treat generalized myasthenia gravis (gMG). *Efgartigimod* is an antibody fragment being developed to help patients suffering from severe autoimmune diseases characterized by high-pathogenic immunoglobulin G (IgG) antibody levels. The therapeutic dose is designed to degrade circulating autoimmune antibodies responsible for the disease. The randomized, double-blind, placebo-controlled, multi-centre, global Phase III trial will assess the efficacy of a 10mg/kg intravenous efgartigimod dose in around 150 subjects over 26 weeks.

## **MuSK Trial Open to MG Patients**

[Click here for full story](#)

The MuSK Trial is a clinical trial of an oral, investigational medicine for the treatment of MuSK-positive myasthenia gravis (MuSK-MG). The trial is open to adults diagnosed with myasthenia

gravis, with a positive anti-MuSK antibody test or a positive anti-AChR antibody test. Trials will be held at various locations across the U.S.

## **Ra Pharma Announces Design of Phase 3 PNH Program**

[Click here for full story](#)

Ra Pharmaceuticals, Inc. announced the completion of End of Phase 2 interactions with the FDA for its global Phase 3 program of RA101495 SC, *zilucoplan*, or paroxysmal nocturnal hemoglobinuria (PNH). Ra Pharma is also developing *zilucoplan* for generalized myasthenia gravis (gMG), and other complement-mediated disorders. The product is designed for convenient, once-daily subcutaneous self-administration.

## **Alexion Pharmaceuticals Offers Programs to Learn about Generalized MG and Treatment**

Alexion Pharmaceuticals is offering a series of educational programs, titled [\*MakinGconnections\*](#), for people to learn more about their FDA-approved medication, Soliris, to treat generalized myasthenia gravis (gMG). Programs are scheduled for November and December. To see the schedule of events, click [here](#).

## **Guptill Featured by Duke Department of Neurology**

[Click here for full story](#)

Dr. Jeffrey Guptill, associate director of the Duke Early Phase Clinical Research Unit (DEPRU) and overseer of dozens of clinical trials for myasthenia gravis, Parkinson's, Duchenne muscular dystrophy, and other conditions was featured in the Duke University School of Medicine Department of Neurology "Spotlight" article. In this week's Spotlight interview, Dr. Guptill talks to us about why he is hopeful that academia and pharmacy will improve treatments for MG over the next several years. He also discusses the scope of DEPRU's work and how exercise, gardening, and diving keep him sane, centered, and engaged when he is not at Duke. Dr. Guptill was the recipient of the first post-doctorate clinical fellowship sponsored by MGFA in collaboration with the American Brain Foundation.



## **New Data Released in “Neurology Today” on Patients’ Benefits Post-Thymectomy**

[Click here for full story](#)

The new study from South Korea involved the review of medical records of 179 acetylcholine receptor antibody-positive MG patients. The study found that at the end of three years, those who had surgery had fewer symptoms and less steroid use than those that did not have surgery. Researchers from Asan Medical Center and University of Ulsan College of Medicine in Seoul reviewed the medical records of 179 MG patients who underwent thymectomy between February 1994 and March 2016. Gil Wolfe, MD, FAAN, Irvin and Rosemary Smith professor and chairman of the department of neurology at the University at Buffalo Jacobs School of Medicine and Biomedical Sciences, who led the groundbreaking 2016 study on thymectomy for MG, said that the randomized trial helped settle much of the debate around treatment in non-thymomatous generalized disease. “Until 2016 there remained some doubt about what you were getting out of thymectomy,” Dr. Wolfe said.

## **Be Part of the MG Patient Registry**

[Click here for more information](#)

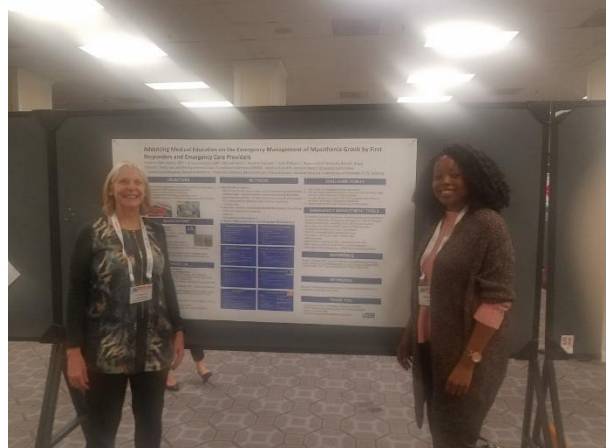
As the MGFA community continues to grow, our organization asks each one of you that resides in the U.S., that is 18 or older, to think about joining the MG Patient Registry. The registry has already made a huge difference for so many people with MG. The information you provide allows researchers to start and continue clinical trials, which lead to increased treatment options. The Myasthenia Gravis Patient Registry is an active database of individuals with myasthenia gravis (MG), developed for the purposes of research, treatment, and patient information. The registry is free to join and all data is protected by federal privacy laws.

## **ADVOCACY**

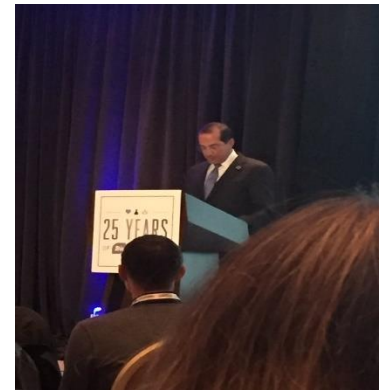
### **MGFA Team Steps Up Advocacy!**

MGFA Staff and Board members stepped up advocacy efforts throughout October by attending national meetings and events. CEO Nancy Law participated in the Muscular Dystrophy Association’s ([MDA](#)) Neuromuscular Collaborative meeting on October 14, which unites many of the groups representing people living with neuromuscular disease. This meeting is a great way for organizations to share resources and insight towards a collective goal of finding a treatment and cure for neuromuscular conditions.

Board Member, Dr. Charlene Hafer-Macko and Nakeshia Betsill, Director of Volunteer & Advocacy Programs at MGFA, attended the National Organization for Rare Diseases ([NORD](#)) Rare Summit on October 15 and 16. The summit brings together industry, nonprofit, government and patient groups together to drive progress for rare diseases. Over 700 organizations attend this annual event. During the summit, Dr. Hafer-Macko presented a poster on emergency management of myasthenia gravis. The poster focused on the development of a training program to educate emergency personnel and first responders about MG and how to handle a myasthenic crisis.



On October 25 and 26, Jessica Hughson-Andrade, National Programs Director at MGFA attended the [Bio Patient Health & Advocacy Summit](#). This summit brings together industry, nonprofit, government and patient groups to discuss timely policy issues and share best practices. Over 200 organizations attend this annual event. The U.S. Department of Health & Human Services Secretary, Alex M. Azar II, was a surprise speaker at the event, where he spoke about proposed changes to Medicare Part B prescription drug prices.



## Rare Disease Organizations Advocate for Support of OPEN ACT

Did you know that 7,000 rare diseases still have no FDA-approved treatment? One great way to ensure that people with MG and other rare diseases get access to more treatment options is to support the OPEN ACT (H.R. 1223 / S. 1509). The OPEN Act incentivizes pharmaceutical companies to repurpose existing drugs to help those with rare diseases like MG.

[Click here for more information](#)

Stay tuned for more information on Rare Disease Week on Capitol Hill 2019, which will take place in February. Rare Disease Week on Capitol Hill brings rare disease community members from across the country together to be educated on federal legislative issues, meet other advocates, and share their unique stories with legislators. More details [here](#).

Open Enrollment for health insurance begins now! From November 1 through December 15, 2018, you can purchase insurance through the marketplace. For more information, please see [www.healthcare.gov](http://www.healthcare.gov).

## **AWARENESS**

### **MG Walks Surpass \$900,000 in Fundraising**

In the most successful fundraising year to date, MG Walks have now raised more than \$935,000. Taking place annually in more than 35 cities nationwide, the MG Walk puts the power directly into the hands of MG patients, and allows everyone battling this illness to become the driving force behind funding for MG research, and ultimately, in finding a cure.

Visit [mgwalk.org/complete-list/](http://mgwalk.org/complete-list/) for the 2018 schedule. Find an MG Walk near you, participate, fundraise and spread the word. Early 2019 Walks will be posted soon.

### **Kaufman, Esmail Interviewed by San Diego CBS Affiliate on Upcoming MG Walk**

[Click here to watch the interview](#)



Author Andrew Kaufman, who served as keynote MGFA 2018 National Conference speaker, and Saadia Esmail, San Diego MG Walk Hero, were both interviewed by San Diego CBS affiliate



Channel 8 for the upcoming MG Walk on Sunday, November 18, at De Anza Cove Park. Kaufman was diagnosed with MG just three years ago following a car accident. The car accident turned out to be a blessing in disguise. “They found the tumor on my thymus, which is connected to MG. If I had not had the car accident, they never would have found it.” Saadia has MG for nearly two decades. The first signs showed up when she graduated college. “The droopiness of the eyelids. If I smiled too much, it looked like I was crying. Friends would ask, ‘are you ok?’, to which I would say I am not crying – I am laughing.” Both Andrew and Saadia hope that the MG Walk gives MG the exposure it deserves, and they are also spreading awareness through writing. Saadia is working on a memoir, and Andrew, [who has already written several bestselling books](#), will share his experience with his readers.

## Mom Fighting MG Inspires Dexter Williams' Return to Notre Dame Football



[Click here for full story](#)

“You know what,” Cheryl Williams says as a smile lights up her face, like she’s giving away a secret. “I’m one of those people that you don’t tell me when to check out. I’ll tell *you* when I’m ready to go. That’s me.”



Cheryl Williams, an MG patient since 2006, has inspired her son Dexter Williams, a running back for Notre Dame, to return to the game. Her strong spirit and tenacity in fighting MG has fueled him to return to football. The Fighting Irish are ranked No. 3 in the nation with an undefeated 7-0 record.

## Rockford Girl Inspires Millions in Viral Video

[Click here for full story](#)

#GoBella is buzzing on the internet as a Rockford girl with MG removes her own trache. She had been living with a trache since she was 1 year old after diagnosed with myasthenia gravis with apnea. “On my Instagram page alone, over 1.2 million people are liking it and viewing it,” said Bella’s dad, Justin Francis. Hours after posting, Bella’s parents received messages of support from people around the world.



One of the best parts about the video? Bella got to take the trache out herself. “I wanted to take it out by myself because I knew I was really brave and I knew the time had come that I could take it out.” Bella is working on a book about her story and looking forward to starting kindergarten in a few weeks.

## MGFA Hosts Regional Conference in Cleveland

The MGFA Cleveland Area Regional Conference, which took place on October 27, 2018, had more than 150 registered! The regional conference brought together experts to provide a full day of MG information and education on: physical therapy, exercise and MG; nutrition and the immune-compromised individual; MG and me for males and females; insurance issues; treatments; staying positive; and a panel of ask the experts. The conference was free and open to all MG patients, caregivers, researchers, family and friends. Special thanks to volunteers Rebecca Molitoris, Drs. Bob and Suzanne Ruff, and Dr. Yuebing Li for their work in organizing this meeting.

## South Florida Launches Support Group

South Florida has a new support group in Miami. They will meet on **November 7, 2018, from 6:30 to 7:30 p.m.** at the Clinical Research Building, 1120 NW 14th St., Room 1381. Although this group is still quite new, great information has been shared with a friendly, positive spirit. If you are in the area, please join them! For more details, please contact Mary Schatz at [mischatz@me.com](mailto:mischatz@me.com) or 646-573-3354.



## Illinois MG Walk Brings Awareness

[Click here for full story](#)

The Southern Illinois MG Walk held October 13, 2018, was featured on WSIL-TV. MG patient and registered nurse Paula McGinnis was interviewed. In her interview, she spoke about educating the community about MG and how to get involved in the Walk.



## Houston MG Walk featured in *The Signal* Newspaper

[Click here for full story](#)

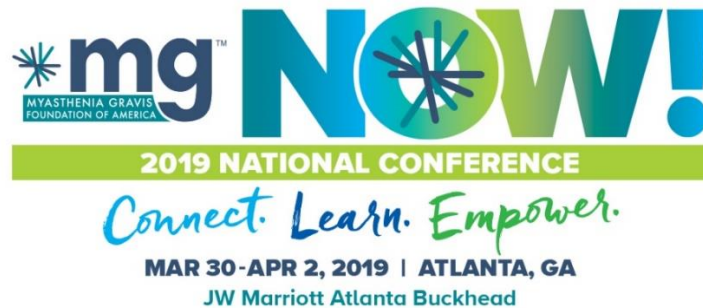
The [2018 Houston MG Walk](#) will take place **November 17, 2018**. The Walk was promoted in *The Signal*, the official student newspaper of the University of Houston-Clear Lake. It will be held at Bear Creek Pioneers Park in Houston. Meena Outlaw will be honored as the Houston MG Walk Local Hero. MG Walk is celebrating its eighth year of raising funds and awareness for MG, having raised over \$5.5 million dollars since the first walk in 2011. Funds from the MG Walk help support research initiatives, advocacy efforts, programs and services and resources for those living with MG and their families. The walk begins at 10 a.m. For further information, visit the [MG Walk website](#).

## 8<sup>th</sup> Annual New Orleans MG Walk Confirmed for Saturday, April 6, 2019

The 2019 MG New Orleans Walk date is confirmed for **April 6, 2019!** Please save the date to help us raise awareness, raise funds and ultimately find a cure for myasthenia gravis. Stay tuned to the MG Walk [website](#) to learn about a Walk near you in 2019.

# MGFA COMMUNITY NEWS

## SAVE THE DATE! 2019 National Conference in Atlanta



The **2019 National Conference** and **National MG Walk** will be held at the JW Marriott in Atlanta, Georgia from **March 30 through April 2**. Registration coming soon, please stay tuned via our [website](#)!

### Well-Known Ophthalmologist Leads Webinar on Ocular MG

Dr. Rudrani Banik, board certified ophthalmologist and associate professor of ophthalmology at Mount Sinai Icahn School of Medicine, led an “Ocular MG” webinar on October 25. Dr. Banik has 13 years of training at some of the finest medical and eye institutes in the country. She has served as full-time faculty at the Albert Einstein College of Medicine, Bronx-Lebanon Hospital Center, and the New York Eye and Ear Infirmary of Mount Sinai. Dr. Banik is sought out as an expert in the media and has been featured in *The New York Times*, *Good Morning America*, *CBS Evening News*, *ABC 7 Eyewitness News*, *CBS NY with Dr. Max Gomez*, *Fox 5 News with Ernie Anastos*, *NY Magazine’s Best Doctors 2017 and 2018*, *The Washington Post*, *New Tang Dynasty TV*, and *Real Simple* magazine.



### “MG & You” Webinar on November 13

Our upcoming webinar titled, “**MG & You**,” presented by Dr. Raghav Govindarajan, will discuss how MG can affect different aspects of your life and what you can do about it. Dr. Govindarajan is the associate medical director of the neurology outpatient clinic, MDA/ALS clinic director and EMG/neurophysiology lab director at University of Missouri. He is board certified in neurology and neuromuscular medicine. He runs a large MG clinic through a

multidisciplinary team, in addition to a support group for MG patients through MGFA. He has many peer-reviewed publications and more than 30 teaching awards. He has been inducted into Alpha Omega Alpha, Gold Humanism Honor Society, Sigma Xi. He is an elected fellow of the College of Physicians of Philadelphia and was honored with Leonard Tow Humanism award by Arnold P. Gold Foundation in addition to the Schwartz Compassionate Caregiver award for the care of his patients.

The “MG & You” webinar will take place on **November 13 at 8pm eastern**. [Click here to register](#)

Stay tuned for more webinars in November and December!

## **MAYA to Host Young Adult Virtual Support Group Series**

The MGFA’s Myasthenia Advocacy for Young Adults (MAYA) group will hold a virtual support group series with two dates scheduled before the end of the year – **“Managing School with Myasthenia Gravis,” November 7 at 8 p.m. eastern;** and **“Starting a Family & Parenting with Myasthenia Gravis,” December 5 at 8 p.m. eastern.** The seminar series is open to MG patients 18 to 35 years old. [Click here to register](#)