



# MG Quarterly

The e-newsletter of the MGFA

**March 2018**

## **MGFA News**

### **Registration Open for 2018 National Conference; Author Andrew Kaufman to Serve as Keynote Speaker**

Registration is officially open for the 2018 National Conference, being held at the Intercontinental Kansas City at the Plaza from April 15-17 in Kansas City, MO.

Known as the “City of Fountains,” Kansas City is an exciting, hidden gem in our nation’s heartland and is famous for its barbecue, fountains, jazz, creative arts scene, sports and more. With Kansas City International Airport less than 25 miles from downtown and offering extensive service from Delta and Southwest Airlines, our location at the Intercontinental Kansas City at the Plaza couldn’t be better. The hotel is an easy walk to Country Club Plaza, a 15-block district with more than 150 shops and dozens of fine restaurants. Other major attractions in town are just a short cab ride away. For more about KC visit [www.visitkc.com](http://www.visitkc.com) and learn more!

The National MG Conference is the largest gathering of the MG Community in the U.S. This year, we expect more than 200 participants will gather to learn, share and socialize with others who understand MG personally and professionally. Content includes understanding MG and its treatment but also coping with its affects and issues in managing one’s life.

For 2018 our Keynote Speaker will be author [Andrew Kaufman](#), whose novels have been on the Top 100 lists for months, and whose book, *The Lion, the Lamb and the Hunted*, became an international bestseller. Andrew also has MG. Andrew’s thrillers have entertained and inspired fans worldwide.

Our presenting sponsor is Alexion Pharmaceuticals, Inc. the maker of Soliris (eculizumab) the recently FDA approved therapeutic for those with refractory MG. Soliris is the only therapeutic approved specifically for gMG since Mestinon was approved in 1955.

The conference will begin at 1:30 pm on April 15, and conclude by 2 pm on April 17. (Continued)

#### **Important MG National Conference Links:**

- [Register for the 2018 National Conference](#)
  - [Register by March 20<sup>th</sup> to get the best room rate](#)
- [Book your hotel at the Intercontinental Kansas City at the Plaza.](#) [Register by March 20<sup>th</sup> to get the discounted room rate.](#)

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- [Sign up for the National MG Walk on April 14](#)
- [Delta Airlines Discount](#) - Taking advantage of this discount is easy! Just click the “Advanced Search” link on [www.delta.com](http://www.delta.com) and enter NMRCL in the box that says “Meeting Event Code.” If your flight qualifies for a discount, it will automatically be applied to your search results.

Make it an even better experience by joining the Walk on April 14th. This is a chance for all who don't have a Walk in their own communities to form or join a team. Team Duck Tapers, comprised of Facebook friends from around the country, was the top team at our New Orleans Walk in 2017, and they are getting organized already. So, plan to come for both the Walk and the Conference. And, the **National MG Walk** will take place on **Saturday, April 14th**, starting at **10 a.m.** For more details and to register, please visit <http://www.mgwalk.org/national/>. We hope many will form or join a team and fundraise for the fight against MG!

For more information about the conference, such as how to register, how to book your room at the MGFA room rate and how to receive a special discount code for your flight, please [click here](#).

## **MG Walk 2018 Season Kicks Off; More Than \$150K Raised Towards \$900K Goal**

The MG Walk Campaign, dedicated to creating awareness, renewing hope, and generating a vast network of community and support, all the while raising important funds for the Myasthenia Gravis Foundation of America (MGFA), has kicked off its 2018 season with more than \$150,000 raised towards the \$900,000 goal. The MGFA would like to thank several national sponsors for the 2018 MG Walks, including Alexion, Brivova, Kroger, NuFactor, Ra, and Pro Health Services. The 2018 National MG Walk Hero was announced in Lauren Jarman, a patient from South Carolina, while the 2018 National MG Walk Medical Ambassador was announced in Dr. Gavin Brown, a neurologist in Georgia.

Several of the 2018 MG Walks have been scheduled, including:

- 2018 New Orleans MG Walk      Saturday, March 3, 2018      Lafreniere Park, Metairie, LA
- 2018 Tampa Bay MG Walk      Saturday, March 10, 2018      Rowlett Park, Tampa, FL
- 2018 Georgia MG Walk      Saturday, March 10, 2018      Brook Run Park, Dunwoody, GA
- 2018 Tallahassee MG Walk      Sunday, March 11, 2018      Cascades Park, Tallahassee, FL
- 2018 South Florida MG Walk      Sunday, March 11, 2018      Tradewinds Park, Coconut Creek, FL
- 2018 North Carolina MG Walk      Saturday, April 7, 2018      Freedom Park, Charlotte, NC
- 2018 National MG Walk      Saturday, April 14, 2018      Intercontinental Kansas City at the Plaza,  
Kansas City, Missouri (in conjunction with the MGFA National Conference)
- 2018 Northern Wisconsin MG Walk      Saturday, May 5, 2018      Green Isle Park Pavilion, Allouez, WI

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- 2018 Southern Wisconsin MG Walk      Saturday, May 5, 2018      Sheridan Park, Cudahy, WI
- 2018 South Carolina MG Walk      Saturday, May 19, 2018      North Charleston Wannamaker County Park,  
North Charleston, South Carolina
- 2018 Tri-State MG Walk in New York      Saturday, June 9, 2018      Hunter's Point South Park Queens, NY
- 2018 Connecticut MG Walk      Sunday, June 10, 2018      Hubbard Park, Meriden, Connecticut

Visit [mgwalk.org](http://mgwalk.org) for continued updates and information.

## **Fundraising Your Way through DIY!**

Raising money for the Myasthenia Gravis Foundation of America has never been easier or more rewarding! With **Do It Yourself Fundraising**, anyone can design a fundraising event or activity that builds on their passions and interests. You name it! Love the idea of an endurance event? Getting ready to celebrate a special occasion like a milestone birthday, anniversary or Bar/Bat Mitzvah? Do you just need a little motivation to host a themed party? No matter your interest, **DIY Fundraising** is your way to bring us closer to a world without MG. And best of all, while raising money for MG, you create more awareness helping those living with this rare disease.

To learn more and to set up your personal fundraising campaign go to <https://give.classy.org/MGFA> or contact Betty Ross, Development Director, at [brross@myasthenia.org](mailto:brross@myasthenia.org)

## **MG and MG Patients in the Media**

### **Minnesota Golden Gophers' Hockey Player Describes Her Battle with Myasthenia Gravis**

University of Minnesota Golden Gophers' junior [Taylor Williamson](#) caught up with the voice of Gopher Women's Hockey Dan Hamann in a radio interview to discuss what it was like to return to the Gophers' lineup after missing 23 games and her battle with a neuromuscular autoimmune disease called myasthenia gravis. [Click here to listen to interview and read the transcript](#)

### **Texas State Representative Mentions MG in Re-Election Interview**

Austin, Texas, native Dawnna Dukes, who was elected to the Texas House of Representatives in 1996, has filed for re-election and sat down with KVUE-ABC TV to discuss her bid for re-election. In her interview, Dukes mentions injuries and a car wreck that caused her to develop full-blown multiple sclerosis and myasthenia gravis. [Click here to read more](#)

## Mayo Clinic Thoracic Surgeon Discusses Thymus Gland

Dr. Stephen Cassivi, a thoracic surgeon at Mayo Clinic, talks about the thymus gland, specifically thymomas and myasthenia gravis, in an interview on *The Progress* website, headquartered in Pennsylvania. [Click here to watch interview](#)

## MG Research and Clinical Trials

### NEWS ABOUT MG CLINICAL TRIALS: THREE NEW TRIALS ACTIVELY RECRUITING!

We at MGFA are excited to provide information about three different clinical trials that are open and actively recruiting participants with MG. The information on these trials has been provided by the companies to MGFA. For information on all trials in MG, please go to [www.clinicaltrials.gov](http://www.clinicaltrials.gov). To watch a video about participating in clinical trials from the 2016 MG National Conference, go to: [Clinical Trials Talk](#)

#### **RA101495**

A new Phase 2 trial for generalized myasthenia has been initiated and examines the experimental drug RA101495.

RA101495 is self-administered subcutaneously daily and works by preventing the body's attack on neuromuscular junctions. It does this by blocking a component of the body's immune system called the complement system.

The main purpose of this study is to test the effectiveness of RA101495 for the treatment of patients with AChR positive MG. Treatment effects will be measured across two MG-specific assessment scales: the Quantitative Myasthenia Gravis (QMG) and the Myasthenia Gravis – Activities of Daily Living profile (MG-ADL), along with other clinical tests. In addition, this study will also look at safety and how well patients feel while on treatment with RA101495.

The trial will last approximately 3 months and will include approximately 7 visits. Patients will also have the possibility to continue receiving the study drug after they complete the study.

The trial is taking place at 30 trial sites across the United States and approximately 5 sites in Canada, and support for travel costs may be available.

Eligibility: 18 to 85 years of age

To learn more about this trial, including a list of study sites, reach out to [trials@raphama.com](mailto:trials@raphama.com) or call 617-401-4060. For information about inclusion/exclusion criteria visit [Clinical Trials.gov - RA101495](#)

Sponsor: Ra Pharmaceuticals

#### **UCB7665**

This is a study to test the safety, tolerability and efficacy of UCB7665 (a subcutaneous infusion) in subjects with moderate to severe generalized myasthenia gravis. Study duration for participants is 18 weeks.

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Eligibility: 18 years old or older

**[Click here to see if you qualify.](#)**

The company will be completing recruitment in the next few months.

For more information go to: [Go to Clinical Trials.gov - UCB7665](#)

Or contact: [1-844-599-2273](tel:1-844-599-2273) / [UCBCares@ucb.com](mailto:UCBCares@ucb.com)

Sponsor: UCB Biopharma

## **AMIFAMPRIDINE PHOSPHATE**

The purpose of this study is to determine the efficacy and safety of amifampridine phosphate in improving the activities of daily living for patients with antibody positive MuSK myasthenia gravis.

This is a randomized, double-blind, placebo-controlled, parallel group study. In addition, a sample of AChR-MG patients will be assessed for efficacy and safety of amifampridine phosphate. Planned duration of participation for each patient is at least 38 days, excluding the screening period. Eligible patients will be titrated to an efficacious dose of amifampridine phosphate and those who demonstrate improvement will be randomized to either placebo or amifampridine, in a double-blind fashion, for 10 days.

Eligibility: 18 years old or older.

For more information and inclusion/exclusion criteria see [Clinical Trials.gov - Amifampridine Phosphate](#). To contact Catalyst send message to [MuSKMG@catalystpharma.com](mailto:MuSKMG@catalystpharma.com) or call toll free 1.844.347.3277

Sponsor: Catalyst Pharmaceuticals

## **Other Health News and Studies**

### **Managing Your Energy**

The University of Washington Healthy Aging Rehabilitation Research Training Center (RRTC) has released a fact sheet entitled "[How to Do a Lot with the Little: Managing Your Energy.](#)" The fact sheet describes various types of fatigue, identifies how fatigue affects our daily lives, and provides tips for reducing fatigue.

Funded by the [National Institute on Disability, Independent Living, and Rehabilitation Research \(NIDILRR\)](#), this RRTC focuses on promoting healthy aging for people with long-term physical disabilities. The RRTC brings together a group of rehabilitation researchers, physicians, psychologists and public health professionals and who carry out research and training activities to better understand the challenges faced by people aging with multiple sclerosis, muscular dystrophy, post-

polio syndrome, and spinal cord injury.

## **Pyridostigmine (Mestinon) Reverses Complications from Botox Treatment of Muscle Disorders**

Physicians at the Medical University of South Carolina (MUSC) report the first pediatric use of a treatment to reverse complications from botulinum toxin therapy. Complications from botox treatment of muscle disorders were reversed when caught early, according to the findings of a study published online ahead of print by *The Journal of Pediatrics* on December 22, 2017. In the study, physicians used pyridostigmine to treat one pediatric patient experiencing immediate complications from botulinum toxin therapy and another with delayed complications in distant muscles. In both cases, physicians recognized complications early and treated patients with the maximum dose of pyridostigmine appropriate for their weight. [Click here to read more](#)

## **News from the MGFA/NYAS 13<sup>th</sup> International Symposium**

Myasthenia Gravis Foundation of America (MGFA) is pleased and proud to see the *Annals of the New York Academy of Sciences* January and February issues, *NYAcademyAnnals*, covering the MG related science presented at the 13<sup>th</sup> International Symposium produced by the MGFA and the Academy in May 2017. These items as seen in *Myasthenia Gravis News* highlight a few of the many informative presentations offered at the symposium, which helps to advance the knowledge of the international community working to understand and treat MG. For *Muscle Cell Receptor Component Increases Myasthenia Gravis Treatment's Punch, Study Shows* go here: [MG News 1](#); for *2 Autoantibodies Identified as Potential New Biomarkers for Myasthenia Gravis, Researchers Say* go here: [MG News 2](#).

## **News from the Public Sector**

**U.S. House of Representatives** [www.congress.gov](http://www.congress.gov)

**U.S. Senate** [www.senate.gov](http://www.senate.gov)

**Health and Human Services (HHS)** [HHS.gov](http://HHS.gov)

## Centers for Medicare and Medicaid Services (CMS)

Starting April 2018, CMS will begin mailing new Medicare cards to all people with Medicare on a flow basis, based on geographic location and other factors. Also starting April 2018, patients will be able to check the status of card mailings in their area on [Medicare.gov](https://www.medicare.gov).

CMS is required to remove Social Security Numbers (SSNs) from all Medicare cards by April 2019. A new, unique Medicare Number will replace the SSN-based Health Insurance Claim Number (HICN) on each new Medicare card. Starting April 2018, CMS will begin mailing new Medicare cards to all people with Medicare on a flow basis by geographic location and other factors.

For More Information click on [Mailing Strategy](#). To address questions from patients, click on [Guidelines](#). To view Medicare Card, click on [overview](#) and [provider](#) webpages.

## National Institutes of Health

The *NIH News in Health* is published monthly to provide practical health news and tips based on the latest NIH research. The January issue contains a collection of popular stories about aging and senior health from past issues has been reviewed and updated. Two notable topics are [Coping with Caregiving: Take Care of Yourself While Caring for Others](#) and [Talking With Your Doctor: Make the Most of Your Appointment](#).

## U.S. Food and Drug Administration (FDA)

On December 18th, FDA conducted a public workshop to discuss methodological approaches that individuals may use when seeking to collect patient experience data for submission to FDA to inform regulatory decision-making. The workshop focused on topics related to collecting comprehensive and representative patient and caregiver input on burden of disease and current therapy. This workshop was intended to inform development of patient-focused drug development guidance as required by the [21st Century Cures Act](#) and to meet a performance goal included in the [sixth authorization of the Prescription Drug User Fee Act \(PDUFA VI\)](#).

The purpose of this public workshop was to obtain feedback from stakeholders on consideration for:

1. Standardized nomenclature and terminologies for patient-focused drug development
2. Methods to collect meaningful patient input throughout the drug development process
3. Methodological consideration for data collection, reporting, management, and analysis of patient input

FDA sought information and comments from a broad range of stakeholders, including patients, patient advocates, academic and medical researchers, expert practitioners, drug developers and other interested persons. For information published prior to the meeting, click on

<https://www.fda.gov/Drugs/NewsEvents/ucm574725.htm> This website will be updated as workshop materials are developed.

## **Centers for Disease Control and Prevention (CDC)**

### **National Center for Chronic Disease Prevention and Health Promotion**

The National Center for Chronic Diseases Prevention and Health Promotion released [Tips for Caregivers](#) providing a checklist for caregivers to communicate with their care recipient's doctor. Suggestions include questions and preparation before, during and after the doctor appointment. CDC also announced that the National Alliance for Caregiving released a [Report on Family Caregiving](#). The report, *From Insight to Advocacy: Addressing Family Caregiving as a National Public Health Issue*, describes the major caregiving issues affecting public health. It includes potential action items for state and local coalitions, health systems, and others. The report identifies common barriers to providing care within the aging network and offers strategies for caregiving advocate engagement. The report also features CDC data collection on caregiving with the Behavioral Risk Factor Surveillance System's (BRFSS) Caregiving Module.

## **Department of Labor (DoL)**

On January 30, the DoL Bureau of Labor Statistics (BLS) issued a report identifying occupations that are likely to experience the most job growth across the United States between 2016 and 2026. Among the five occupations requiring a bachelor's degree that will have strong job openings over the next decade, "Registered Nurse" jobs are projected to increase by 437,000, a 15 percent increase over the current number. The BLS report stated, "There will be an ongoing emphasis on preventive care. Growing rates of diabetes, obesity and other chronic conditions, and baby-boomers who are living longer than previous generations are all driving the need for registered nurses." To view the report, click on [Occupations with the most job growth - Bureau of Labor Statistics](#)