



MGFA December 2017 E-Update

MGFA News

Dear Readers: Happy New Year! We wish you the best of everything in 2018. Yours, MGFA

MG Walks Close in on Goal of \$900K as 2017 Comes to Close

The 2017 MG Walk season has been the most successful since the Campaign kicked off in 2011, raising more than \$850,000 this year alone. Since the inception of the MG Walk, more than \$5 million has been raised to support MGFA's efforts to fund research, provide patient services, raise awareness, advocate on behalf of people who have MG and ultimately find a cure.

In order to make a last minute donation to the 2017 MG Walk or for more information on the 2018 MG Walk, please visit MGWalk.org or call 1-855-649-2557 for more information.

HAVE YOU HEARD THE NEWS?!
REGISTRATION IS NOW
OPEN!

WISCONSIN
MAY 5th, 2018

NATIONAL WALK
KANSAS CITY
APRIL 14th, 2018

GEORGIA
MARCH 10th, 2018

NEW ORLEANS
MARCH 3rd, 2018

TAMPA BAY
MARCH 11th, 2018

TALLAHASSEE
MARCH 12th, 2018

SOUTH FLORIDA
MARCH 11th, 2018

NORTH CAROLINA
APRIL 7th, 2018

SOUTH CAROLINA
APRIL 7th, 2018

MG walk
Myasthenia Gravis
Foundation of America

MORE 2018 WALKS LAUNCHING SOON!

MG Research and Clinical Trials

MG Treatment Gets First FDA Approval Since 1950s

For more than 60 years, steroids and acetylcholinesterase inhibitors have been widely used as therapeutic options for patients with myasthenia gravis (MG), mostly because alternative means of immunotherapy have always been associated with serious adverse events (AEs). Alexion Pharmaceuticals, Inc. announced that the U.S. Food and Drug Administration (FDA) has approved Soliris (eculizumab) as a new treatment for adult patients with generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody-positive. The drug exhibited clinical benefit for these patients who had previously failed immunosuppressive treatment and continued to suffer from significant unresolved disease symptoms. [Click here to read more.](#) See the Neurology Advisor article here: [Click here to read more](#)

Clinical Trial Begins for Firdapse

A new clinical trial to determine the efficacy and safety of amifampridine phosphate in improving the activities of daily living for patients with antibody positive MuSK myasthenia gravis has opened. [Click here](#) to read more about the study.

Belgian Antibody Proves Effective to Treat Myasthenia Gravis

Argenx has released the results from a Phase II trial with ARGX-113 (efgartigimod), in which the antibody therapy has proved to provide a significant improvement in patients with generalized myasthenia gravis. A total of 24 patients were recruited for the trial. They all received the standard care for myasthenia gravis (corticosteroids and/or immunomodulatory agents) in addition to four weekly doses of either ARGX-113 or placebo. After 6 weeks, 75% of patients treated with ARGX-113 showed a significant improvement in their symptoms, compared to just 25% of those receiving the placebo. A clear improvement was seen starting from the first week after the first infusion. [Argenx Press Release/](#)

Phase 1 Trial of GTP-004 Initiated as Therapy for Myasthenia Gravis Treatment Side Effects

GT Biopharma has launched a proof-of-concept Phase 1 trial of its investigational therapy GTP-004 for the treatment of MG. The trial's primary goal is to demonstrate the therapy improves the gastrointestinal side effects of current treatments. GTP-004 is a fixed-dose combination tablet of

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two approved therapies – Mestinon (pyridostigmine) and an antagonist to Mestinon’s gastrointestinal side effects.

New RA 101495 Clinical Trial

Ra Pharmaceuticals is recruiting participants for a new clinical trial. This Phase 2 Study seeks to evaluate the safety, tolerability, and preliminary efficacy of RA101495 in subjects with generalized myasthenia gravis. Administered subcutaneously (an injection under the skin), RA101495 is designed to prevent the body’s attack on the space across which nerve fibers transmit signals to muscle fibers, called the neuromuscular junction. The trial will last approximately 3 months and will include approximately 7 visits. It is taking place at 21 sites across the country. Patients will also have the possibility to continue receiving the study drug after they complete the study. For more information about the trial, visit [MGFA Clinical Trials](#) and/or visit clinicaltrials.gov.

More on Clinical Trials

UCB is currently running a clinical trial to **Test the Safety, Tolerability and Efficacy of UCB7665 in Subjects with Moderate to Severe Myasthenia Gravis**. To learn more on this and other clinical trials for MG visit the MGFA website at [MGFA Clinical Trials](#) or go to clinicaltrials.gov

MG and MG Patients in the Media

The Assistance Fund Opens Copay Program for Individuals with Myasthenia Gravis

The Assistance Fund, an independent charitable patient assistance foundation that helps patients and families facing high medical out-of-pocket costs, announced the launch of a program to provide financial support to patients with myasthenia gravis. “Perhaps there is nothing more frustrating for patients with a life altering disease like myasthenia gravis (MG) than knowing that there are treatments that can help, but not being able to afford them,” said Nancy Law, chief executive officer for the Myasthenia Gravis Foundation of America (MGFA), who herself has had MG for more than 20 years. “We at MGFA are grateful that The Assistance Fund will be able to help many patients with copays, deductibles, health insurance premiums and other medical expenses –

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allowing them to pursue optimal care without undue financial burden.” Support for medication/treatment only includes FDA approved therapies, that is Edrophonium chloride; Enlon; Mestinon; Neostigmine bromide; Prostigmin; Pyridostigmine bromide; and Soliris. Treatments such as IVig; PLEX; Cellcept and others cannot be covered. [Click here to read more](#)

Walk Hero Leah De La Torre Featured in Newspaper

Fallbrook resident Leah De La Torre, MG Walk Hero of the Greater Los Angeles MG Walk on Nov. 18 in Santa Monica, was featured in the *Fallbrook & Bonsall Village News*. Last year, De La Torre was too weak to participate and watched as her husband and a cousin took part in the walk. This year, her husband pushed her in her new wheelchair as part of a nine-person team. [Click here to read more](#)

California Walk Featured in the News

The 2017 Inland Empire MG Walk was featured in *The Press-Enterprise*. The walk took place Sunday, Nov. 19, at Menifee’s Spirit Park. At this year’s event, Esther and David Bolger were honored as Inland Empire MG Walk Local Heroes. [Click here to read more](#)

Nebraska Patient Writes Article to Promote Walks, Donations

MG Patient Jon Sass authored an article promoting the need for donations to the MGFA, and his brother and sister-in-law Bert and Sharon Sass, participated in the 2017 Arizona MG Walk in honor of him. [Click here to read more](#)

Lyme Disease Sufferer, Originally Diagnosed as Having MG, Launches Podcasts

Nurse practitioner and Lyme disease sufferer Cindy Kennedy decided this summer to create a website with podcasts to share information about her long-term experience with the disease that can be difficult to diagnose and treat in some people. She was originally diagnosed with MG because of double vision. The treatment left her with "debilitating" fatigue. She had to decrease her work hours, could no longer do the exercise regime that had once "invigorated" her and her physical and emotional changes impacted her family as well. Kennedy said she was "finally diagnosed" in 2015 with what she terms chronic Lyme disease, but said disease symptoms, which can mimic other conditions, manifested much earlier though standard Lyme tests did not show Kennedy positive for the disease. Metrics show the podcasts have doubled in downloads each month and have been listened to in 17 countries. [Click here to read more](#)

Paula McGinnis TV Interview about MG, MGFA, and Southern Illinois MG Walk

On October 12, Ms. McGinnis was interviewed by Stefanie Martinez on WPSD about the MG Walk in Metropolis, IL, the home of “Superman” played in the 1950’s television show by George Reeves, the first MGFA MG ambassador. She also described the work of the MGFA to fund research, awareness and patient services and her experiences as a person with MG. To view the interview, click on

[INTERVIEW: Walking for Myasthenia Gravis Research](#)

MG Research

Study shows that cognitive therapy could help MG patients with anxiety, depression or insomnia

A new study urges consideration of Cognitive Behavioral Therapy (CBT) as a first-line treatment for myasthenia gravis (MG) patients who also suffer from anxiety, depression, or insomnia disorders. The study, “[Cognitive-Behavioral Therapy for Psychiatric Comorbidity in a Case of Muscle-Specific Kinase–Positive Myasthenia Gravis](#),” appeared in the journal *The Primary Care Companion for CNS Disorders*. The post, [Cognitive Behavioral Therapy Could Help MG Patients with Anxiety, Depression or Insomnia](#) appeared first on *Myasthenia Gravis News*, which stated that MG patients frequently have depression and anxiety. Medications may help, though some patients suffer adverse events. CBT, an evidenced based type of psychotherapy, can be used to treat depression, anxiety and insomnia without harmful side effects. CBT’s goal is to critically examine and change the thinking or behavior behind a patient’s difficulties. For more on CBT [Click Here](#)

Study: Relatives of individuals with myasthenia gravis have a higher risk of developing the disease

Researchers in Taiwan have found that first-degree relatives of individuals with myasthenia gravis have a higher risk of developing the disease. The risk is greater for patients’ siblings, the study found. The study, “[Familial aggregation of myasthenia gravis in affected families: a population-based study](#),” appeared in the journal *Clinical Epidemiology*. In this study, authors evaluated the familial aggregation and heritability of myasthenia gravis and the relative risks of other autoimmune diseases in the relatives of patients. They performed a population-based family study using the Taiwan National Health Insurance (NHI) Research Database

Other Health News and Studies

New Study Examines Use and Cost of Orphan Drugs

Orphan drugs accounted for only 7.9% of total drug sales in the U.S. in 2016, according to a [study](#) issued on October 17 by the Quintiles IMS Institute. The study, commissioned by the National Organization for Rare Disorders (NORD), analyzed the role of the Orphan Drug Act and orphan drug usage and costs, to address a number of public policy and health care financing issues in the rare disease community. To view the report, click on [New Study Examines Use and Cost of Orphan Drugs](#)

Myasthenia Gravis and Crisis: Evaluation and Management in the Emergency Department

To make it easier for fellow physicians in the emergency room to handle patients who develop a myasthenic crisis, a research team at the [University of Texas Southwestern Medical Center](#) authored a review, presenting available data of its diagnosis and treatment. Reviewers stated that myasthenic crisis is a common complication in patients with myasthenia gravis (MG), marked by exacerbation of MG symptoms and respiratory failure. But with the rarity of MG, and its similarity to a range of other neuromuscular diseases, they argue that proper detection and treatment might fail. The review, "[Myasthenia Gravis and Crisis: Evaluation and Management in the Emergency Department.](#)" was published in the [Journal of Emergency Medicine](#).

Study of Drug Interactions

On November 29 the University of Michigan Institute for Healthcare Policy and Innovation issued results of its poll of nearly 1,700 adults ages 50 to 80. The poll revealed that, while many older Americans take multiple medications, only about one-third ever discuss possible interactions between drugs. "Interactions between drugs, and other substances, can put older people at a real risk of everything from low blood sugar to kidney damage and accidents caused by sleepiness," said Dr. Preeti Malani, who directed the nationwide poll that was sponsored by AARP. "At the very least, a drug interaction could keep their medicine from absorbing properly," said Malani, a professor at the University of Michigan Medical School. More than 60 percent of respondents said they believe their doctor and pharmacist are equally responsible for identifying

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and talking about possible drug interactions. A full report of the findings and methodology is available at www.healthyagingpoll.org.

Study of Drug with Potential to Treat Autoimmune Diseases

A University of Colorado Boulder study, funded by the National Institutes of Health discovered a drug-like compound that they predict could someday revolutionize treatment of rheumatoid arthritis and other autoimmune diseases by inhibiting a protein instrumental in prompting the body to start attacking its own tissue. “We have discovered a key to lock this protein in a resting state,” said Hang Hubert Yin, a biochemistry professor in the [BioFrontiers Institute](#) and lead author of a [paper](#), published on November 20, 2017 in *Nature Chemical Biology*, describing the discovery. “This could be paradigm shifting. “More than 23.5 million Americans suffer from autoimmune diseases like rheumatoid arthritis, scleroderma and lupus, in which an overzealous immune response leads to pain, inflammation, skin disorders and other chronic health problems. Three of the top five selling drugs in the United States aim to ease their symptoms. But no cure exists, and treatments are expensive and come with side effects. “Given the prevalence of these diseases, there is a big push for alternatives,” Yin said.

News from the Public Sector

Health and Human Services (HHS) HHS.gov

Health Resources and Services Agency (HRSA)

Increasing Access to Clinicians in Rural Areas

On October 19 HRSA announced it had granted nearly \$282 million in awards to primary health care clinicians and students through the [National Health Service Corps \(NHSC\)](#) and [NURSE Corps](#) Scholarship and Loan Repayment Programs. “Through NHSC and NURSE Corps scholarships and loan repayment for more than 12,000 recipients, we’ve helped communities recruit and retain quality primary care clinicians committed to providing care where they’re needed most,” said HRSA Administrator George Sigounas, MS, Ph.D. “In rural communities facing higher rates of provider shortages, the NHSC is making an impact with one in three NHSC clinicians serving in a rural area.” [Read the news release](#)

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U.S. Food and Drug Administration (FDA)

Disposal of Unused Medicines

On October 25 the FDA published a consumer update titled *How and Where to Dispose of Unused Medicines*. The FDA wrote, “Is your medicine cabinet full of expired drugs or medications you no longer use? Your medicine is for you. What’s safe for you might be harmful for someone else. You can dispose of your expired, unwanted, or unused medicines through a drug take back program — or you can do it at home.” For details, click on [Consumer Updates](#)

Administration for Community Living (ACL)

Caregiver Support

November 2017 was National Caregivers Support Month. However, caregiving continues every month of the year and caregivers need support on an ongoing basis. For the past 17 years, the National Family Caregiver Support Program (NFCSP) has anchored ACL’s efforts to support family caregivers of older adults, persons with Alzheimer’s disease and related disorders, and older relatives caring for children. The NFCSP serves more than 750,000 family caregivers annually, providing them with: information; assistance with accessing services and supports; counseling, training and support groups; respite; and supplemental goods and services like transportation, home modifications and medical equipment. For more information click on [Caregiver Support](#)

National Institute on Disability, Independent Living, and Rehabilitation Research (NIDILRR)

People with Long-Term Physical Disability Have a Lot to Share about Successful Aging

The National Rehabilitation Information Center (NARIC) is the library of the **National Institute on Disability, Independent Living, and Rehabilitation Research (NIDILRR)**. The October 25 edition of the **NARIC** weekly digest of new research, *Research in Focus*, described the work of the University of Washington Healthy Aging Rehabilitation Research and Training Center (RRTC) on “Healthy Aging with Disabilities.” The RRTC held focus groups with 49 adults age 45-80 with physical disabilities who had muscular dystrophy, multiple sclerosis, post-polio syndrome, or spinal cord injury. The researchers asked participants to explain what “successful aging” meant to them, as well as the qualities and resources they had in their lives that helped them to age successfully. The ideas that participants mentioned most often were:

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1. Resilience and adaptability: The participants described having a positive outlook and adapting to changes in their lives as being important for successful aging.
2. Autonomy and choice: The participants felt it was important to have control over life decisions, such as choosing where to live and what adaptive equipment to use.
3. Social connectedness: The participants described having strong relationships with others, such as spouses and friends.
4. Physical health and access to healthcare: The participants described the importance of maintaining their physical wellness, so they could keep participating in valued life activities, like working or pursuing hobbies.

The authors noted that successful aging can be enhanced by social and community resources. Participants mentioned resources such as support groups for people to meet others with their same medical condition, community wellness programs for older adults, and in-home supports enabling people to “age in place” and take charge of their living situation. Go to www.myasthenia.org to learn about MGFA support groups throughout the country.

To read more about the study, click on [Learn more here](#). To view Healthy Aging RRTC factsheets and research briefs on aging with physical disabilities, click on <http://agerrtc.washington.edu/>

National Academies of Science, Engineering, Medicine (NASEM)

Making Medicines Affordable

The NASEM released a new report in November, “Making Medicines Affordable: A National Imperative.” In announcing the release, the NASEM stated that over the past several decades, the biopharmaceutical sector in the United States has been successful in developing and delivering effective drugs for improving health and fighting disease, and many medical conditions that were long deemed untreatable can now be cured or managed effectively. NASEM further stated that at the same time, spending on prescription drugs has been rising dramatically, to the point that many individuals have difficulty paying for the drugs that they or their family members need. The report recommends actions aimed at improving the affordability of prescription drugs without discouraging continued innovation in drug development. The report also looks at related areas including the role of generics and biosimilars, intellectual property, financial transparency, drug advertising, as well as insurance benefit designs. To view Report Highlights, click on [\(HTML\)](#) To view the Report Recommendations, click on [\(HTML\)](#)

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Accessible Air Travel Update

FAA Reauthorization – Disability Provisions

Congress has voted to extend the Federal Aviation Administration's (FAA) operating authority until March 31, 2018, which represents a six-month extension. The extension, formally called the Disaster Tax Relief and Airport and Airway Extension Act of 2017 ([HR 3823](#)), funds FAA operations at its current funding level and includes none of the proposed provisions in the full reauthorization bills that would increase access to air travel for passengers with disabilities. The exclusion of these provisions was expected, however, as a simple extension allows Congress time to come to an agreement on the final FAA reauthorization bill. This will allow the MG Community time to contact their members of Congress to express support for the disability provisions included in the legislation. Go to [Senators](#) and [Representatives](#) to act.

Advocacy

Orphan Drug Tax Credit

MGFA & National Organization for Rare Diseases (NORD)

The MGFA lent its support to NORD's advocacy letter regarding the *Tax Cuts and Jobs Act (Act)* opposing the Senate proposal in the Act to reduce the **Orphan Drug Tax Credit** by 50 percent and the House of Representatives version to eliminate the tax credit entirely. To view the NORD letters, click on the November 10 letter [Advocacy Letter](#) and the December 18 [NORD Statement](#)

The final *Tax Cuts and Jobs Act* adopted by Congress on December 20 and forwarded to the president for his signature retained the tax credit, but reduced it by 50 percent as provided in the Senate version. On December 20 NORD stated to advocates, "Your voices made an impact, and for that we are incredibly grateful. Throughout tax reform there were over 4,000 emails, countless phone calls, and 1,500 uses of the hashtag #SaveOrphanDrugs for the Orphan Drug Tax Credit alone. That's amazing! We will continue to stand up for the rare disease community and we hope that you will continue to join us. But for now, all we'd like to say is "Thank you!!"