E-Update
January 2017

MGFA News and Announcements

Change of Logo

MGFA has introduced a new logo. Shown here, the logo was designed to bring a new focus onto MG while conveying a sense of community, accessibility and inclusiveness. MGFA will incorporate the logo gradually into its communications, brochures and other electronic and print media.

Registration Open for 2017 MGFA National Conference in New Orleans

The MGFA is proud to announce that registration for the 2017 National Conference is now open.

The conference will be held at the Astor Crowne Plaza in New Orleans' French Quarter from March 26 - 28. Registration is available here

Hotel registration is available here.

Click here to view the preliminary agenda.

If you have any questions regarding your booking, call the hotel's toll-free number at 877-408-9661.

Evaluation of the Risk of Falls Among the MG Community

Whether falling is a major concern for you or not, MGFA invites you to participate in a survey of the MG community. Research literature does not exist that evaluates the risk of falls in the MG population. Julia Naumes, OTD, OTR/L an occupational therapist and Charlene Hafer-Macko, MD a neurologist are working on this issue. Dr. Naumes, is affiliated with St Anthony Hospital in Gig Harbor, WA, and Dr. Hafer-Macko is affiliated with the University of Maryland School of Medicine and the Baltimore Geriatric Research, Education and Clinical Center within the VA Maryland Health Care System in Baltimore. To find out more about falling from those in clinical remission to those with severe MG symptoms, Drs. Naumes and Macko have created a survey to determine 1) the prevalence and frequency of falls and level of concern about falls; 2) the level of concern for falls in relationship to the MG disease severity; and 3) the activities that are associated with the greatest fear of falling. They also hope to learn how much the fear of falling is prevalent in the MG community and want the largest possible sample representing all the MG Community.

You may already have seen an item on the falls survey on the MGFA website or seen related promotions during the fall 2016. Thank you if you already participated. If not, please share your experience by going to https://www.surveymonkey.com/r/MGFALLS.

MG Walks

2017 MG Walks Begin Feb. 18 in Hawaii

The locations and dates for the first 11 MG Walks of 2017 have been announced for the spring season, including a first stop in Hawaii on Feb. 18.

Here are the upcoming MG Walks:

DATE	CITY	LOCATION
Feb. 18	Hawaii	Kauai Coastal Pedestrian Path in Kappa, HI
March 11	Tampa Bay	R.E. Olds Park, Oldsmar, FL
March 11	Georgia	Brook Run Park, Dunwoody, GA
March 12	Tallahassee	Cascades Park, Tallahassee, FL
March 12	South Florida	Tradewinds Park, Coconut Creek, FL
March 25	New Orleans	Lafreniere Park, Metairie, LA
April 8	South Carolina	James Island Park, Charleston, SC
April 8	North Carolina	Barber Park, Greensboro, NC
May 6	Southern Wisconsin	Sheridan Park, Cudahy, WI
May 6	Northern Wisconsin	Pamperin Park, Green Bay, WI
May 6	New England	Pope John Paul II Park, Boston, MA

For more information and to register for these Walks visit www.mgwalk.org

The MG Walk campaign is dedicated to creating awareness, renewing hope and generating a vast network of community and support, all while raising critical funds for the Myasthenia Gravis Foundation of America.

Our MG Patients, Support Groups in the News

North Carolina MG Patient Completes Very Successful Christmas Toy Drive
Grayson Thorne, a seventh-grader at Springfield Middle School in North Carolina who was diagnosed in 2015 with MG, concluded a very successful holiday toy drive for Duke Children's Hospital, including 2,000 items from books and blocks to puzzles and stuffed animals, board games, hot wheels, Barbie dolls and tea sets. For two months, Grayson and his family collected toys to donate to the hospital, where Grayson has been a patient.
Read more at:
http://www.wilsontimes.com/stories/2000-toys-headed-to-hospital,78894

Research and Medical Industry News

MGFA Medical/Scientific Advisory Board Member Honored

Gil I. Wolfe, MD, Professor and Chairman Irvin and Rosemary Smith Chair of Neurology at the University of Buffalo (UB) Jacobs School of Medicine and Biomedical Sciences was honored with the university's Clinical Research Achievement Award for his promising advances in clinical research. The research competition was sponsored by the UB's Clinical and Translational Science Award (CTSA), which was granted by the National Institutes of Health (NIH) to a consortium of academic and health care institutions in 2015. Wolfe, won the award for his work on "Randomized Trial of Thymectomy in Myasthenia Gravis," published in the *New England Journal of Medicine*.

Dr. Wolfe will have the opportunity to present his research at the annual CTSA Forum March 28 at the UB Clinical and Translational Research Center (CTRC) in Buffalo. With the support of the CTSA, Dr. Wolfe, the top winner, has been nominated by UB to be considered for a prestigious Top 10 Clinical Research Achievement Award, a signature program of the Clinical Research Forum, which takes place in Washington, D.C., in April. For details, go to http://medicine.buffalo.edu/news_and_events.html

Alexion Submits Applications Seeking Approval of Soliris® (Eculizumab) as Treatment for Patients with Refractory Generalized Myasthenia Gravis (gMG)

Alexion announced that they have filed regulatory submissions for Soliris for the treatment of patients with Refractory gMG in both the United States and Europe. Soliris, a terminal complement inhibitor, is thought to work in MG by inhibiting the complement pathway to prevent destruction of the neuromuscular junction (the space across which nerve fibers transmit signals to muscle fibers.)

We at MGFA are pleased that for the first time in nearly eight decades there will be a targeted therapy for MG being considered for approval by the U.S. and European regulatory process. This news is especially exciting as it is particularly focused on those with refractory generalized myasthenia gravis— those whose disease does not respond well to first line treatments. As has often been seen in other disease states, we can be hopeful that this development may open the door to the development of more targeted treatments for MG. Click here to read the full press release from Alexion.

Brussels Pharmaceutical Company Launches Second Phase Study for MG Treatment

Argenx (Euronext Brussels: ARGX), a clinical-stage biopharmaceutical company focused on creating and developing differentiated therapeutic antibodies for the treatment of cancer and severe autoimmune diseases, announced the initiation of a Phase II proof-of-concept study of ARGX-113 in patients with myasthenia gravis (MG). The double-blind, placebo controlled Phase II study will enroll up to 24 MG patients with confirmed generalized muscle weakness. ARGX-113 will be dosed on top of current standard of care, corticosteroids and/or immunomodulatory agents. The primary endpoints of the trial are safety and tolerability and secondary endpoints include efficacy, impact on quality of life and an assessment of pharmacokinetics (PK) and pharmacodynamic (PD) markers. In Phase I clinical trials, ARGX-113 demonstrated favorable safety and tolerability across multiple doses and dosing regimens with promising pharmacodynamics effects relating to speed, depth and duration of IgG reduction.

Read more at:
http://www.globenewswire.com/news-release/2017/01/09/904189/0/en/argenx-launches-Phase-II-proof-of-concept-study-of-ARGX-113-for-the-treatment-of-myasthenia-gravis.html

New Understanding of Autoimmune Function in MS Described as Breakthrough

A breakthrough in the understanding of how the body's autoimmune system affects multiple sclerosis (MS) and information about a new, highly effective MS therapy will be presented at the America's Committee for Treatment and Research in Multiple Sclerosis (ACTRIMS) Forum 2017, Thursday, Feb. 23, in Orlando. The expected launch in early 2017 of ocrelizumab, the first B-cell therapy to be approved by regulatory agencies, will significantly alter the MS treatment landscape.

Read more at:
http://www.newswise.com/articles/view/666838/?sc=mwhn

3-D Printed Medical Devices to Help Doctors Better Visualize Complex Health Care Cases

Right now, manufacturers use 3D printing to create devices matched to a patient's anatomy (called "patient-specific" devices) as well as devices with very complex internal structures. These capabilities have sparked huge interest in the 3D printing of medical devices and other products, including food, household items, and automotive parts. And since the U.S. Food and Drug Administration (FDA) is responsible for protecting the public health, the agency is reviewing these 3D printed products—and researching them—to make sure they are safe and effective for the public. 3D printed medical devices can help doctors better visualize complex health cases. This process can be used to make patient-specific medical devices, and because of its versatility, 3D printing also has medical applications for FDA-regulated drugs and biologics. Read more at:
http://www.fda.gov/ForConsumers/ConsumerUpdates/ucm533992.htm?source=govdelivery&utm_medium=email&utm_source=govdelivery

Using Robotics and Automation to Improve Accessible Transportation Options

A new grant opportunity from the National Institute on Disability, Independent Living, and Rehabilitation Research (NIDILRR) at the Administration for Community Living (ACL) has been announced under the Disability and Rehabilitation Research Projects Program (DRRP) for Using Robotics and Automation to Improve Accessible Transportation Options for Individuals with Disabilities.

The purpose of the DRRP program is to plan and conduct research, demonstration projects, training, and related activities (including international activities) to develop methods, procedures, and rehabilitation technology that maximize the full inclusion and integration into society, employment, independent living, family support, and economic and social self-sufficiency of individuals with disabilities.

This DRRP grant opportunity, open through March 21, 2017, is for advancing the application of automation and robotics to enhance accessible transportation for travelers with disabilities and improving opportunities for a seamless travel chain that meets the diverse needs of travelers with disabilities (including mobility, vision, hearing, and cognitive disabilities). Click on the highlighted text to view the Grant Opportunity page.

News from the Public Sector

The White House

One of President Trump's first actions on January 20th was to sign an Executive Order "Minimizing the Economic Burden of the Patient Protection and Affordable Care Act Pending Repeal."

> Section 1. States:
> *By the authority vested in me as President by the Constitution and the laws of the United States of America, it is hereby ordered as follows:*
> *Section 1. It is the policy of my Administration to seek the prompt repeal of the Patient Protection and Affordable Care Act (Public Law 111-148), as amended (the "Act"). In the meantime, pending such repeal, it is imperative for the executive branch to ensure that the law is being efficiently implemented, take all actions consistent with law to minimize the unwarranted economic and regulatory burdens of the Act, and prepare to afford the States more flexibility and control to create a more free and open healthcare market.*

The 5 sections that follow provide instructions to the heads of agencies and departments with authorities and responsibilities under the Act. You can read the Executive Order by going to: https://www.whitehouse.gov/the-press-office/2017/01/2/executive-order-minimizing-economic-burden-patient-protection-and To read the full text of the White House press release, click on http://www.cnn.com/2017/01/20/politics/trump-obamacare-executive-order/index.html on the CNN website.

The MGFA is concerned that Congress is likely to repeal the Patient Protection and Affordable Care Act (ACA) very soon, and the law guarantees that people with chronic conditions have access to affordable health insurance, and Congress could take this away. As a member of the National Health Council and our fellow member patient advocacy organizations, we are asking those in the MG community to call or write their Members of Congress to demand they not take away access for people with chronic conditions. Contact information for your senators and representatives, and a sample message for you to personalize and use, are below.

Contact Information:
Call or write your Senators and Representative.
To find out who your Senators are: https://www.senate.gov/senators/contact/
To find out who your Representative is:
http://www.house.gov/representatives/find/

Sample Message:
As someone with a chronic pre-existing condition, I urge you to not repeal the Patient Protection and Affordable Care Act until there is a guarantee that I and others like me will still have access to equal or better coverage.
(Include your personal story about living with your condition and how the ACA has helped you access your care)

A vote to repeal the law without an adequate replacement means that I could lose access to the health care I need.

Health and Human Services (HHS)

HHS Analysis: Uninsured Rate for Americans with Pre-Existing Conditions Dropped Sharply When Affordable Care Act (ACA) Reforms Implemented

On January 5, 2017 HHS released a new analysis which revealed that without ACA protections, more than half of non-elderly Americans could face discrimination in health care. Since the ACA became law, millions of Americans no longer face coverage denials, higher costs, or coverage carve outs because of their medical histories. A new analysis from the HHS provides a first look at what happened to uninsured rates for Americans with pre-existing health conditions when the ACA's major insurance market reforms took effect in 2014. It finds that, between 2010 and 2014, the share of Americans with pre-existing conditions who went without health insurance all year fell by 22 percent, meaning 3.6 million fewer people with pre-existing conditions went uninsured. While data for individuals with pre-existing conditions are available only through 2014, the uninsured rate for all Americans has fallen by an additional 22 percent through mid-2016, and Americans with pre-existing conditions have likely seen similar additional gains.
Read more at:

http://npvi.com/pharmacy-today/uninsured-rate-for-americans-with-pre-existing-conditions-

dropped-sharply-when-major-aca-reforms-were-first-implemented/ Scroll down to see the article.

For the HHS Website go to https://www.hhs.gov/healthcare/about-the-law/pre-existing-conditions/index.html

U.S. Food and Drug Administration (FDA)

What does the U.S. Food and Drug Administration regulate?

The FDA is responsible for protecting the public health by regulating human drugs and biologics, animal drugs, medical devices, tobacco products, food and animal feed, cosmetics, and products that emit radiation. A recent FDA consumer update describes FDA's regulatory process.
Here is a guide to how FDA regulates products —and what the agency does (and doesn't) approve.

FDA Encourages More Participation and Diversity in Clinical Trials

The FDA describes clinical trials as voluntary human research studies designed to answer specific questions about the safety and effectiveness of drugs, vaccines, devices, and other therapies—or to study new ways of using existing treatments. The FDA is encouraging more people—including members of diverse racial and ethnic groups, women, and older adults—to participate in clinical trials. Read the Consumer Update to learn why.

How to Report Product Problems and Complaints to the FDA

The FDA protects the public health by helping to ensure the safety, efficacy, and security of human and veterinary drugs, biological products, medical devices, and products that emit radiation—and by helping to ensure the safety and security of the nation's food supply and cosmetic products. The FDA also regulates tobacco products. If you use a product regulated by the U.S. Food and Drug Administration and have an unexpected reaction or other problem, the FDA wants to hear from you. Learn how to report problems.

Congressional Budget Office (CBO)

The CBO, with the U.S. House of Representatives Joint Committee on Taxation (JCT) analyzed the effects on Affordable Care Act (ACA) coverage or premiums that would result from H.R.3762, which would leave ACA market reforms in place while repealing the mandate penalties and subsidies. In brief, CBO and JCT estimated that enacting that legislation would affect ACA insurance coverage and premiums primarily in these ways:
A. The number of people who are uninsured would increase by 18 million in the first new plan year following enactment of the bill. Later, after the elimination of the ACA's expansion of Medicaid eligibility and of subsidies for insurance purchased through the ACA marketplaces, that number would increase to 27 million, and then to 32 million in 2026.
B. Premiums in the nongroup market (for individual policies purchased through the marketplaces or directly from insurers) would increase by 20 percent to 25 percent—relative to projections under current law—in the first new plan year following enactment.

The increase would reach about 50 percent in the year following the elimination of the Medicaid expansion and the marketplace subsidies, and premiums would about double by 2026.
To read the report which was released on January 17, 2017, go to https://www.cbo.gov/publication/52371

Government Accountability Office (GAO)

In December, 2016 the GAO released the results of its study *GAO-17-143* of the Food and Drug Administration (FDA) foreign drug inspection program. Stating that globalization has complicated FDA's oversight of drugs marketed in the United States, the GAO found that the FDA has improved its foreign drug inspection program but needs to assess the effectiveness and staffing of its foreign offices
FDA has not yet assessed its foreign offices' contributions to drug safety. The GAO stated that FDA has made progress in its strategic planning for its offices in China, Europe, India, and Latin America, but the lack of an assessment of their contributions to drug safety is inconsistent with federal standards for internal controls.

FDA reports that more than 40 percent of finished drugs and 80 percent of active pharmaceutical ingredients are produced overseas. Beginning in 2008, FDA established foreign offices to obtain better information on products coming from overseas and perform inspections.
The number of foreign inspections has consistently increased each year since fiscal year 2009. Beginning in fiscal year 2015, FDA conducted more foreign than domestic inspections.
For a summary of the report, including additional findings and recommendations, click on
http://www.gao.gov/assets/690/681688.pdf?utm_medium=email&utm_source=govdelivery

American Autoimmune Related Diseases Association (AARDA)

On December 29th AARDA (www.aarda.org) announced its latest Autoimmune Heroes as part of its year-long 25th anniversary celebration. They are Founder and Executive Director Virginia Ladd, Director of Operations Jerry Ladd, Assistant Director Patricia Barber and InFocus Editor and Executive Assistant Eula Hoover. The association stated that these four individuals were recognized as the driving force behind the organization's founding and tremendous growth and success in creating a national dialog around autoimmunity and autoimmune disease. In addition, they were recognized for advocating, facilitating and funding collaborative research and public awareness campaigns, and providing a support system for the 50 million Americans and their families who are impacted by the more than 100 autoimmune diseases such as myasthenia gravis.