

E-Update



E-Update August 2016

Myasthenia Gravis News

Sanders & Wolfe Head Panel for Myasthenia Gravis Treatment Guidance

Don Sanders, MD, Professor of Neurology, Duke University and **Gil I. Wolfe, MD**, professor and Irvin and Rosemary Smith Chair of Neurology, Buffalo General Medical Center are co-directors of an expert panel that has published the first international treatment recommendations for patients with myasthenia gravis (MG). Published July 26 in *Neurology*, the “**International Consensus Guidance for Management of Myasthenia Gravis**” offers clinicians up-to-date recommendations for a multifaceted approach to caring for patients with MG, a chronic autoimmune neuromuscular disease that affects some 700,000 people around the world. Patients will benefit by receiving a standard approach to their care modeled on the available evidence, Wolfe notes, adding that insufficient studies exist on which to base a formal practice parameter. The consensus task force was appointed and funded by MGFA, and the organization has also paid to create open access for the public, not just to Neurology subscribers. Both patients and physicians can use this information in decision making about treatment as well as advocacy for access and reimbursement for treatments from payers. We are grateful for the diligent work of the members of the consensus task force and believe this is an important step forward in better understanding the management and treatment of MG.

[For the full scientific article click here.](#)

[Read more here.](#)

New York MG Walk Hero Featured on Fox5 News

New York patient and this year's Tri-State MG Walk Hero, Elizabeth Roque, was featured on FOX 5 News in New York to discuss her battle with MG.

[Watch the telecast here.](#)

19-Year-Old Texas Resident Featured in San Angelo News

Nineteen-year-old MG patient Keelie Brydson, from Pflugerville, Texas and a sophomore at Texas A&M University, was featured in the San Angelo News with her personal MG story as she shared her battle with MG since the age of 14.

[Read more here.](#)

Durham MG Patient Patricia Fife Doesn't Let MG Define Her

Durham MG patient Patricia Fife had her editorial published on MG in The News & Observer. "People with MG are as smart, capable, interesting and sensitive as any able-bodied person. Like snowflakes no two MG patients are alike. With proper treatment we can all live fulfilling lives despite MG. I want to make a difference and have a positive impact on the world. I don't want this disease to define me. I have myasthenia gravis; it doesn't have me!"

[Read her article here.](#)

Portland MG Patient Blogs about Her Battle at Mango Health

Portland MG patient Missy Gillespie runs a blog, "Mango Health," where she chronicles her daily battles with MG. Read Missy's [blog here](#).

Guilford, NC County Commissioner Raises Awareness of MG

County Commissioner Ray Trapp searched for information when his 3-year old daughter was recently diagnosed with MG. When he learned that MG is a rare disease, he decided to try to raise awareness. He contacted Vicki Ruddy, co-facilitator of the Triad MG Support Group, and together they crafted a proclamation that was adopted by the commissioners, declaring June as Myasthenia Gravis Awareness Month in Guilford County. Trapp said he hopes to organize a local MG walk next June and plans to meet with Guilford County's Emergency Services to discuss the signs and symptoms that MG patients may exhibit during medical emergencies. For more information [click here](#).

MG Walk Campaign surpasses \$500,000 and is more than half way to its 2016 fundraising goal!

The 2016 MG Walk Campaign is off to its best start in 6 years! Through mid-summer, the MG Walk has raised more than \$500,000 and is poised to reach its goal of \$900,000 by this Fall. Throughout the months of September, October, November and December, more than 20 MG Walks will take place in the cities listed below:

Cleveland – September 24
Northern Illinois – September 25
Delaware Valley – October 1
Portland – October 1
DC Metro – October 2

Seattle – October 2
Southern Illinois – October 8
Mississippi – October 8
Utah – October 15
Fall New England – October 15
Colorado – October 16
Kentucky – November 5
Indiana – November 6
Tennessee – November 12
Houston – November 12
Austin – November 12
Greater Los Angeles – November 19
Sacramento – November 19
Bay Area – November 20
Inland Empire – November 20
Arkansas – December 3
Arizona – December 4

Registrations for Walks that take place in the Fall are open and all are encouraged to sign up, start a team and begin their fundraising efforts. To begin your registration, click on an MG Walk listed above or visit www.MGWalk.org. If there is not an MG Walk in your backyard, please consider starting a Virtual team and participate anywhere with your team...local park, gym or just fundraise! Also, if you are interested in bringing a new MG Walk to your community, please contact the MG Walk office.

Fundraising for Spring and Fall Walks are open through the end of the year! Please note that any 2016 MG Walk registrant who raises \$1,000 or more will automatically be entered into a raffle for a chance to attend the 2017 MGFA National Conference in New Orleans! For every \$1,000 raised by your team, Captains will receive one raffle ticket for a terrific opportunity to win complimentary conference registration for two, which includes airfare and hotel accommodations. Deadline is December 14.

The MG Walk Office is available to assist you and your team to achieve all your goals and enhance your MG Walk experience. Please contact the MG Walk team anytime at 1-855-MG-Walks (1-855-649-2557) or at Info@MGWalk.org.

Together, we will take the necessary steps toward the ultimate finish line...a world without myasthenia gravis!

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Research and Advocacy News

Surgical removal of the thymus benefits myasthenia gravis patients without a chest tumor, according to NEJM paper

A new study published in the New England Journal of Medicine addresses a question doctors have sought to clarify for decades: whether a surgery conducted since the 1940s benefits the patients it targets. The study found that surgical removal of the thymus gland from patients with myasthenia gravis, a rare autoimmune disease that affects neuromuscular function, provides significant benefit in patients who do not have a thymic tumor. The new study provides a definitive answer that neurologists have sought for decades, according to Gil I. Wolfe, MD, Irvin

and Rosemary Smith Chair of the Department of Neurology in the Jacobs School of Medicine and Biomedical Sciences at the University at Buffalo, clinical chair of the worldwide study and first author. Dr. Wolfe is a member of MGFA's Medical Scientific Advisory Board and this study was supported by the Myasthenia Gravis Foundation of America (MGFA), the Muscular Dystrophy Association (MDA) and National Institute of Neurological Disorders and Stroke (NINDS).

Read more at: <http://www.buffalo.edu/news/releases/2016/08/012.html>

Eculizumab shows promise in treating refractory generalized myasthenia gravis (gMG)

Newly reported data from a clinical trial led by a UNC School of Medicine researcher show that eculizumab may be helpful in treating refractory generalized myasthenia gravis (gMG), an extremely rare form of MG, a neuromuscular disorder characterized by weakness and fatigue of skeletal muscles. Current MG treatments are ineffective for those with refractory MG. James F. Howard Jr., MD, Distinguished Professor of Neuromuscular Disease, professor of neurology, medicine and allied health, and chief of the Neuromuscular Disorders Section in the UNC School of Medicine, **presented the results** July 7 at the 14th International Congress on Neuromuscular Diseases (ICNMD) in Toronto.

[Read more here.](#)

MGFA Joins with Research America – an Alliance for Discoveries in Health to Support Cures Now Legislation

The MGFA has joined with Research America – an Alliance for Discoveries in Health to support Cures Now legislation. The 21st Century Cures Act (HR6) also known as Cures Now, aims to speed cures by removing barriers to collaboration; incorporating the patient perspective; modernizing clinical trials; and providing new incentives for the development of drugs for rare diseases, among other strategies for advancing cures. In addition, the bill calls for an investment of additional funding to the NIH (\$1.75 Billion) and the FDA (\$110 Million) for 5 years. Supporters also present the bill as reassurance that America's continued leadership in global medical and biopharmaceutical industries will help maintain and develop hundreds of thousands of jobs. For more details, see the bill summary at:

[https://energycommerce.house.gov/sites/republicans.energycommerce.house.gov/files/114/Cures 2015FACTSHEET.pdf](https://energycommerce.house.gov/sites/republicans.energycommerce.house.gov/files/114/Cures%2015FACTSHEET.pdf)

YOU CAN HELP support needed action on this bill by visiting the Research America website: **http://cqrcengage.com/ram/CuresNOW_home** where you can put in your zip code and find your representatives as well as send them a message or even a video. The site also has additional information and ideas to help advocates take action and make a difference. Congress needs to be galvanized into taking action on Cures Now and you can help. See the update below under "U.S. Congress."

New Research Studies Identify Potential Cause of and New Treatment for Autoimmune Diseases

The American Autoimmune Related Diseases Association, Inc. (AARDA) is spotlighting two new research studies originally reported in "Science Daily." The first study advances understanding

of a potential cause of autoimmune disease, while the second examines a new treatment approach that could have wide-ranging implications for many autoimmune diseases. In both cases, AARDA believes the research is promising and additional studies are needed to confirm the findings.

[Read more here.](#)

New Therapy Treats Autoimmune Disease without Harming Normal Immunity

In a study with potentially major implications for the future treatment of autoimmunity and related conditions, scientists from the Perelman School of Medicine at the University of Pennsylvania have found a way to remove the subset of antibody-making cells that cause an autoimmune disease, without harming the rest of the immune system. The autoimmune disease that the team studied is called pemphigus vulgaris (PV), a condition in which a patient's own immune cells attack a protein called desmoglein-3 (Dsg3) that normally adheres skin cells. Current therapies for autoimmune disease, such as prednisone and rituximab, suppress large parts of the immune system, leaving patients vulnerable to potentially fatal opportunistic infections and cancers. The Penn researchers demonstrated their new technique by successfully treating an otherwise fatal autoimmune disease in a mouse model, without apparent off-target effects, which could harm healthy tissue. The results are published in an online First Release paper in Science.

[Read more herre.](#)

University of Michigan Examining Sleep Issues in MS Patients

Multiple sclerosis looks different from person to person. In many individuals, though, the difficulty in maintaining a sense of self and in keeping up intellectually can be the disease's most devastating manifestations. With this in mind, University of Michigan researchers are exploring a new way to improve cognitive issues, such as memory, attention and mental processing in MS patients: by examining sleep.

[Read more here.](#)

Catalyst Pharmaceuticals Agreement with FDA

Catalyst Pharmaceuticals has entered into an agreement with the FDA to conduct a clinical trial protocol for its second phase III trial on Firdapse for the treatment of Lambert-Eaton Myasthenic Syndrome (LEMS) following receipt of a 2015 "refusal-to-file" letter from the FDA in connection with its New Drug Application for Firdapse.

Catalyst reports that it expects to initiate a small, efficient short-term phase III study on Firdapse in the second half of 2016. The company has initiated an investigator-sponsored study for the symptomatic treatment of MuSK-antibody positive myasthenia gravis (MuSK-MG). [Click here to](#)

view the company's press release of August 9, 2016.

Study Finds that Majority of People with Mobility Disabilities Are Not Living in Accessible Homes

A National Institute on Disability, Independent Living, and Rehabilitation Research (NIDILRR) funded study released on August 3, 2016 found that, although mobility aids, such as wheelchairs and scooters, allow people with mobility disabilities to get around and be more active in their communities, some people may be living in homes that do not meet their needs. Home features like stairs and narrow doorways make homes less accessible for mobility aid users. Living in an inaccessible home can make it harder for people with mobility disabilities to live, work, and participate in their communities. For more information, [Read this article](#)

NIDILRR is in the process of transitioning to the [Administration For Community Living \(ACL\)](#) in the U.S. Department of Health and Human Services (HHS).

News from the Public Sector

Health and Human Services (HHS)

HIPPA (Health Insurance Portability and Accountability Act of 1996) Reaches 20th year August 21, 2016 marked the 20th anniversary of HIPPA law which was enacted primarily to allow people to transfer and continue health insurance after they change or lose a job. It also is known for its provisions designed to protect health care privacy and the security of health care data. For more information, go to <http://www.hhs.gov/blog/2016/08/19/hipaa-20-bipartisan-achievement.html>.

HHS Announces Investments in health centers quality care and patient centered care delivery; rural health programs targeting chronic care; quality care improvement systems and primary care delivery

On August 4, 2016 HHS Secretary Sylvia M. Burwell announced more than \$8.6 million in funding for 246 health centers in 41 states, the District of Columbia, the Federation of Micronesia and the Northern Mariana Islands. The awards will help to improve quality of care and patients' and providers' experience of care through the Patient-Centered Medical Home (PCMH) health care delivery model. [Read the news release.](#)

On August 11, 2016 HHS announced new awards for five rural health programs administered by the Federal Office of Rural Health Policy (FORHP). Of those funds, more than \$10 million will go to the [Delta States Rural Development Network Program](#), targeting chronic disease in the eight states of the Delta region (Alabama, Arkansas, Illinois, Kentucky, Louisiana, Missouri, Mississippi, and Tennessee). [Four additional programs received funds](#) that will expand use of

telehealth technology for rural veterans and other patients, assist providers with quality improvement activities, and support policy-oriented research to better understand the challenges faced by rural communities.

On August 18, 2016 Secretary Burwell announced more than \$100 million to 1,304 health centers in all 50 states, the District of Columbia, and 7 U.S. territories to recognize health center achievements in providing high quality, comprehensive care. Health centers will use these funds to expand current quality improvement systems and infrastructure and to improve primary care service delivery in the communities they serve.

[Read the news release.](#)

Administration for Community Living (ACL)

Celebrating the 26th Anniversary of the Americans with Disabilities Act

July 26, 2016 marked the 26th anniversary of the Americans with Disabilities Act (ADA) According to the ACL, “the ADA made clear that people with disabilities of all ages should experience equal opportunity, independent living, economic self-sufficiency, and full participation in all aspects of society. From curb cuts and accessible buses to the integration of people with disabilities in schools, workplaces, businesses, and neighborhoods, the ADA has made communities more accessible and more inclusive.” For more information, go to:

<http://www.adaresources.org/>

The Disability Connection July newsletter Disability.gov contains [10 Things to Know about the ADA and Beyond](#)

Complaints against airlines by people with disabilities increased in 2016

<https://www.disability.gov/airline-consumer-complaints-by-passengers-with-disabilities-increased-in-2016/>

The U.S. Department of Transportation reports that the total number of airline complaints filed during the first six months of 2016 was down 12.2 percent compared with the same time period in 2015. However, complaints by passengers with disabilities increased during this time period.

For more information go to: <https://www.transportation.gov/airconsumer/file-consumer-complaint#DisabilityandDiscriminationComplaints> or visit Disability.gov to learn more about the [rights of air travelers with disabilities.](#)

Enhancing Protective Services for Adults and People with Disabilities

On August 18 the ACL’s Administration on Aging announced grants totaling \$2.2 million to assist 13 state Adult Protective Services (APS) systems in addressing the abuse, neglect, and exploitation of older adults and people with disabilities. States will use the two-year grants to enhance statewide APS systems, improve practices and data collection, and interface with ACL’s National Adult Maltreatment Reporting System (NAMRS). For a list of states receiving grants and description of their projects, go to:

http://acl.gov/NewsRoom/NewsInfo/2016/2016_08_18.aspx

Food and Drug Administration (FDA)

Precision Medicine Initiative

In support of the President's Precision Medicine Initiative, the FDA issued two sets of draft guidance that when finalized, according to the FDA, "will provide a flexible and streamlined approach to the oversight of tests that detect medically important differences in a person's genomic makeup." To see the FDA's July 6, 2016 press release, go to:

<http://www.fda.gov/NewsEvents/Newsroom/PressAnnouncements/ucm509814.htm>

Government Accountability Office (GAO)

Drug Shortages Report

On July 7, 2016 the GAO released report GAO-16-595: Drug Shortages: Certain Factors Are Strongly Associated with This Persistent Public Health Challenge. The GAO describes drug shortages as a serious public health concern. In compliance with statutory directive, the GAO examined trends in drug shortages; FDA's efforts to prioritize reviews of drug submissions to address shortages; trends in FDA warning letters issued to sterile injectable manufacturing establishments for noncompliance with manufacturing standards; and the relationship between certain factors and shortages of sterile injectable drugs. To view the report, go to

<http://www.gao.gov/products/GAO-16-595>. Highlights are available at <http://www.gao.gov/assets/680/678282.pdf>

U.S. Congress

Cures Legislation Vote Postponed in Senate

In July the Senate announced that it would not vote on its Cures Legislation, also known as the Senate Innovations for Healthier Americans Initiative, until September at the very earliest. The National Organization for Rare Diseases (NORD), of which MGFA is a member, issued a statement criticizing this postponement and wrote in part, "On behalf of the 1 in 10 Americans with rare diseases, most of whom are still waiting for a treatment or cure, we are disappointed that Senate Cures was not able to pass at this point. We will keep pushing for this legislation through the summer recess and into September to ensure the voices of patients and families are heard." For the full NORD statement, go to <http://rarediseases.org/nord-issues-statement-senate-postpones-vote-cures-legislation/> for more information go to

<http://everylifefoundation.org/open-act/> The MGFA has joined with Research America – an Alliance for Discoveries in Health to support Cures Now legislation in the U.S. House of Representatives. See MGFA Joins with Research America – an Alliance for Discoveries in Health to Support Cures Now Legislation in this update.

Accessible Air Travel

The Muscular Dystrophy Association (MDA) continues to work closely with a diverse set of stakeholders to advocate for enactment of increased accessibility to air travel. The Senate version of the Federal Aviation Authority (FAA) reauthorization bill contains provisions targeted for disabled passengers. Congress was required to reauthorize the FAA because its operating authority was set to expire in July. The House and Senate did not reach an agreement on the reauthorization. However, they did pass an extension to allow more time to work out a solution. According to the MDA, the extension agreement includes some policy provisions, including two sections that impact passengers with disabilities. The first requires the Department of Transportation to move forward with several regulatory rule making efforts that impact travel for disabled passengers, and the second requires a report to Congress regarding the training of personnel providing assistance to passengers who use wheelchairs.

National Organization for Rare Diseases (NORD)

Rare Diseases and Orphan Products Breakthrough Summit

NORD announced that registration is open for its October 17-18 Rare Diseases & Orphan Products Breakthrough Summit in Arlington, VA. NORD describes the summit as an "...opportunity to collaborate with the top leaders from the FDA, NIH, industry, patient groups, payers and research institutions to address the progress and innovations in rare disease diagnosis, treatment, patient engagement and market access of orphan products." For more information, go to: <http://rarediseases.org/summit-overview/>

American Red Cross

Safety during a Power Outage

The American Red Cross website contains a Disaster and Safety Library to assist people in preparing their homes, schools and workplaces in the event of a disaster or emergency. The Red Cross provides fact sheets, preparedness checklists, recovery guides and other information to keep users informed and safe. The web site contains "**Tools and Resources**" including a "**Power Outage Checklist**." For information and check lists for a variety of disasters, go to <http://www.redcross.org/prepare/disaster-safety-library>