



MGFA E-Update

July 2017

MGFA News

MGFA Announces Next Annual Conference Location

MGFA is thrilled to announce that the next Annual Conference will take place April 15th to 17th in Kansas City, MO at the Intercontinental Kansas City at the Plaza. 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. To get a KC introduction [Click Here](#). Kansas City International Airport is less than 25 miles from downtown KC and offers 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. And, our national walk will take place on April 14th – details will be forthcoming – but plan to form teams and be a Walker in KC! ***Mark your calendars*** for a fun and educational experience that rare occasion when you can meet so many others in the MG Community both patient and professional. More information will be forthcoming soon. Watch the MG website and Facebook site for more!

Corporate City Skylines Light Up Teal for June Awareness

Many MG support group volunteers, along with members of the MGFA board, were successful in getting corporate buildings and landmarks in various U.S. cities to light up their buildings the color of teal to celebrate MG Awareness Month in June. Cities participating in the light up campaign this year included Mobile, Atlanta, Chicago, Cleveland, Tampa, and Los Angeles. Many thanks to those dedicated volunteers who made this unique feat possible to help further MG awareness!

7,500 People Proclaim “#MGStrong” on Social Media for June Awareness

For the second consecutive year, the MGFA conducted a social media campaign for MG patients, family members, friends and caretakers to partake in changing their profile picture on social media web sites, Facebook and Twitter, to showcase a special Twibbon in conjunction with MG Awareness Month in June. This year, more than 7,500 people updated their profile pictures with the #MGStrong Twibbon. Thanks to all who participated in helping us raise MG awareness!

Did you join the MG Patient Registry?

If not, make a point of doing so this week. You'll be helping the entire MG Community -- "The only way we can really understand MG is if we have more information about who gets MG, when they get it, their symptoms" ... Robert Ruff, MD, PhD, Chairman Medical/Scientific Advisory Board. Through the registry the MG community will help identify how each of us was diagnosed, how we are being treated, our insurance challenges, and how we are feeling. By making a patient community more accessible and understandable, a patient registry and its bounty of information can encourage pharmaceutical developments to pursue drug discovery in a disease such as MG.

To join go to [MG Patient Registry Link](#).

Highly Acclaimed Chef, Sean Brock, Named as MGFA Ambassador

Chef Sean Brock has graciously volunteered to be MGFA's Awareness Ambassador. Announcing Chef Brock's honor, MGFA chief executive officer Nancy Law stated, "We applaud Chef Brock for giving a voice to other patients with MG who have experienced similar frustrations and barriers to diagnosis."

Known for his Southern culinary creations, restaurants in Charleston, SC and Nashville, TN, and his work on The Mind of a Chef, Brock has spoken out about the challenges he faced in getting diagnosed and managing MG. He is planning a series of exclusive dinners with Michelin award-winning chefs that aim to raise funds for the MGFA's mission. To view the press release, visit: <https://www.send2press.com/wire/chef-sean-brock-honored-by-myasthenia-gravis-foundation-of-america-for-awareness-efforts/>

MG Walks Top \$500,000 Mark Thus Far in 2017

As of the end of June, the MG Walks for the first half of 2017 have raised more than \$500,000. With 60% of the MG Walks the walks yet to occur, the walk campaign is more than halfway toward the year-end goal of raising \$900,000. Since the inception of

the MG Walks in 2011, 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. Visit www.mgwalk.org for information or call 1-855-649-2557.

UPCOMING WALKS

- **Sept. 10** **Indiana**
White River State Park, Museum Lawn, Indianapolis, IN
- **Sept. 17** **Kentucky**
EP "Tom" Sawyer State Park, Shelter #1, Louisville, KY
- **Sept. 23** **Ohio**
Rocky River Reservation, Sycamore Shelter Cleveland, OH
- **Sept. 24** **Chicagoland**
Miller Meadow, Forest Park, IL
- **Sept. 30** **Virginia**
South Run Park, Springfield, VA
- **Sept. 30** **Maryland**
Middle Branch Park, Baltimore, MD
- **Sept. 30** **Portland**
Summerlake Park, Tigard, OR
- **Sept. 30** **Utah**
Sugar House Park, Big Field Pavilion, Salt Lake City, UT
- **Oct. 1** **Delaware Valley**
Lorimer Park, Abington Township, PA
- **Oct. 1** **Seattle**
Seward Park, Seattle, WA
- **Oct. 7** **Gettysburg (Pennsylvania)**
Gettysburg Area Recreation Park, Gettysburg, PA
- **Oct. 8** **Colorado**
Cherry Creek State Park, Hobie Hill Shelter, Aurora, CO
- **Oct. 14** **Mississippi**
Sam Coopwood Park, Holly Springs, MS
- **Oct. 14** **Southern Illinois**
Washington Park, Metropolis, IL
- **Nov. 18** **Houston**
Stude Park, Houston, TX
- **Nov. 18** **Sacramento**
William Land Park, Sacramento, CA
- **Nov. 18** **Greater Los Angeles**
Location: TBA
- **Nov. 19** **Austin, Texas**
East Metro Park, Manor, TX
- **Nov. 19** **Inland Empire**

- Spirit Park, Menifee, CA
- **Nov. 19** **Bay Area**
Lake Merced Park, San Francisco, CA
 - **Dec. 2** **Arkansas**
Lake Dardanelle State Park, Russellville, AR
 - **Dec. 3** **Arizona**
Chaparral Park, Scottsdale, AZ

The 13th International Conference on Myasthenia Gravis and Related Disorders

We were excited about this conference that had been more than a year in planning, and it exceeded even our very high expectations. Nearly 300 scientists and clinicians from 5 continents gathered in the New York Academy of Sciences (MGFA's partnering organization), May 15-17 for a 3 day meeting on MG and myasthenic syndromes. This meeting is held only once every 5 years, and is considered the premiere meeting in the world on MG. There were more than 50 renowned speakers and more than 100 posters presented. The distinguished steering committee was led by MGFA MSAB members Linda Kushner, PhD, of George Washington University, and Ted Burns MD, University of Virginia. MGFA thanks all involved who worked tirelessly to make this an amazing event, with cutting edge science and so much hope for better treatments. We also thank all of our sponsors, led by Alexion Pharmaceutical and CSL Behring as Gold and Silvers sponsors. And we thank our donors and Walk fund raisers whose generosity helps to fund meetings like this to advance research.

MGFA Scientific Session to be held September 13 in Phoenix, AZ

Not to be overshadowed by the International Conference, the annual MGFA Scientific Session will attract as many as 200 scientist and clinicians attending the annual meeting of the American Academy of Neuromuscular and Electrodiagnostic Medicine (AANEM). Twenty-two abstracts have been reviewed and accepted for platform presentations. The conference will be led by MGFA MSAB members, Jeffrey Guptill, MD (Duke), Michael Hehir, MD (U. of Vermont) and Amanda Guidon, MD (Massachusetts General Hospital). We also plan to launch our new Partners in MG Care Program which will help to identify and cultivate top MG health care providers across the country, and to be the foundation of an expanded process for MG patients seeking referrals.

MG Friends Program Launched

MGFA is pleased to announce the launch of our long awaited one-on-one telephone peer support program: MG Friends. The first volunteers have completed orientation and training, and have begun to connect with patients---both those seeking someone to talk to about a specific concern in living with MG, and as follow up for new contacts who have connected with MGFA for the first time, to ensure their questions are answered and needs addressed. Find out how you can volunteer to become an MG Friend. Please check the MGFA website www.myasthenia.org or call the office if you want to learn more.

MG Research and Clinical Trials

Patient Visits inspire University of Southern California (USC) researchers to develop crystal model to study molecular structure of MG

As a molecular biology researcher in Professor Lin Chen's [Molecular and Computational Biology Lab](#) at the USC Dornsife College of Letters, Arts and Sciences, Kaori Noridomi gets an up-close view of the targets of her investigations. When she began studying the molecular structures of myasthenia gravis, she decided to step out of the lab for a better view. Noridomi met with patients who have MG and went so far as to attend a fundraising walk that supported research of the disease. Motivated by the patients' stories, Noridomi and a team of scientists, including Chen, developed a 3-D, crystal structure of the disease to fully view its molecular interactions with a neural receptor that is the regular target of the disease. It is the first high-resolution visual display of the molecular interactions. For details, see <https://news.usc.edu/122847>

Alexion Pharmaceuticals, Inc.

Alexion Pharmaceuticals, Inc. announced on June 23rd that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion to extend the current therapeutic indication for Soliris[®] (eculizumab) to include the treatment of refractory generalized myasthenia gravis (gMG) in patients who are anti-acetylcholine receptor (AChR) antibody-positive. The final decision from the European Commission (EC) is anticipated in the third quarter of 2017. If approved, Soliris will be the first treatment available in the European Union (EU) for patients with refractory gMG who are anti-AChR antibody-positive, and the first and only complement inhibitor approved for this disease. To see the Alexion announcement, click on <http://ir.alexionpharm.com/releasedetail.cfm?ReleaseID=1031177>

Adverse Outcomes after Surgery for Patients with MG

Using reimbursement claims from Taiwan's National Health Insurance Research Database, researchers analyzed 2290 patients who received major surgery between 2004 and 2010 and were diagnosed with myasthenia gravis (MG) preoperatively. In the study designed to validate the comprehensive features of adverse outcomes after surgery for patients with MG, researchers found that patients with MG had increased risks of postoperative adverse events, particularly those experiencing emergency care, hospitalization, and thymectomy for care of MG. They stated that their findings suggest the urgency of revising protocols for perioperative care for these populations. For the article released on June 30, 2017, click on <https://doi.org/10.1371/journal.pone.0180433>

Yale New Haven Hospital Studies MG in Clinical Trial

The Yale New Haven Hospital was featured in a television interview on WTNH News Channel 8 in New Haven, Connecticut. The television segment and article discussed the clinical trial on MG, and interviewed Dr. Richard Nowak, Director of the Yale Myasthenia Gravis Clinic, and MG patient Ed Czaczkes, a clinical trial patient. [Click here to watch the segment](#)

Plasmapheresis a Potentially Life-Saving Approach to Veterinary Immunotherapy

For some canine diseases, such as myasthenia gravis, the exchange of plasma can work so quickly and so effectively that a dog who previously could not walk five steps a few hours before treatment can potentially run long distances on his own a few hours afterwards. "The response can be nearly immediate," said clinical assistant professor Alessio Vigani, who recently conducted a plasmapheresis therapy session at the NC State Veterinary Hospital. "And after the second session there's usually a long-lasting response that can be maintained at home." A relatively new therapy in veterinary medicine, plasmapheresis (also known as therapeutic plasma exchange or TPE) can be used to treat diseases associated with a dysfunctional immune system, known as immune-mediated diseases. The NC State Veterinary Hospital is one of just a handful of veterinary medicine institutions in the country that offers plasmapheresis for dogs. [Click here to read the article](#)

MG Patients in the Media

MGFA Ambassador Sean Brock Featured in *New York Times*

Renowned chef Sean Brock who has lived with MG for 3½ years and was recently named MGFA's Awareness Ambassador, was featured on July 3 in a *New York Times* Food Section article about his efforts to implement a healthier lifestyle as part of taking charge of his health. The article chronicles Brock's battle with post-traumatic stress disorder and diagnosis of MG. Dealing with rising fame, a busy professional life owning restaurants in Charleston, SC and Nashville, TN with more to come in Savannah, GA and Greenville, SC, and a major television project, as well trying to manage his MG symptoms, he unwittingly sacrificed his health and well-being. After an "intervention" by people who care about him, Brock sought treatment and returned after his "rebirth" that he says could help change an industry that has always demanded too much. He is committed to becoming an advocate for increasing awareness and research into MG and living with a more balanced approach to his work. [Click here to read the article](#)

Video Game Character Based on Young Person with MG

Victor Mendeveil, a young person with MG reports that "In December, I sent a letter to the developers of the popular mobile video game, High School story, explaining my MG story and asking them to add an MG storyline to their game to raise awareness. I never imagined that would make an entire storyline and character based on me! They even attached a link to the MGFA! High School story is a free app that can be downloaded on almost any Apple device. (Victor says that it is available for download on iOS6.0 or later, compatible with iPhone, iPad, and iPod touch.) The game will need to be updated to Version 4.9 to play "V Is For Victory." Victor says that he believes that players need to play a bit through the game until they reach his quest. To download App Store click on [High School Story App](#).

Los Angeles Publication Discusses MG Patient's Journey

The *Los Angeles Wave* featured MG patient Tameika Isaac Devine, of Columbia, SC, who has had MG since the age of 15. Devine discusses her journey and battle with MG. She is the founder of the Possibilities Institute, a peak performance consulting and public speaking firm specializing in providing personal and professional development for people who are called to a life of change. At age 29, Devine became the first African American woman elected to the Columbia, South Carolina, City Council and now serves as the mayor *pro tem*. She also is a partner in her own law firm, Jabber & Isaac. [Click here to read the article](#)

Former Kentucky Attorney General, MG Patient Dies at 75

Dave Armstrong, a former Kentucky attorney general whose long public career included helping guide the state's largest city and its most populous county toward a merged

government, died Thursday after battling MG. Armstrong, a Democrat, was a fixture in Louisville politics for years, first as a two-term Jefferson County judge-executive and then during a term as Louisville's mayor. "I was proud to know Dave during his nearly five decades of public service, and I firmly believe that the city of Louisville and the entire commonwealth of Kentucky admired him for his leadership and care," said U.S. Senate Majority Leader Mitch McConnell, himself a former Jefferson County judge-executive. "We will all miss Dave's compassion, dedication and vision for our city and our state." Louisville Mayor Greg Fischer remembered Armstrong as a "true public servant" who dedicated most of his life to the city and state. [Click here to read the article](#)

Soviet Dissident Advocate and MG Patient Dies at 84

Edward Kline devoted his career to supporting Soviet dissidents in Russia and promoting their cause abroad. He died on June 24 in Manhattan from complications of MG according to his wife, Jill. Mr. Kline became the principal contact in the United States for Andrei D. Sakharov, the Nobel Peace Prize-winning Russian physicist and human rights campaigner who was confined in domestic exile in the Volga River city of Gorky, east of Moscow, from 1980 through 1986. In collaboration with Robert L. Bernstein, the president of Random House and himself a human rights advocate, Klein shepherded Sakharov's memoirs into publication in the United States and for worldwide distribution. For details click on

<https://www.nytimes.com/2017/06/29/world/europe/edward-kline-dead-american-sakharov-supporter.html>

Actor, Screenwriter, Producer and MG Patient Dies at 84

Roger Smith, who starred as a hip detective in the TV show *77 Sunset Strip*, died on June 4 at the age of 84. He and his wife actress Ann Margret were featured in a recent *People* magazine article about their 50th wedding anniversary. Diagnosed with MG in 1980, he quit his career to become his wife's manager. For additional details click on

<http://www.hollywoodreporter.com/news/roger-smith-dead-77-sunset-888323>

Other Health News and Studies

Kaiser Family Foundation

The Kaiser Family Foundation (KFF) has released a new interactive state-by-state map that provides data on the expected net change in premiums from the Affordable Care Act (ACA) to the proposed Senate Better Care Reconciliation Act (BCRA). To learn more about the map including the statistics included for each state, as well as other categories such as gaps in coverage for pre-existing conditions, coverage and premiums for older adults, provision of key health benefits, and counties at risk of having no insurers, go to <http://www.kff.org/health-reform/>. The KFF also released a

series of [state specific fact sheets](#) which address the number of people covered by Medicaid in each state and what a Medicaid block grant or per capita cap would mean for each state.

Study finds Safety Concerns for 1 in 3 FDA-approved Drugs

Nearly one out of every three drugs approved by the FDA has a new safety issue detected in the years after approval according to a study led by Yale associate professor of medicine and public health Dr. Joseph Ross and published May 9 in the [Journal of the American Medical Association](#) (JAMA). Dr. Ross noted that, while most of the safety concerns are not serious enough to require withdrawal of a drug from the market and point to the need for ongoing monitoring of newly approved drugs, they also demonstrate that the FDA's current process is working. "The fact that the FDA is issuing safety communications means it is doing a good job of following newly approved drugs and evaluating their safety up in the post-market period." For details, click on <http://news.yale.edu/2017/05/09/new-safety-concerns-identified-1-3-fda-approved-drugs>

Argenx Recruiting for Phase 2 Trial of MG Drug

Argenx, a Netherlands-based biotech, is in a Phase 2 trial testing its IgG-blocking drug as a treatment for myasthenia gravis, and immune thrombocytopenia, a blood clotting disorder, and is expanding the MG trial to sites in the United States. MGFA will be assisting with recruiting. [To learn more about this drug, ARGX-113, go to their product pipeline page: http://www.argen-x.com/en-GB/content/argx-113/22/](#)

Study: Accentuate the Positive to Reduce Risk of Chronic Disease

People who experience not just positive emotions but a diversity of positive emotions appear to have lower levels of systemic inflammation, which may reduce their risk for chronic diseases such as diabetes and heart disease, according to research published by the American Psychological Association. "There is growing evidence that inflammatory responses may help explain how emotions get under the skin, so to speak, and contribute to disease susceptibility," said lead author Anthony Ong, PhD, of Cornell University and Weill Cornell Medical College. "Our findings suggest that having a rich and diverse positive emotional life may benefit health by lower circulating levels of inflammation." The research was published in the APA journal *Emotion*. [Click here to read the article](#)

News from the Public Sector

U.S. Senate

Action Alert: Tell your Senators to Oppose the Health Care Bill Today!

The Senate has released their version of the Affordable Care Act (ACA) repeal, called the Better Care Reconciliation Act (BCRA), and planned to pass it by the end of June but delayed voting until after the July 4th recess.

The patient advocacy community stands in strong opposition to the BCRA because it will result in tens of millions of people losing access to affordable health insurance and remove protections for people with chronic conditions. People with chronic conditions will pay more for less benefit. This is unacceptable.

The Myasthenia Gravis Foundation of America is joining with the National Health Council and our fellow [member patient advocacy organizations, and other organizations including the AARP, National Organization on Rare Disorders \(NORD\), the American Academy of Neurology and American Medical Association](#), to rally people to call their Senators, demanding that they do not pass the Better Care Reconciliation Act. If possible, we urge you to call as soon as possible. Your Senators need to hear from you!

How to Take Action:

- To find your Senators: <https://www.senate.gov/senators/contact/>
- Call your Senators and base your comments on the template message below.
- Template Message:

As someone living with a chronic condition, I urge you to not vote for passage of the Better Care Reconciliation Act, as it will reduce access to health insurance and remove important patient protections for people like me. (Include your personal story about living with your condition and the importance of access to affordable health insurance that covers the products and services that you need.)

As you consider repealing and replacing the ACA, please consider how it will impact constituents like me who need access to care.

Thank you.

You can learn more at: www.nationalhealthcouncil.org/healthreform

Health and Human Services (HHS)

Centers for Medicare and Medicaid Services (CMS) New Medicare Cards

CMS announced the development of new Medicare cards that will no longer contain Social Security numbers, a major step to combat fraud and illegal use. CMS explained that the new cards will use a unique, randomly-assigned number called a Medicare Beneficiary Identifier (MBI), to replace the Social Security-based Health Insurance Claim Number currently used on the Medicare card. CMS will begin mailing new cards in April 2018 and will meet the congressional deadline for replacing all Medicare cards by April 2019. CMS has a website dedicated to the [Social Security Removal Initiative](#) (SSNRI) where providers can find the latest information and sign-up for newsletters. CMS is also planning regular calls to share updates and answer provider questions before and after April 2018. Click on [CMS Press Release](#) to review the May 30th release.

Office of the Assistant Secretary for Preparedness and Response (ASPR) Health Care Industry Cybersecurity Task Force Report

Last year HHS established the [Health Care Industry Cybersecurity Task Force](#) following the passage of the Cybersecurity Act of 2015. The Task Force was composed of government and private industry leaders who are innovators in technology and leaders in healthcare cybersecurity. The Task Force held public meetings and consulted with other experts to develop recommendations to address the growing challenge posed by cyberattacks. The task force issued its report on June 2. For details and a PDF version of the task force report, click [Better Protecting the Healthcare System – and, Ultimately, Patient Care – Against Cyberattacks](#)

Agency for Healthcare Research and Quality (AHRQ) Analysis of Patient Safety Event reports

AHRQ released an analysis of more than 80,000 patient safety event reports at a large mid-Atlantic health system. The AHRQ funded study results, announced in *Journal of the American Medical Informatics Association*, revealed that 76 were caused by electronic health record systems that had stopped working. The analysis further showed that in nearly three-quarters of those instances, correct downtime procedures either were not followed or did not exist. The most common safety incidents, recorded over a three-year period ending in January 2016, involved patient misidentification, the miscommunication of clinical information when ordering labs tests or seeking lab results and ordering incorrect medications. Study authors concluded that all facilities should reduce patient risks by developing and practicing procedures for downtimes that may occur during regular maintenance or due to equipment failures, power outages or cyber-

attacks. To view AHRQ's electronic newsletter click on <https://www.ahrq.gov/news/newsletters/e-newsletter/570.html>. To access the abstract, click on [abstract](#).

Health Resources and Services Agency (HRSA) Health Workforce Report

The National Center for Health Workforce Analysis (NCHWA) released a new report and four fact sheets on the national supply of and demand for:

- Geriatricians
- General Pediatricians
- Neurology Physicians and Physician Assistants
- Physical Medicine and Rehabilitation Physicians and Physician Assistants
- Physicians and Physician Assistants in Emergency Medicine

Using HRSA's Health Workforce Simulation Model, the supply of and demand for all five professions are expected to grow between 2013 and 2025. General Pediatricians, Physical Medicine and Emergency Medicine professions will have a sufficient workforce to meet the increased demand. Due to population shifts and physician requirements for an increasing number of older Americans, the United States will have a shortfall of 26,980 Geriatricians and 820 Neurology Physicians in 2025. [Visit NCHWA's website to learn more](#)

U.S. Food and Drug Administration (FDA) Summer Food Safety

The FDA website contains information for summer food health at "Barbeque Basics for Preventing Foodborne Illness." The article appears on [the FDA's Consumer Updates page](#), which features the latest on all FDA-regulated products.

Centers for Disease Control and Prevention (CDC)

Report Card on the Nation's Health "Health, United States 2016" with a "Chartbook on Long-term Trends in Health" prepared by the National Center for Health Statistics (NCHS) is available on the NCHS web site at: <http://www.cdc.gov/nchs/hus.htm>. This is the 40th annual report card on the nation's health, and it examines long-term trends in the health of the U.S. population and the health care system over the past 40 years. The report includes data from a variety of federal government and private sector sources and is organized around four major areas: health status and determinants, health care utilization, health care resources, and health care expenditures.

Travelers' Health Website This website contains several features for travel planning including *CDC Health Information for International Travel*, commonly known as the "[Yellow Book](#)." Published every two years with, the latest health recommendations for

international travel and useful information for travelers, it is written primarily for health care providers, including doctors, nurses, and pharmacists. The book offers an in-depth look at travel health risks and ways to prevent them, advice for people with special travel health needs, and more. It includes immunization requirements, diseases related to travel, advice about travelers with specific needs, such as those with chronic medical conditions or weakened immune systems. For details, click on <https://www.cdc.gov/features/travelershealth.html>. Also see the MGFA's Travel Tips 1 & 2 at <http://www.myasthenia.org/LivingwithMG/InformationalMaterials.aspx> .

The Government Accountability Office (GAO)

On June 14, 2017, the Government Accountability Office (GAO) released:

VA Information Technology: Pharmacy System Needs Additional Capabilities for Viewing, Exchanging, and Using Data to Better Serve Veterans, GAO-17-179

The Department of Veterans Affairs (VA) has system capabilities through multiple computer applications that support its clinicians and pharmacists in prescribing and dispensing medications to patients. However, pharmacists cannot always efficiently view necessary patient data among Veterans Health Administration (VHA) medical sites. In addition, pharmacists cannot transfer prescriptions to other VHA pharmacies or process prescription refills received from other VHA medical sites through the system. As a result, the system does not provide important capabilities for pharmacists to make clinical decisions about prescriptions efficiently, which could negatively affect patient safety. To access the report click on <http://www.gao.gov/products/GAO-17-179>. For highlights click on: <http://www.gao.gov/assets/690/685259.pdf>

On June 26, 2017, the GAO released two reports:

Generic Drug User Fees: Application Review Times Declined, but FDA Should Develop a Plan for Administering Its Unobligated User Fees.

GAO-17-452, May 25

Since the enactment of the Generic Drug User Fee Amendments of 2012 (GDUFA), the FDA reliance on user fees has increased from \$121 million in fiscal year 2013 to \$373 million in fiscal year 2016, or 45 percent of total program obligations in fiscal year 2013 to 76 percent in fiscal year 2016. FDA carried over \$174 million in unobligated user fees at the end of the fourth year of the GDUFA 5-year period. GAO found that although FDA uses an internal management report to track user fee cash flows for internal purposes, it lacks a plan for administering its carryover—one that includes a fully-documented analysis of program costs and risks to ensure that program operations can be sustained in case of unexpected changes in collections or costs. For the report, click on <http://www.gao.gov/products/GAO-17-452>. For highlights click on <http://www.gao.gov/assets/690/684952.pdf>

Physician Workforce: Locations and Types of Graduate Training Were Largely Unchanged, and Federal Efforts May Not Be Sufficient to Meet Needs.

GAO-17-411, May 25

GAO highlights: The federal government has reported physician shortages in rural areas; it also projects a deficit of over 20,000 primary care physicians by 2025. Residents in graduate medical education (GME) affect the supply of physicians. The GAO found that, from 2005-2015, residents were concentrated in the Northeast and in urban areas, and, while many trained in primary care, primary care residents often subspecialize in other fields. Federal efforts to increase GME in rural areas and primary care were limited. GAO stated that in 2015, [we recommended](#) HHS develop a plan for its health care workforce programs—it has yet to do so. To view the full report click on <http://www.gao.gov/products/GAO-17-411>

National Organization for Rare Diseases (NORD)

Senate Better Care Reconciliation Act of 2017. NORD, representing the 30 million Americans with rare diseases, including MG, issued a statement regarding the release of the Senate Leadership's *Better Care Reconciliation Act of 2017* discussion draft.

"Senate Leadership today released a discussion draft of the *Better Care Reconciliation Act of 2017* (BCRA), an amended version of the *American Health Care Act* (AHCA) that passed the House of Representatives in May. As we at NORD have with every other iteration of the AHCA, we evaluated the proposal using NORD's Principles for Health Coverage Reform published in February. We find that the BCRA fails to meet several of the NORD Principles, and we are therefore compelled to oppose the legislation as written." To view NORD's statement of concerns, click on www.rarediseases.org.

Rare Diseases and Orphan Products Breakthrough Summit. This annual summit will be held October 16 – 17, 2017. NORD announced that it covers two days with more than 80 speakers and 600 attendees and offers countless opportunities to connect with thought leaders, partners, patient advocacy groups and orphan product development companies. For details click on [Rare Diseases & Orphan Products Breakthrough Summit](#).

Muscular Dystrophy Association (MDA)

To understand the scale and scope of challenges faced by the neuromuscular disease community when traveling by air, the MDA surveyed its community at the end of 2016 and asked about their flying experience and received more than 2,000 responses.

Seventy percent of respondents reported that they experienced accessibility problems. MDA also had positive progress to report. Many airlines have dedicated webpages to providing guidance and support to travelers with disabilities. Also, recently the U.S. Department of Transportation has made great efforts to educate air carriers and the passenger community about not only the increase in complaints but also training and best practices when it comes to travelers with disabilities. Recently, the U.S. Senate introduced a bill that strengthens the rights of disabled passengers under the Air Carrier Access Act. To view the survey results and the senate bill, click on <https://strongly.mda.org/advocacy/> For more information about traveling with limited mobility, visit [MDA's Accessible Air Travel Resource Center](#).