

WEAR MG ON YOUR SLEEVE for June Myasthenia Gravis Awareness Month

By Tommy Santora, MGFA Communications Committee Chair,
New Orleans Support Group Leader



Amy Brill, a Myasthenia gravis patient in Levittown, New York, wears MG awareness on her sleeve - literally. Years ago, she got a tattoo on her arm spelling out "Hope" in teal letters, with the "O" shaped like a snowflake and a teal MG ribbon inside the letter. "That may not be such a good influence, but it works for me, LOL," Brill said.

While the MGFA does not encourage "tats" to promote myasthenia gravis awareness (although that's one way to promote awareness year-round), we thank Amy for her permanent dedication. But we are also here to let you know the many ways that you can spread awareness of MG, especially in June during **Myasthenia Gravis Awareness Month**, a nationwide movement created by the Myasthenia Gravis Foundation of America, Inc. (MGFA) to raise awareness to the general public of the often misunderstood and under-diagnosed disease.

continued on page 4



2018 MG Walks

TRISTATE (NYC): June 9

CONNECTICUT: June 10

NEW JERSEY: June 10

KENTUCKY: September 15

INDIANA: September 16

OHIO: September 22

CHICAGOLAND: September 23

and more!

continued on page 12



2017 SCIENTIFIC SESSION

Overview: The 2017 Scientific Session of the MGFA was held at the Annual Meeting of the American Association of Neuromuscular & Electrodiagnostic Medicine (AANEM) on Wednesday, September 13th, 2017 in the conference center of the Marriott Desert Ridge Hotel.

The meeting was chaired by Drs. Amanda Guidon, Jeff Guptill, and Mike Hehir. Drs. Guptill and Hehir were both recipients of MGFA/AAN Fellowships. There was an audience of about 200 people. The session provided a venue for young investigators to present data using talking and traditional posters.



continued on page 10



Highlights in This Issue:

- 1 Wear MG on Your Sleeve
- 1 Scientific Session
- 6 MG Strong
- 21 Genetic Associations in MG
- 24 The Power of Support Groups

For full contents turn to page 3

CEO'S LETTER



Dear MG Community Friends and Colleagues,



BE THE CHANGE

Change. It is truly the only constant, a part of every life. There are the inevitable changes—like aging. Even though we KNOW certain things WILL HAPPEN—they still somehow manage to surprise us. How did my sweet baby girls grow (practically overnight) into extraordinarily competent engineers, with families of their own? And darn it, when did those wrinkles on my face arrive?

Then there are the changes we instigate in our own lives: a new job, a wedding, a vacation. Changes we plan for.

There is an old Yiddish expression, “Mann Tracht, Un Gott Lacht.” It translates as, “Man plans, and God laughs.” Whatever your spiritual beliefs, we all know life has its ups and downs. That new job may not work out; the marriage that was so joyfully planned isn’t always happily ever after; the imagined week on a sunny beach turned out to include a hurricane. And then, there is MG.

No one plans to be diagnosed with myasthenia gravis. Or any other serious, chronic illness. In the memoir, *Tuesdays with Morrie*, sportswriter Mitch Albom recounts a series of conversations with his former professor, who had been diagnosed with ALS (Lou Gehrig’s disease). Morrie tells his former student, “Everybody knows they’re going to die, but nobody really believes it. If we did, we would do things differently...Most of us walk around as if we’re sleepwalking...doing things we automatically think we have to do.”

For me, the diagnosis of MG was that first real understanding of my own vulnerability and mortality. There was relief—finally understanding the frustrating symptoms I had lived with for so

long. And anger—that the “enlarged heart” I had been diagnosed with 8 years before was, in fact, not my heart at all, but a tumor of my thymus. (Really? What’s a thymus?) And fear—facing an uncertain future for myself and my family.

But eventually, there was acceptance. I was going to have to live with the change and whatever it was going to bring, whether I wanted to or not. And, in time, I did. Of course, there were, and continue to be, losses, but there have been gifts too—as Morrie advised. Experiencing the world more fully was the

first. Knowing that you have limitations and must make choices and set priorities eventually forces you to figure out what matters most to you—how you want to

spend your life, and being selective about who you want in it. For me, one of those gifts has been becoming a leader in the MG Community, opening my heart and my world to allow others with MG to enrich my life. It is YOU, the members of the MG Community, who inspire me to use this unplanned and unwanted change in my life to make a difference in the world for people with MG. That is the difference from, “Accept the Change,” which is hard enough, to “Be the Change,” which is transformational.

In this issue of *Focus on MG*, there are inspiring stories from remarkable people who show us what it means to “Be the Change.”

- Anaya, just 18 years old, who was diagnosed at age 12 and is now leading a movement to support teens with MG across the nation.

While only a few of us have the education and expertise to work in a research lab, each one of us has the ability to “Be the Change.” Each one of us can find our own way to change the world.

- Saadia, a mother of three who figured out how to embrace those little white prednisone pills, side effects and all, and is starting a support group in San Diego
- Tommy, who leads MGFA's efforts in raising awareness---and gives us practical ideas about how each one of us can be an MG educator.
- Kim, Rachel, and Kathie---just a few examples of the amazing people who lead MG support groups in communities from coast to coast.

In this issue of *Focus on MG* we also recognize the members of the 2017 MGFA Circle of Strength, who each gave more than \$1000 to support our mission. And we honor our top fund raisers too, who helped to make 2017 the best MG Walk year in history! MGFA is dependent on donations to support research and programs—and these people are our leaders.

And, of course, there are summaries of some of the very exciting research that continues to move us closer to a cure. We are so grateful to the scientists and clinicians who dedicate their lives to finding pathways to better treatments, better disease management, and eventually an end to myasthenia gravis. They, too, live the change.

While only a few of us have the education and expertise to work in a research lab, each one of us has the ability to “Be the Change.” Each one of us can find our own way to change the world. Ordinary people accomplish extraordinary things, when driven by passion and commitment. It is all of us, who are working so hard to live with MG every day, who will also bring us closer to a world without MG. Whether you tell your story, start an MG Walk team or a Do It Yourself fund raising event, make a donation, lead or volunteer for a support group, support a newly diagnosed person in social media, advocate for health care or research with a letter to your congressperson, you can make a difference.

Never underestimate the inspiration you are for others as you live with myasthenia gravis with dignity and courage. Your life may be different from what it was before MG---but it matters. That is what it means to “Be the Change.”

Thank you,

Nancy Law

MGFA Chief Executive

Table of Contents

1	Wear MG on Your Sleeve
1	2017 Scientific Session
2	CEO'S Letter – Nancy Law
6	MG Strong
17	Circle of Strength
18	MG Walk
21	Genetic Associations in Myasthenia Gravis
24	The Power of Support Groups
26	Patients & Doctors
29	Emergency Preparedness
30	What's Hot Off the Press in Neuromuscular Junction Disorders?
32	2017 Top Fundraisers and Top Teams
35	My Body – by Kait Masters

Catch up with the MG Community, learn more and share your story...on our website, Instagram, Facebook and Twitter.

myasthenia.org   

This publication is intended to provide the reader with general information to be used solely for educational purposes. As such, it does not address individual patient needs and should not be used as a basis for decision making concerning diagnosis, care, or treatment of any condition. Instead, such decisions should be based upon the advice of a physician or health care professional who is directly familiar with the patient.

355 Lexington Avenue, 15th Floor, New York, NY 10017-6603

800.541.5454 | 212.370.9047 fax | mgfa@myasthenia.org | www.myasthenia.org

You can simply wear an MG Walk shirt around town to encourage people to ask, “What is the MG Walk?” Or you can attend a neighborhood fair, man an educational booth on MG, and scare people (speaking from personal experience(!)) by having on display an MG Walk poster, proceeding to talk about MG, and then, as I experienced, hearing the woman, as she walks away, say “Wow, I hope I don’t get “MG-Walk” one day!”

Or on a more serious note, you can do what Daniel Schwartz, an MG patient in Alpharetta, Georgia, suggests. Daniel will often hang out with his friends and people doing physical activities that he can’t do anymore, such as play kickball. “I watched some friends play kickball because a good friend was the captain. He told teammates, ‘I was just going to watch because I could not run.’ Some people asked, and I explained it to them. ...

When describing it to people, keep it short and simple. If you make it too long, people are going to react how most people would when hearing a lecture on rocket science. And it’s human nature to tune someone out when you get confused. They aren’t ignoring you, they’re just trying to figure out what you were saying. So keep it very simple. For example, I say my muscles and nerves don’t communicate,” Schwartz said.

Or go out in the community and ask restaurants and companies to run donation collections or fundraising nights to support MG awareness; the worst thing they can do is tell you ‘No,’ Schwartz said. “But remember, MG has given you extreme mental fortitude. If a company says no, or it doesn’t work out, dust yourself off and try again.” You can find help in developing a Do It Yourself (DIY) fundraising project through the MGFA’s Classy website and by calling MGFA and requesting a DIY Fundraising kit 1-800-541-5454.

The month of awareness also serves to let people know the work being done by the MGFA to lend support to those with the condition, and how fundraising by the organization is continuing to improve research to both control the disease and to help one day find a cure. The MGFA is a very important resource for you to use not only throughout the month of June, but year round, so visit the web site and MGFA social media pages to stay up-to-date on the latest efforts of the organization and also download the latest educational materials and brochures centered around MG.

BELOW ARE SOME OTHER WAYS THAT YOU CAN INCREASE AWARENESS OF MG IN YOUR COMMUNITY:

- **Engage in Social Media:** #MGStrong will be promoted again by the MGFA this year as a Twitter and Facebook hashtag that patients, caregivers, family members, friends, doctors, nurses, etc., can add to their profile pictures to create awareness of MG. Please consider adding this hashtag to your profile photo and maintaining that photo through the month of June. Also share the photo among your social media friends and encourage them to also champion our cause. Close to 10,000 people used #MGStrong last year.
- **Promote Your Support Group Meetings:** Reach out to your Support Group leaders and encourage them to schedule a meeting for June, and center the meeting topic on “MG Awareness” - What is MG? What medications/treatments are available to control MG? What current research is being conducted to expand MG treatments? - At least a month before the meeting, publicize the meeting in libraries, grocery stores, neurologists’ offices, pharmacies, social media, newspapers, etc., and try to target more people by saying, “New MG Patients and Caregivers Welcomed.” Even your normal group participants will attend to hear an update on the latest treatments and research, and new patients or new support group members may be motivated or encouraged by “MG Awareness” month as a reason to attend.
- **Find A Support Group and Attend a Meeting:** You can find a list of the MGFA support groups at: <http://myasthenia.org/CommunitySupport/SupportGroupCalendar.aspx>



- **Our national network of MG support groups grew to more than 60 in 2017, with 20 new leaders trained, and a few groups shared by MGFA and local MG organizations.** The new Patient and Community Services Committee launched several initiatives - perhaps the most exciting of which is the long-awaited MG Friends program - which now has 17 active volunteers who ensure that every new contact receives a follow up call, and that no one with MG, no matter where they live, has to face this disease alone. This committee also formed a council for young adults — which is rapidly growing under the name of Myasthenia Advocacy for Young Adults (MAYA).

- **Inform People Close to You of MG:** Not only among your family and close friends, but create awareness about MG in your workplace, organization, school, or other avenues, by explaining to people what MG is, and how it affects you. When someone you encounter casually looks at you doubtfully perhaps because of eyelid ptosis or your inability to smile, take this as a teachable moment. Try to explain succinctly. You might say, “I see you are wondering why my eyelids seem so droopy (or why I’m wearing an eye patch, etc.). I have MG. It’s an autoimmune disease that causes muscle weakness. This is one symptom. If you are curious you can learn more at www.myasthenia.org.” Become the teacher, and share your knowledge to educate people around you.



- **Distribute Posters, Flyers and Educational Materials:** Please be on the lookout from the MGFA for the 2018 June Awareness Poster. You can print the poster from <http://www.myasthenia.org/LivingwithMG/InformationalMaterials.aspx>. Find it listed just below the brochures. Or, call MGFA at 1-800-541-5454 to request a supply. Bring that poster to libraries, grocery stores, neurologists’ offices, pharmacies, etc., and ask them to display the poster throughout the month. Also, feel free to request from the MGFA any brochures you would need to share with distribution sites as well.



- **Participate in/Support an MG Walk:** The MG Walk Campaign, having raised more than \$5 million in its first seven years, is dedicated to creating awareness, renewing hope, and generating a vast network of community and support, all while raising important funds for the MGFA. Taking place annually in more than 30 cities nationwide, the MG Walk puts the power directly into the hands of MG patients, and allows everyone battling this illness to become the driving force behind funding for MG research, and ultimately, in finding a cure. In addition to fundraising, the MG Walk also allows patients with MG to open up and discuss their journey in a safe and nurturing environment, many for the first time, while experiencing an overwhelming sense of community from their loved ones and fellow MG patients. Find a Walk near you, participate, fundraise and spread the word. Walks take place every year from March-December, so there are plenty of opportunities to participate in the MGFA’s annual fundraiser.



- **Contact Your Local Representatives:** Request City Proclamations: A proclamation is a formal way to make a public announcement. Mayors, council members, or local government administrators write proclamations to commend people or to announce upcoming events, and one way in which many MG patients have succeeded in years past, is getting local and statewide proclamations of June as MG Awareness Month. Identify the appropriate contact person in your state and local government. Governmental office web pages will provide address, telephone and e-mail information. Many state web sites have a proclamation request link for you to submit your request electronically or download forms.



- **Contact Office Buildings to Light Up the City “Teal:”** Under the innovative initiative of MGFA Board Member, Patient, and Curing MG with Duct Tape Promoter Celia Meyer, we lit up the sky in 2017. Celia led the charge of many MG support group volunteers asking corporate buildings in

continued on page 6

various U.S. cities to light up their buildings in the color teal to celebrate MG Awareness Month in June. Cities participating in the light up campaign this past year included Mobile, Atlanta, Chicago, Cleveland, Tampa, and Los Angeles. Contact the MGFA for more information on how you can ask your office buildings to light up the skyline teal, or visit the various office buildings web sites to inquire about community support.

Last, but not least, if you haven't done so already, join the MG Patient Registry



at <https://mgregistry.soph.uab.edu/MGRegistry/PortalLogin.aspx>. 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, former Chairman of the 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. If you have already joined but have not updated your information, please also consider doing so. When Nancy Law, the CEO of MGFA, talks with drug companies contemplating or launching trials, our Patient Registry enrollment of more than 2,300 for a rare disease is a major factor, and that number has more than doubled over the last year. It may not sound like a lot to us, and we want to continue to grow, but it is a major accomplishment for the organization.

Whatever you decide to do for **June is MG Awareness Month**, do not forget that you're not alone in this journey to raise MG awareness. Do not hesitate to ask a fellow MG patient what has worked for him/her in the past to spread awareness, and use that information to take on your own initiative. There is a lot of good news to spread about the MGFA, and how your organization has tremendously improved the scope and outreach of our MG community and support system. As you battle every day, continue to realize that there is significant work going on behind the scenes to help us one day find a cure for MG, and please do your best to let people know about it! ✨



Do You Want to Wake up to a World without MG?

YES?

Then help by joining the MG Patient Registry

The MGFA Patient Registry is helping to expand our knowledge of MG and move us closer to improved treatments and a cure. By making a patient community more accessible and understandable, a patient registry and its bounty of information can encourage pharmaceutical developers to pursue drug discovery in a disease.

To learn more about the registry please visit www.myasthenia.org home page banner and click on the banner when it turns to MG Patient Registry. Or, call the MGFA office at (800) 541-5454 and request the *MG Patient Registry* brochure.





My MG Story — Anaya Mitchell



My illness hasn't changed, every day is still unknown. However, I now approach every day as a chance to write my own story rather than let my illness write it for me.

When I was diagnosed with myasthenia gravis at 12 years old, I was devastated. Playing sports; swimming, basketball, soccer and baseball; were a major part of my life. I'd played sports since I was four years old and it was a major part of my identity to family and friends. At 12, I began to notice my limbs were weak and I couldn't move as quickly. Everyone wondered if my passion for sports was changing because I was becoming a teenager. However, I continued to enjoy sports but my body was not cooperating.

After a year of wondering about my physical condition and going to various doctors, I was diagnosed with myasthenia gravis. Great, I said aloud, "now I can get some medication to make *this* go away". Knowing that life was going to return to normalcy was always in my mind as I received my diagnosis. I didn't realize how this disease could strip away my physical and mental abilities in such a short amount of time. Once diagnosed, I was given Mestinon and steroids as medications for my physical condition and my parents found a therapist to help me cope with my condition.

As a middle school student, I wanted to be like my middle school friends; attend games, walk home from school, go to parties and dance. All of these activities were difficult to do because my arms and legs were weak and I needed to rest so my body could function in school and home.

I kept wondering why this was happening to me, but my family continued to keep me in good spirits and allowed me to attend as many social activities as possible, so I could feel like a regular middle school student. However, after one year of Mestinon and steroids, my parents and I decided to have a thymectomy surgery to see if my physical condition could improve. Again, this did not provide the expected result, but I continued to have hope.

Attending high school brought a new set of challenges and an awareness of my capabilities in spite of my illness. In high school, my mom wrote a letter to request special education and related services. Initially, the school denied the request because we had a 504 plan and they thought it had

enough services and supports for me to be successful in high school. However, after the first marking period, I missed 25 days of school because I couldn't physically walk the building even with an elevator pass to go from floor to floor. We had another meeting to determine medical records, and evaluations were needed for eligibility for special education. We provided medical information to the school and the school gave me tests. At the final meeting, the CST determined special education was right for me because of my illness. I was given an IEP that provided home instruction when I was absent for a period of time, transportation because I could not walk like other students, modified assessments because of my health, additional time for tests and assignments and Pass/Fail for some of my elective classes.

My school district was very accommodating due to my illness. Having my hometown group of friends push my wheelchair, help me in school and make sure I don't feel like an outsider due to my many absences is important. As a teen you want to see and be a part of everything that happens but my health did not always allow it. Although, everything was falling into place for school, my health didn't get better, so, we moved to Rituximab treatment. Currently, we are continuing to await results but we continue to have hope and faith.

Six years later, I've accepted my diagnosis of MG and decided to help other teens through their diagnosis. Although I continue to have periods of physical weakness, I haven't let my illness overshadow my dreams. Participation in the annual MG walks, NY support group, and meeting with regional staff have helped me recognize that the best way to retain my positivity is to work with

continued on page 8



continued from page 7

others who have the diagnosis. Attending the NY MG Support Group has allowed me to watch and learn how a support group should be for teens. My ultimate goal is to initiate a regional or national teen support group to help others who struggle through this diagnosis. I was fortunate to have my family navigate high school and college options to ensure all options were viable. My illness hasn't changed, every day is still unknown. However, I now approach every day as a chance to write my own story rather than let my illness write it for me.

Saadia's Story — I Have MG

A friend of mine emailed me recently about taking Prednisone for a possible autoimmune disorder that she may have. She read that people with MG often took this medication and she wanted my thoughts on it. For me, Prednisone is a miracle drug. It allows me to talk, to walk, to breathe, to laugh, to smile, to swallow, and to live as normally as someone who has never taken a drug in his life. So I told her, yes, don't worry about the side effects. Don't suffer because you are too afraid to take a drug that does cause side effects, but on the flip side, what are you losing out on if you don't? Life.

I have 3 young children. *I have MG.* I work as a substitute teacher for active kids with disabilities. *I have MG.* I take an average of a 5-mile hike with my husband every week. *I have MG.* I drive just as much as an Uber driver everyday with and without traffic. *I have MG.*

Am I on drugs to keep my MG under control? Yes. Would I have it any other way?

As with many people who have MG, there are the good days and the not-so-good days. In my definition, there are no "bad" days unless you are on a ventilator.

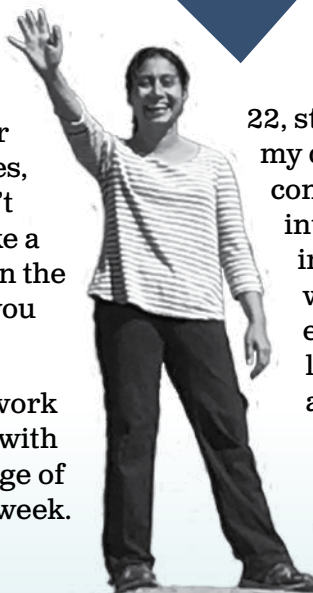
No. I have a strong faith and no one knows what tomorrow will bring. But if today, I can do all my tasks and take care of my family and actively raise my children, then I don't mind taking the Prednisone and dealing with the osteopenia that I developed in my early 30s or the bouts of depression that thankfully have now disappeared, knock on wood. And if I get cataracts a few years earlier or if I have to start a higher dose and gain weight, so what's the big deal? I'll add on another hiking day.

As with many people who have MG, there are the good days and the not-so-good days. In my definition, there are no "bad" days unless you are on a ventilator. When I was at my worst, when I could not take a step or swallow or breathe, yes, I would feel like I was better off dead. But I would

also read the news to find out what's happening in the world. And that puts everything into perspective doesn't it? When I read stories of people hurt or dying in a warzone, when my eyes passed an article's headline about a massive earthquake, or when I saw the statistics on the rising rates of cancer and heart disease, it gave me hope. I could have been a lot worse.

I started off with ocular MG when I was 22, straight out of college and searching for my dream job. Wide-eyed and idealistic, I was confident I'd land a job working for a major international NGO where I could set off to improve the plight of the poor across the world. I had a slight problem though. At each interview, I could not lift my eyes to look at the hiring manager for more than a few seconds at a time. Not a great first impression, no phone calls followed.

Thinking it was my eyeglass prescription, my mom made an appointment with the ophthalmologist but my number had



only slightly changed. So the doctor referred me to a neuro-ophthalmologist because he found my eyelids to be droopier than usual.

At the Neurology Center, Dr. David Katz, a tall lean man with wire-rimmed frames, didn't take long to offer an accurate diagnosis, confirmed with blood work and tests that sent electrical shocks up my hands and arms. It was 1999 and the statistic he quoted of people with MG, 1 in a 100,000, made me think I should try my luck at the lottery too. He prescribed Mestinon, a pill that immediately opened up my eyelids and got rid of my one-sided smile. He said it worked like a Tylenol, alleviating the symptoms but not the cause. Unfortunately, there is still no known cause for MG.

Mestinon was my savior for a year. By the end of 2000 though, it was not sufficient. The initial ocular MG was generalizing to other muscles throughout my body. The double vision was more intense so I had to wear special lenses over my glasses to alleviate the condition. Driving was out of the question, not that I had a car to begin with. But more unsettling than the double vision was the difficulty in swallowing. ***I found myself sitting at the dinner table for literally two hours one evening trying to swallow a couple of meatballs.*** And so began my love affair with Prednisone. Symptoms improved but all I could think about was food even

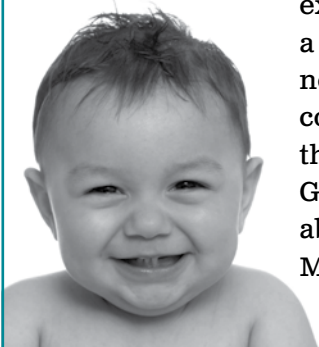
in my dreams. What to eat, where to eat, when to eat, and how much I could eat. The pounds started piling up faster than the food on my plate at any given meal.

I've kept an Excel spreadsheet on my MG, how it's progressed and regressed over the years, which medications I've tried and tested, which have failed (for example, Imuran didn't work because my liver enzymes went haywire), and which I've kept safely guarded in my medicine cabinet since MG came into my life. The two constants are Mestinon and Prednisone. On my graph, the chart for Prednisone over the past 17 years looks like a seismograph during an earthquake, peaks and troughs and jagged peaks again. But thanks to this medication, I have a life. It's not an easy journey, although currently I'm doing better than I ever have. A glance at my graph proves it.

My neuro-ophthalmologist, Dr. Katz, once said that the practice of medicine is more of an art than a science. I like to believe he meant it's individualistic, and not every case can be filed under the "general" category. Every person is unique, everybody is different, every symptom can be unusual, and every reaction to a drug can have countless manifestations. For me, I know with certainty that I will always have a special "high" every time I open up a bottle of my medicine and see the pure white tablets waiting to be picked up. To me, they are more than just medicines; they are the reason I am alive. ✨

SMILE FOR MGFA

Did you know you could donate to the Myasthenia Gravis Foundation of America while shopping on Amazon? It's simple to set-up on an existing account or by creating a new one. You can shop as you normally do, there's no change in cost or convenience to you. Tens of thousands of products are covered. Go to <http://smile.amazon.com/> about to learn more and make MGFA your charity!



Join the fun and use MGFA's new Twibbon to promote awareness of MG.



Go to <https://twibbon.com/support/mgfa-june-awareness> to use the new Twibbon on your social media sites.



continued from cover

Posters give new investigators an opportunity to present their work without the pressure of standing in front of hundreds of people. I liked the attention paid to posters, which enable young investigators to present their work and get feedback. In the text below I provide brief discussions of the presentations. The abstracts can be viewed on the MGFA website.

PLATFORM PRESENTATIONS

KEYNOTE PRESENTATION

Incorporating Outcome Measures into Clinical Practice

S Muppidi, Stanford Univ, Palo Alto CA

This talk was aimed to encourage clinicians to use standardized measures to evaluate how MG is affecting a patient, how severe the symptoms are, and the direction of the clinical course (improve or worsen in response to a new intervention). The first described was the Quantitative MG index (QMG) which assesses 13 items that are graded in 5 increments from absent to severe. The score ranges from 0-39. A 3 point change is considered to be significant. Manual muscle testing (MMT) evaluates the strength of 18 selected muscle functions. MMT is one of the first measures used to assess the severity of MG. MG-ADL (activities of daily living) assesses how MG is affecting a person's ability to function independently. The MG-ADL is a good tool for following disease severity. MG-QOL (quality of life) measures how MG is affecting a patient's ability to carry out tasks required to live independently. The current version of MG-QOL evaluates 15 items. The MG composite score combines elements of MG-ADL, QMG and MMT to assess the severity of MG. The MG impairment index is being evaluated to comply with FDA guidelines used to evaluate medication efficacy (Neurology 87: 879-886, 2016). This measure has been validated (measures what it is supposed to measure and the score reflects severity of disease determined by other measures). The responsiveness of the MG impairment index to clinical changes is being assessed now. These indices are best if used from the beginning of caring for a patient - continued



use of these measures provides a "clinical video" of how a patient's MG is changing and responding to different interventions.

TALK 1

A Redcap Database for the MG Clinic and Multicenter Collaborations

D Sanders (Durham, NC), M Small (Durham, NC), S Budinger (Durham, NC), J Guptill (Durham, NC)

This talk was essentially the implementation of the Keynote talk. The group at Duke recognizes that the Electronic Medical Record (EMR) is becoming the standard in American healthcare and they have devised a tool (REDCap (Research Electronic Data Capture) to enable a uniform format for collecting information about MG which will allow information about patients from all over the US to be integrated into a single data set. This tool will facilitate national studies of MG as well as to encourage multisite clinical trials involving MG. The information that can be collected by this tool will complement the data in the MG Patient Registry about the distribution of various forms of MG. Patients who really want to help advance the understanding and treatment of MG are encouraged to enroll in the Registry. The trick is to get the REDCap system to work within the EMR due to the variety of EMR systems in use. An even greater limitation is that the EMR used in many large health care systems, known as the EPIC system, is not easy to alter because EPIC is optimized for billing, not collecting patient information in a manner that will be easily used for clinical studies.

"Patients who really want to help advance the understanding and treatment of MG are encouraged to enroll in the Registry."

continued on page 19



2017 Circle of Strength

We have been fortunate to be blessed by the generosity of many individuals and families who have written checks and made bequests to help in the fight against MG. This is a list of all those who have made contributions over \$1,000 in 2017. Thank you to these generous supporters, our Circle of Strength. Thank you too, to all of you who have given to the Foundation at whatever level, and to all those who give their time and energy to our cause. Thank you!



\$25,000+
Anonymous
Benefactor
(\$50,000)

\$10,000
– \$24,999

Mark & Kathleen
 Aitken-Cade
 Walter Capp
 Brian Gladden
 Thomas Larsen

\$5,000 – \$9,999

Nancy Law
 Marcia & William
 Lorimer
 Edward Walsh
 Darrell and Linda Webb

\$2,500 – \$4,999

Jack Ambrosio
 Janet Berkley
 Gregg Brody
 Hannan Chaugle
 Judith Craver
 Steven Grant
 Patti & Leo Kessel
 Nelson Machado
 Kirk Munsch
 Tatsuji Namba
 Nancy Gills
 Robert & Suzanne Ruff
 Susan Klinger
 & Sheldon Gartenstein
 James & Sandi Thompson

\$1,000 – \$2,499

Terese Ackermann
 Dinah Afsahi
 Dheeraj Ahuja
 Michael Alderman
 Joann Andruk
 Ardith Breneman
 Gavin Brown
 Roy Carroll
 Nona Hasegawa
 & Stew Cogan
 Sherry & David Cole
 Ken Crerar
 David Croonquist
 Virginia Cunningham
 Christopher D’Agostino
 Charles Daniels
 Thomas Defanti
 Charles Defanti
 Faith Deleon & Pastor
 Norman Harding
 Larry Dix
 Charles E. Durney, Post 27
 Gary Eder
 Robert Elliot
 Brad Eschman
 Tricia Foerster
 Andriana Friesen
 Sylvia Fuhrman
 Leni Fuhrman
 Keyyanna Genz
 Russell Gibbs
 Roberta Greenberg
 Evan Greene
 Harriet Griesinger
 Kathleen Halston
 Robert E. & Lois J. Hughes
 Richard Jeffery
 Robert Johns
 Julia Johnson
 Sarah Kiesling
 Brian Klaponski
 William Kuhn
 Linda Kusner
 Rollin and Sharon Lacy
 Carole Larkin
 Sandra Larkin
 Gabriela Lens
 Yuebing Li
 Helen Machado
 Maria Martini

\$1,000 – \$2,499

Kathy and Ed Mateer
 Rich Mauch
 Paula McGinnis
 Robert McPheeters
 Jackie McSpadden
 William Merritt
 Celia Meyer
 Donald Miller
 Christine Moldauer
 Joan Monk
 Morgan and Morgan
 Charles Mowbray
 Beth Nash
 Rebecca Nielson
 Deborah Oeder
 Kevin & Becky O’Leary
 Colleen Parsons
 Jeff Pilgrim
 Stephen Poff
 Jennifer Reynolds
 Lizette Rivera
 Michaeline Roach
 Anne Rubin
 Corey Russell
 Donald Sanders
 David Saperstein
 Samuel Schulhof
 Arlene & Judith Schwartz
 Betty Shine
 Pasqua Simone
 Margot Slater
 Christina Smith
 Steven Snow
 Don Somers
 Jackie Spencer
 Leona Stenholm
 Gary Strauss
 Arthur Strauss
 Mark Swift
 Elizabeth Swize
 Ellen and Larry Teaff
 Bradley Tusk
 Thomas Ursic
 Richard & Julia Webb
 Allan Weiss
 David Wind
 Richard Woodie
 Gaynell Wortman





Register For The 2018 MG Walk Season TODAY!

The 2018 MG Walk season is off to a great start with successful events in New Orleans, Florida and Georgia and we are well on our way to reaching our \$900,000 goal! If you haven't already, now is the *perfect* time to reactivate your team, or start one for the first time and help us to bring even more resources to the MG community! For a list of all the upcoming Walks this spring and fall and to register, please go to MGWalk.org.



NEED IDEAS ON HOW TO GROW YOUR TEAM?

Check out our new "Team Captain Tips - Recruitment & Retention" resource on the MGWalk.org/tools website. This document is filled with creative ideas on how to retain your past team members and or look to recruit new members!

The MG Walk Campaign is the flagship national awareness and fundraising campaign for the MGFA, and the perfect opportunity to become active in the amazing MG community, and help us get one step closer to our ultimate goal... a world without myasthenia gravis.

LAUREN JARMAN 2018 NATIONAL MG WALK HERO

"The MG Walk represents opportunity for everyone affected by MG to get involved, share their stories, and spread awareness to those that don't understand what it is like to live with this sometimes invisible disease."



2018 MG Walks

- TRISTATE (NYC): June 9
- CONNECTICUT: June 10
- NEW JERSEY: June 10
- KENTUCKY: September 15
- INDIANA: September 16
- OHIO: September 22
- CHICAGOLAND: September 23
- PORTLAND: September 29
- SEATTLE: September 30
- SOUTHERN ILLINOIS: October 13
- VIRGINIA: October 13
- MARYLAND: October 14
- DELAWARE VALLEY: October 20
- GREATER LOS ANGELES: November 17
- HOUSTON: November 17
- SACRAMENTO: November 17
- AUSTIN: November 18
- BAY AREA: November 18
- SAN DIEGO: November 18
- ARKANSAS: December 1
- ARIZONA: December 2
- COLORADO: TBD
- GETTYSBURG: TBD
- MISSISSIPPI: TBD
- NEW MEXICO: TBD
- UTAH: TBD

VIRTUAL: ANYWHERE, ANYTIME!





CELEBRATING 8 YEARS OF RAISING AWARENESS AND FUNDS FOR THE MG COMMUNITY!

NEW RESOURCES TO TAKE YOUR FUNDRAISING TO THE NEXT LEVEL!

The MG Walk Team wants to make sure you have all of the tools necessary to have the most successful fundraising season yet! Go to the MGWalk.org/tools and check out the new resource “Tips For Fundraising”. Within the document you will find tips & tricks to help guide you and your team in your outreach and fundraising.

DONATE



GAVIN BROWN 2018 NATIONAL MG WALK MEDICAL AMBASSADOR

“I have been involved with the MG Walk for 5 years including as the 2017 Georgia MG Medical Walk Chair. My patients, fellow physicians, nurses and staff at the Laureate Medical Group and Northside Hospital have all embraced what the MG Walk means to the MG community. Each year I am honored to walk alongside the many persons with MG and their family & friends. I strongly encourage the MG medical community from around the country to support the MG Walk.”



STAY CONNECTED   

MGWalk.org | 1-855-MGWalks (649-2557) | Info@MGWalk.org | #mgstrong

Genetic Associations in Myasthenia Gravis

Ricardo Roda MD/PhD, Assistant Professor of Neurology
Johns Hopkins School of Medicine



One of the more interesting questions I am asked by patients in clinic, after I make a diagnosis of myasthenia gravis, is “Will my children also have this?” Or, “Will I pass this on?” What is at the heart of this question is whether in fact there is a genetic factor, or genetic susceptibility to developing myasthenia gravis.

This is a rather complex question, and there are several ways to look at it.

There are of course genetic disorders which mimic typical myasthenia gravis. They

result from mutations in genes whose products make up the neuromuscular junction. These disorders are generally present from birth or infancy, and are called “congenital myasthenia syndromes (CMS),” and do not respond to immunosuppression. In some cases, CMS may remain undiagnosed for several years. Recent advances and availability of genetic testing has made the diagnosis of these syndromes less challenging. Nonetheless, clinical suspicion as well as awareness of their presentation remains important in making the diagnosis.

“Will my children also have this?”

A more difficult question is whether acquired, or auto-immune myasthenia gravis, has a genetic component, or as I am often asked, whether it “runs in families.”

There are several documented cases in the literature of families in which the diagnosis of myasthenia gravis amongst its members is more common than one would expect. It implies that in some families, there are inherited factors that are passed along which make the development of myasthenia more likely. However, and this is an important point, the presence of these factors alone is not sufficient for the disease to be present. They merely increase the likelihood of something triggering the disease.

One way to determine if there is a familial component to myasthenia gravis, is to simply look at patients with the disease and then to determine if other family members are also affected. As far back as the 1970s, and using patient cohorts from Finland (264 patients) and the United States (702 patients), a familial occurrence for myasthenia gravis of between 3.4-7.2 % was well established.^{1,2} These studies included first-degree relatives (parents, children and siblings) as well more distant relatives (cousins). The numbers indicate that the probability that someone in your family will have myasthenia gravis, if someone else already suffers from it, is higher than one might expect for the general population. These numbers have more recently been confirmed in a study from Spain (462 patients) which showed a prevalence of 3.46%, while a larger study from Taiwan (6638 patients) estimates that there is an almost 8 fold higher chance that one may develop myasthenia if there is an affected relative.^{3,4}

These numbers are well below what one would expect for genetic disorders in which mutations in a single gene leads to disease. What they do suggest however, is that there are certain genetic factors (likely more than one) that increase the risk for developing myasthenia gravis.

Once it was established that there is in fact a

genetic component to MG, then the ensuing question became which specific set of genes could be responsible. This becomes a clinically relevant question as one would hope that new therapeutic targets could be developed based on the answer to it. One would expect, given the nature of the disease, that genes involved in the regulation of the immune system would be highly suspect in affecting the risk of development of myasthenia. The immune system relies on a complex set of checks and balances, to ensure that an immune response is unleashed only on a specific target; hopefully foreign agents trying to infect the body.

One way to look at genetic factors and disease correlation is through genome wide association studies (GWAS). These studies are able to look at single DNA changes known as polymorphisms (SNPs) and to compare them between groups, one of patients with disease and another of “controls”, i.e. people who are not affected. If there are specific changes that correlate with the disease, then the genes associated are identified as possible risk factors. This approach has been used in other neurological disorders such as multiple sclerosis (MS) and amyotrophic lateral sclerosis (ALS) to successfully identify genetic associations. In general, the quality of such studies depends on the number of patients enrolled (usually many thousands), as well as how much the members of the disease or affected group are like each other. In other words, it’s easier to spot DNA differences between patients and controls who are otherwise from similar backgrounds.

Three large GWAS studies were carried out over the past few years for myasthenia gravis, either looking at early-onset (EOMG), late-onset (LOMG) or both groups as a whole.

An early GWAS study, which enrolled 649 northern European participants, and which focused on patients with EOMG revealed an association with genes in three areas designated in *TNIP1*, *PTPN22* and *HLA-B*08*.⁵ *TNIP1* has an ubiquitin binding domain and is known to interact with the members of the NF- κ B family, while the *PTPN22* SNP had already been described in association with a loss of the ability to remove auto-reactive B cells.⁶ The HLA variant suggests a role for cytotoxic T-cells restricted to this HLA gene, as causative of myasthenia gravis.

The second study was an international GWAS

study, also looking for genetic variants that alter susceptibility to myasthenia gravis. The study looked at a cohort of 1032 white individuals with acetyl-choline receptor antibody positive myasthenia gravis. Its results were then confirmed in a cohort of 423 Italian patients with myasthenia. This study identified variations within or in proximity to three genes as increasing the odds of developing myasthenia: *CTLA4*, *HLA-DQA1* and *TNFRSF11A*.⁷ The authors also stratified their population amongst early and late onset MG and searched for SNPs which may show an age related preference.

One way to look at genetic factors and disease correlation is through genome wide association studies (GWAS).

CTLA4 is a molecule that is expressed on the surface of T-cells. T-cells play important roles in mediating the immune response in myasthenia gravis. CTLA4 acts as a “brake pedal” in the immune system, tending to reduce or turn off the immune response. When CTLA4 is knocked out genetically in animals, it leads to animals with overactive immune systems, which suggests that a defect in CTLA4 may predispose to the development of MG. An antibody that blocks CTLA4 (*Ipilimumab*) is currently used to activate the immune system for treatment of recurrent melanoma. Patients treated with this antibody can develop autoimmune diseases as a side effect of blocking CTLA4, including myasthenia gravis. This serves as a confirmation of the GWAS results. It should also be mentioned that an IgG1-CTLA4 hybrid antibody (*abatacept*) that blocks activation of CTLA4 is currently approved to treat rheumatoid arthritis, and there are current clinic studies to assess its possible effectiveness in myasthenia gravis (D. Drachman, personal communication). It has been very effective in treating experimental myasthenia gravis in laboratory animals.

The HLA-DQ molecules play an important role in presenting molecules to the immune system to trigger an immune reaction. Four SNPs were found to be associated with disease in this study.


continued on page 18

The Power Of Support Groups

Support Groups are instrumental in how MGFA and many other MG organizations deliver service to the community. Support Groups bring together people who share common life experiences and offer support, resources, and educational programming as well as social and recreational activities.


Understanding the power in Support Groups keeps the MG community connected to each other, resources, great doctors and information. It's also an opportunity to.....

Share Your Story




Support Groups are places where we share our stories and we listen to the stories of others. Support Groups provide this opportunity to share your experiences openly and freely in a safe setting. Sharing your journey will not only offer you a sense of empowerment, but will help others in finding reassurance and learning new strategies to living with MG.

Learn from Professionals



Support Groups offer educational programming and invite guest speakers directly from your community to present on a variety of topics. There are opportunities to learn about exercising techniques, insurance information, wellness, diet, etc.

Offer Support to your Family & Friends



Support groups are led by the community and naturally become family-oriented. In certain situations, we typically turn to our family and friends first, but they may need support too.

Meetings are an opportunity for your family, caregiver and / or friends to learn more about MG. While we encourage family to attend meetings, there are also social and recreational events to invite your loved ones to! Holiday events, luncheons, summer BBQ's, ice cream socials, local MG Walks and more !

“Stories make us more alive, more human, more courageous, more loving.”
– Madeleine L’Engle,
Author

To join a Support Group near you, visit MGFA’s Support Group Calendar for a listing of local meetings (link). Although MGFA has a growing network of Support Groups across the country, if there isn’t one near you, ask your doctor about the nearest hospitals and facilities that may host meetings that are not under an organization. Our calendar also lists or links to some support groups managed by local MG organizations.

***If you do not see an MG organization and Support Group on our site and would like it to be listed, feel free to send us an email mgfa@myasthenia.org*

A MESSAGE FROM OUR SUPPORT GROUP LEADERS...



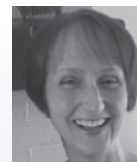
Rachel Adam-Pegram
Support Group Leader
Charlotte, NC MG
Support Group

“I have had MG for 30 years. I feel that having a Support Group locally is essential for patients. It’s an opportunity to meet people in real life who have MG and also have a good support system available, for them, in their own city!”



Kathie Bibeau
Support Group Leader
Pacific NW MG Support
Group

“I enjoy being a Support Group Leader in the MG community because it allows me to share my experiences and to connect with patients and their families assuring them that they are not alone in their journey.”



Kim Eldridge
Support Group Leader
Kentucky Support
Group

“Volunteering with the MGFA has been a wonderful way to give back to the MG community. I enjoy helping educate other people with MG about the disease and treatments, as well as how to advocate for themselves.”

OUR MG FRIENDS ARE HERE TO SUPPORT YOU!

MGFA's patient-to-patient phone support program, **MG Friends**, helps to ensure that everyone in the community gets the information they need, and know they are not alone in living with myasthenia gravis. With the programs launch this past summer, we have connected over 80 people with MG to our highly trained and professional phone support volunteers!

While support groups are critical to service delivery, there are many people who are not able to access this service or who feel more comfortable talking one-on-one than sharing in a group setting. MG Friends provides the opportunity for a person with questions and concerns to connect to someone else with MG—no matter where they live in the United States. Are you or someone you know looking for phone support? Here's what you need to know:

WHEN does an MG Friend help?

Phone support services are open to people with MG, caregivers and family members. Common reasons for connecting with an MG Friend include:

- Being in your first year of diagnosis.
- Experiencing fear, confusion, a sense of isolation, and concerns about the future.
- Life changes or worries: employment, family, relationships, communication, etc., related to MG
- Worried about treatment side effects.
- Coming out of remission, or experiencing new symptoms or changes.

continued on page 18

MEET SOME OF OUR DEDICATED MG FRIENDS...



Calvin Arnason, MG Friend

"In my nearly 30 years of major adventures with Myasthenia Gravis I have been literally LIFTED UP and UPLIFTED by many, many others in so many ways - my volunteering for MGFA is a very small attempt at payback for what I have received."



Nadine, MG Friend

"I was diagnosed with MG about 20 years ago. I had not met anyone with the disease until a year ago when I started attending the New York MG Support Group. A few months ago I became an MG Friend and it has been an overwhelmingly amazing experience. I love that I can lend an open ear to someone who does not have easy access to a support group due to distance, etc. I can help them locate the proper websites for gathering information and platforms that will teach them how to cope with the unpredictable nature of the disorder."



Andy Waymouth, MG Friend

"I was diagnosed at 17 and have been diagnosed for 14 years, 13 years were only ocular symptoms but the past year I have seen generalized symptoms. Being a volunteer means that I get to help people during the rougher times in their life, and show them that life isn't over after diagnosis. It also means I get to see other people through all walks of life and remind me on my bad days that there is a large group of people who can help me and understand what I am feeling."

Genetic Associations continued from page 15

An interesting result is that when the investigators stratified their patient population based on age of onset, a different set of HLA-DQ molecules were associated with each cohort, suggesting that age of onset may also be associated with underlying genetic variability in the immune system. Other HLA associated genes have also been reported in association with myasthenia gravis, for example HLA-DRB1 is associated with juvenile myasthenia gravis.⁸

TNFRSF11A which encodes the receptor activator of nuclear factor- κ B, is a risk gene in patients with late onset myasthenia gravis.⁹

In summary the currently available evidence points to a significant genetic component to developing myasthenia gravis. The empiric data from family studies indicates that the risk of developing the disease is higher in those who have affected family members, but the number is still fairly low. There is no single gene that can cause someone to develop myasthenia gravis, and as such it should still be considered a “sporadic” disease. Studies with large numbers of patients have been able to shed some light into a hitherto inscrutable problem of identifying the genetic factors. However better and larger studies are still needed to determine the most important genes that increase the risk of developing myasthenia. ✨

References

1. Namba, T., Brunner, N. G., Brown, S. B., Muguruma, M. & Grob, D. Familial Myasthenia Gravis: Report of 27 Patients in 12 Families and Review of 164 Patients in 73 Families. *Arch. Neurol.* 25, 49–60 (1971).
2. Pirskanen, R. Genetic Aspects in Myasthenia Gravis. *Acta Neurol. Scand.* 56, 365–388 (1977).
3. Liu, F.-C., Kuo, C.-F., See, L.-C., Tsai, H.-I. & Yu, H.-P. Familial aggregation of myasthenia gravis in affected families: a population-based study. *Clin. Epidemiol.* 9, 527 (2017).
4. Salvado, M. et al. Study of the prevalence of familial autoimmune myasthenia gravis in a Spanish cohort. *J. Neurol. Sci.* 360, 110–114 (2016).
5. Gregersen, P. K. et al. Risk for myasthenia gravis maps to a 151Pro Ala change in TNIP1 and to human leukocyte antigen-B*08. *Ann. Neurol.* 72, 927–935 (2012).
6. Menard, L. et al. The PTPN22 allele encoding an R620W variant interferes with the removal of developing autoreactive B cells in humans. *J. Clin. Invest.* 121, 3635–3644 (2011).
7. Renton, A. E. et al. A Genome-wide Association Study of Myasthenia Gravis. *JAMA Neurol.* (2015). doi:10.1001/jamaneurol.2014.4103
8. Popperud, T. H., Viken, M. K., Kerty, E. & Lie, B. A. Juvenile myasthenia gravis in Norway: HLA-DRB1*04:04 is positively associated with prepubertal onset. *PLOS ONE* 12, e0186383 (2017).
9. Seldin, M. F. et al. Genome-Wide Association Study of Late-Onset Myasthenia Gravis: Confirmation of TNFRSF11A and Identification of ZBTB10 and Three Distinct HLA Associations. *Mol. Med.* 21, 769–781 (2015).

Support Groups continued from page 17

WHAT does an MG Friend volunteer provide?

Through phone and email communication, our MG Friends provide:

- Confidential conversations.
- Someone with MG experience, who will listen actively and unhurriedly.
- Techniques to overcome the day-to-day MG challenges.
- Local resources that may be helpful (free or low-cost transportation, food banks, friendly visits, support groups, MG Walks).
- Information and answers to FAQ.
- Neurologist names and contact information from the MGFA Physician Referral List and / or the Partners in MG Care program.
- Comfort, empathy and emotional support.

WHO are MG Friends?

Our MG Friends are men and women of different ages and experiences with both life and with MG. They have at least two years of experience living with MG (either as a person diagnosed with the disease or a family member). They ...

- Demonstrate empathy and a sincere desire to help others.
- Present good listening skills.
- Satisfactorily complete the MG Friends training program.





continued from page 10

TALK 2

Efficacy of Eculizumab is Sustained over 52 Weeks in Patients with Achr+ Refractory Generalized Myasthenia Gravis (Gmg): Interim Results from the Open-Label Extension of Regain

J Howard (Chapel Hill, NC), J Wang (New Haven, CT), F O'Brien (Lexington, MA), R Mantegazza (Milano, IT), REGAIN Study Group

Medical treatment has entered a new era with the emergence of biopharmaceuticals, which are chemicals produced in living cells, often yeast, that act on specific chemicals present in the human body. An important class of agents is the monoclonal antibodies (drug names that end in “mab”). Eculizumab is a human monoclonal antibody that specifically targets a key element in the complement pathway, complement element N^o5 or C5. The complement pathway is critical in causing damage to the postsynaptic membrane in most autoimmune forms of MG, with the exception of MuSK-MG. Therefore, Eculizumab has the potential to block the damage caused by the autoimmune attack on the endplate. The results, including those presented here, of the REGAIN study (Eculizumab) extension for the treatment of refractory AChR-MG, are very encouraging. The downside of Eculizumab is the current charge which may well be over \$100,000 per year. If the price of treatment can be reduced, Eculizumab can become an extremely useful tool to fight and eventually cure MG.

TALK 3

Immune Profiling of Circulating Follicular Helper T Cells in Musk-Mg Patients

Y Li (Guangzhou, Guangdong), M Russo (Durham, NC), W Liu (Guangzhou, Guangdong), S Raja (Durham, NC), J Guptill (Durham, NC), J Yi (Durham, NC)

Note: Lay translation of the start of the abstract: “MG is an autoantibody-dependent, CD4 T-cell mediated autoimmune disease of the neuromuscular junction . . .” – There are two general classes of immune cell, aka white cells, specifically lymphocytes. B-cells produce

antibodies and T-cells regulate the B-cells. A subset of CD4 T-cells (CD-4 refers to a specific marker (a distinct molecular element on the cell surface)) present on the surface of a T-cell. The B-cells produce the antibodies that target specific elements of the neuromuscular junction such as the acetylcholine receptor (AChR) or muscle-specific kinase (MuSK) – in addition, there are more antibody targets being recognized and discovered.

This talk focused on the role of Tfh cells, which are follicular helper cells. The Tfh cells are present in the lymphocyte germinal centers (sites where lymphocytes multiply) as well as in circulating blood. Tfh cells enhance the responsiveness of B-cells to T-cell signals. In a sense the Tfh cells change how well the B-cells listen to the T-cells (think of the T-cells as parents and the B-cells as children – what parent wouldn't love to have an effective Tfh to get children to do what you tell them?).

TALK 4

B-Cell Depletion in Late-Onset Myasthenia Gravis is Safe and Effective; A Case Series

S Sahai (Los Angeles, CA), R Lewis (Los Angeles, CA), A Maghzi (Los Angeles, CA)

Rituximab is a monoclonal antibody that targets a class of lymphocytes implicated in MG – B-cells with a CD20 surface marker. Rituximab has the potential to block the action of a class of immune cells that has been shown to be critical for many forms of MG. Most importantly, Rituximab has a much milder side effect profile compared to prednisone-like drugs. Several studies have shown Rituximab to be effective for MuSK-MG. This study targeted older patients with AChR-MG and appreciable side effects due to prednisone or who were refractory to prednisone. All patients greatly improved and either completely tapered off or greatly reduced their dose of prednisone. This was a small study of only 6 patients, but the findings are encouraging for future use of Rituximab as an alternative to prednisone, provided the cost of Rituximab can be brought down. Though the charge for Rituximab is currently much less than for Eculizumab, the current charge exceeds \$50,000 per year. ✨

For more on the 2017 Scientific Session, please see www.myasthenia.org



How do you get the most out of the patient/doctor relationship —Patients & Doctors Offer Advice

In January 2018, MGFA sent a questionnaire to its Medical/Scientific Advisory Board and to the MG community, asking “How do you get the most out of the patient/doctor relationship?” Here we provide a summary of the answers that we received. Many ideas and tips from both doctors and patients overlap.

THE DOCTORS’ PERSPECTIVES

“NO MG patient is typical, so a community neurologist is not likely to have real life experience in caring for the complex issues of every patient,” says Dr. Henry Kaminksi, George Washington University School of Medicine and Health Sciences and former chairman of the MGFA Medical/Scientific Advisory Board (M/SAB).

What can an MG knowledgeable neurologist do for me?

An MG Knowledgeable neurologist can do the following for you--Yuebing Li, MD, Cleveland Clinic, M/SAB Education Committee:

- Confirms the MG diagnosis, as it is often based on combined information rather than a single test.
- Decides on the need for treatment, as not every MG patient needs a medication.
- Chooses the most appropriate treatment based on symptoms, severity and patient characteristics. A physician unfamiliar with MG is unable to do this.
- Monitors appropriately the treatment course, including treatment response, side effect recognition, and medication adjustment. A successful treatment plan relies on the physician’s experience.
- Provides up-to-date treatments for MG patients.

- Networks with other neuromuscular physicians in case a patient needs a referral.
- Provides opportunities to participate in MG trials.

Keeping your regular appointments with your local primary doctor, contacting your MG specialist directly and encouraging the communication between your local primary doctor and the MG specialist are all positive measures to assure an appropriate management of your MG. – Riccardo Maselli, MD, UC Davis

Why is it important to work with an MG expert even if they are located far from where I live?

Treatment of MG is individualized and specialized. Frequent medication adjustment is needed for many patients. Steps are often needed to avoid side effects. You want to be under the care of specialists who are familiar with MG and its treatment, especially at the initial active stage. Once a treatment regimen is established and your MG is stable, you could choose to work with a physician locally.

MG experts have the experience to get the best results for you so you can get back to as normal

a life as possible. They also are aware of research and new treatments and can advise you and your doctors to ensure that you are getting the best possible care.

If I can't see my MG expert regularly, how can I facilitate a collaboration between my MG expert and my local physician?

Keeping your regular appointments with your local primary doctor, contacting your MG specialist directly and encouraging the communication between your local primary doctor and the MG specialist are all positive measures to assure an appropriate management of your MG.

– Riccardo Maselli, MD, UC Davis

This may mean that you need to interview several doctors before finding one who will work with your neuromuscular neurologist. MG experts are happy to work with your local physician and can come to a plan that can be implemented locally even when you may only see your MG expert on a limited basis.

How can I get the most from my doctor's visit?

- Clearly state the goals of your visit to your physician.
- Focus on MG related symptoms if you know what they are.
- Provide the highlights of your medical history and your medication list.
- Write down questions you want to ask and ask them one by one.

- Ask about your long-term treatment plan, possible major side effects and their probability of occurring and possible alternative treatments.

What are best practices for preparation and for follow-up to my doctor's visit?

If you have been seen by other physicians, please make sure that your prior medical records have been sent to the provider you are about to see. If there isn't much, the best approach is to obtain a copy and hand-deliver it to the new physician at the time of the visit. Please make sure that information about MG diagnoses is included.

Think about the most important questions for you, write them down and ask at the visit.

If you feel that your MG is worse, or you are experiencing side effects from a medication, please do not hesitate to call (rather than waiting for the next appointment).

Between doctor's visits are there things I should be doing to keep track of my MG and health generally?

- Sleep well.
- Eat healthy.
- Do regular light exercise.
- Avoid infections.
- Enjoy life. ✨

We want to thank the following contributors to this article:

- William Bailey
- Gene Casagrande, DDS
- Michael Gates
- Janet Genovese
- Stormee Genz
- Cathi Gilliam
- Jonathan Goldstein, MD
- Rachel Higgins
- Robert Horbatt
- Frank Johnson
- Linda Ann Joslin
- Henry Kaminski, MD
- Barry Levine
- Yuebing Li, MD
- Ricardo A. Maselli, MD
- Rebecca Molitoris
- Janet Myder
- Bridget Noujaim
- Julie Roberts
- Sara Rutledge
- Elroy Tschirhart
- Kristina Voskes
- Beverly Watrous
- Andy Waymouth
- Paula Wright

Remember that you are an important part of your "health team." Don't simply accept everything that the doctor says. Be a part of the decision-making by asking questions, expressing your concerns and views. Decisions should be made together with you. — Janet Myder

continued on page 22



THE PATIENTS' PERSPECTIVES



How do you get the most out of your MG doctor's visit? This question received a lot of responses with many worthwhile tips:

Record your questions and chart your symptoms/ups & downs between doctor's visits. Use an old fashioned notebook, a calendar, or an electronic device, whatever works best for you. Note anything you need to discuss with your doctor.

Use the MyMG app as a way to monitor symptoms. You can regularly take the MG Quality of Life 15 (MG QOL 15) survey and/or the MG Activities of Daily Living (MG ADL) survey as ways to track your ups and downs. You can do this on your computer or phone (get the App through the MGFA website: www.myasthenia.org) and then generate charts with your results via computer to bring to your doctor visit.

Be sure to bring a list of current medications and allergies to your visit.

Do you need any prescription renewals? Prepare for your doctor visit by reviewing your notes and organizing questions/ information in priority order.

Ask if your doctor minds recording his comments on your phone. This may be easier than trying to take notes but not all doctors may feel comfortable with this.

Be prepared to ask questions as the doctor makes recommendations or new prescriptions.

"Remember that you are an important part of your "health team." Don't simply accept everything that the doctor says. Be a part of the decision-making by asking questions, expressing your concerns and views. Decisions should be made together with you." — Janet Myder

What's important in choosing an MG doctor?

It's important to find a doctor who really knows MG. Ask how many MG patients the doctor has. Check in with others you know with MG. Call MGFA for referrals. Other sources may be MDA or another trusted doctor.

If you live in an area with few or no MG experts, you may want to consider traveling to a larger community where you can find an MG expert and/or go to a University Hospital which has MG experts on staff. Although you may not be able to go to this doctor regularly you can work with him/her and find a local doctor who can consult with the MG expert on your care. Doing this could make a tremendous difference in your disease management and quality of life.

"Another important characteristic to look for when selecting a neurologist is to look for one who is a good listener and is able to communicate information to you in way that can be easily understood. If your physician uses any medical terminology that you do not understand, ask him to clarify in terms that are easy for you to comprehend." — Janet Genovese

Is the doctor involved with MG research?

Find a doctor with whom you have rapport, who listens, who doesn't disregard you. *"Another important characteristic to look for when selecting a neurologist is to look for one who is a good listener and is able to communicate information to you in way that can be easily understood. If your*

“When I was first diagnosed, my husband and I interviewed 6 Family Practitioners. Five were really not knowledgeable and arrogant. The one I chose didn’t know that much about MG, but this is why I picked Dr. Kimberly Warfield: She said, “I had 15 mins in medical school, so I don’t know that much. I am willing to learn.” That did it for us. She has learned MG and communicates with Dr. Horvit, regularly. She knows MG. — Linda Ann Joslin

physician uses any medical terminology that you do not understand, ask him to clarify in terms that are easy for you to comprehend.”—Janet Genovese

It’s important to have a physician who works with you toward a common goal or gives you evidence or statistics that can guide your decisions.

Does the doctor have hospital privileges and if so, with which ones? Can you easily get to this hospital? Does it have a good reputation? Look beyond a few comments by friends or family to assess the hospital’s reputation. If the doctor does not have an affiliation with a hospital, what is the plan if you are hospitalized?

Is the doctor available both for regular appointments and emergency or urgent needs?

Find out which hospital the doctor uses/you can use for emergencies. Is there an MG expert associated with the hospital?

“When I was first diagnosed, my husband and I interviewed 6 Family Practitioners. Five were really not knowledgeable and arrogant. The one I chose didn’t know that much about MG, but this is why I picked Dr. Kimberly Warfield: She said, “I had 15 mins in medical school, so I don’t know that much. I am willing to learn.” That did it for us. She has learned MG and communicates with Dr. Horvit, regularly. She knows MG. — Linda Ann Joslin

What do you do before and after your doctor’s visit to make the most of it?

If possible, plan to take your spouse, caregiver or a close friend with you to your doctor’s visit – 2 minds are better than one. Compare notes on what

the doctor said directly after the visit to make sure you’ve captured everything.

“I ask the doctor’s office to print off their notes and I finish writing down my notes so it’s fresh in my mind. I immediately call my spouse, if he hasn’t gone with me to my appointment and inform him of my recent visit.” — Cathi Gilliam

What are the 3 most important questions you regularly ask your doctor? Here are questions that respondents shared:

What do my lab reports mean? Is there a trend?

Should my medications stay the same or be adjusted?

Would I benefit from any of the new drugs or treatments that have recently been approved?

Are there any changes needed for my course of treatment?

If any new treatments, medications or tests are ordered, what is the rationale, risks and benefits of the treatment?

Based on my symptoms, what does my treatment plan look like for the foreseeable future? What goals do we have?

Can I take a supplement that I have heard about?

Here are my thoughts, what are yours?

Is there any new research, or clinical studies that show promise or that I could be a part of?

How do you think I am doing?

Do you have guidance on exercise?

What signs and symptoms should I be aware of that may indicate the need for immediate medical care?

If I have a flare in symptoms, who should I contact to avoid an exacerbation?

“I ask the doctor’s office to print off their notes and I finish writing down my notes so it’s fresh in my mind. I immediately call my spouse, if he hasn’t gone with me to my appointment and inform him of my recent visit.” — Cathi Gilliam

continued on page 31



Congratulations to our 2017 Top Fundraisers and Top Teams!

We can't do what we do without YOU!

WALKER	TEAM	WALK	AMOUNT
Lauren Jarman	Vent Stoppers	South Carolina	\$20,875
Gary Eder	Team Gary	TriState	\$15,045
Thomas Larsen	Tom's Rockets - Blast Off For The Cure!	Virginia	\$14,716
Leni Fuhrman	NY Trailblazers	TriState	\$14,095
Susan Klinger	NY Trailblazers	TriState	\$10,739
Jerry Friedman	Team Evan Greene	Greater Los Angeles	\$10,213
Gaynell Wortman	Team Amanda	South Carolina	\$9,230
Evan Greene	Team Evan Greene	Greater Los Angeles	\$9,056
Nancy Law	Team MG Blizzard	Virtual 2017	\$8,750
Nelson Machado	Team Mario	TriState	\$8,675
Jessica Larkin	MuSK-eteers	New England	\$8,130
Sharon Eucce	My My Myasthenia - Sharona	Bay Area	\$6,935
Phil Cogan	NY Trailblazers	TriState	\$6,431
Dhira Bluestone	MTD_Lite	TriState	\$6,260
Nicolette Hoffman	Knockout MG for Nicolette	Tallahassee	\$5,750
Sheldon Katz	Team Katz	Greater Los Angeles	\$5,615
Alexis Rodriguez	Rockin' Rodriguez	Georgia	\$5,558
Debora Buzinkai	Team Zippy	TriState	\$5,300
Harry J O'Leary	Team O'Leary	Janesville	\$5,146
Dawn Warner	Dream Vacations	Georgia	\$4,685
Walking For Dad	Walking For Dad	TriState	\$4,581
Cherille O'Connor	Step it up for a Cure	Greater Los Angeles	\$4,450
Philip Aitken-Cade	Tom's Rockets - Blast Off For The Cure!	Virginia	\$4,350
Harper Daley	Team Harper	Portland	\$4,170
Larry Dix	Team Cleveland Clinic	Ohio	\$3,920
Joni Kendrick	Central Texas Support Group	Austin	\$3,920
Celia Meyer	Team Duck Tapers	New Orleans	\$3,906
Vickie Henderson	elle est forte	Arkansas	\$3,887
Karen K	The Weiss Guys	Tampa Bay	\$3,765
Gavin Brown	Gavin and the Sparkling Synapses	Georgia	\$3,710
Lynn Bauer		New England	\$3,500
Kristina Voskes	Team Voskes	Colorado	\$3,440
Kathleen Sullivan	Kash's Crew vs MG	Chicagoland	\$3,410
Laura Nyanjom	snowflake ninjas	Maryland	\$3,380
Lori Lappe	The Crusaders	Bay Area	\$3,306
Andrea Carbone	Kiss Myas(thenia Gravis)	Virginia	\$3,304
Jessica Milanes	Team Jess	Bay Area	\$3,240
Elizabeth Frenna Roque	I Swear I'm Not Drunk	TriState	\$3,175

WALKER	TEAM	WALK	AMOUNT
Steven Grant	TEAM GRANT	TriState	\$3,150
Jessica Simmerman	The Weiss Guys	Tampa Bay	\$3,141
Amanda Adkins	Team Adkins	Houston	\$3,093
Roger Morse	Team Duck Tapers	New Orleans	\$3,055
Abby Kovalsky		Bay Area	\$3,030
Danielle Buathier	Justice League	Ohio	\$3,015
Rachel Moldauer	Rach's Team	TriState	\$3,001
Paula Mcginnis	Rocknmc	Southern Illinois	\$2,940
Tommy Santora	Team Santora's Prednisone Poppers	New Orleans	\$2,895
Marilyn Cohen	The Speedsters	South Florida	\$2,803
Shannon Whitfield	Team Whitfield	Georgia	\$2,795
Angie Espinosa	The Crusaders	Bay Area	\$2,765



On behalf of everyone in the MG community throughout the country, thank you for all you do, and continue to do to show love and support to all those affected by MG. Because of you, we get closer and closer every day to a world without myasthenia gravis! ✨

My Body – by Kait Masters

Today during my infusion, I was asked what loving my body means to me.

I'll be honest, this is a loaded question for me. Being someone with an invisible and disabling illness, I've always had a complicated relationship with my body. It has let me down a lot over the last 16 years.

Loving my body means giving myself grace and being kind to myself. Loving my body also means I've had to navigate and process the emotional heaviness of being truly very sick with no visible indications and with very little control over my body.

There have been great disappointments, scary seasons, and surgeries. I have had my sternum cracked, and my thymus removed and biopsied. I have a medical port implanted in my arm because I have all but lost vein access now. 9 of my scars from surgeries and biopsies have turned into large keloids. The treatment I respond best to has given me aseptic meningitis 3 times. There have been times when my muscles were so weak and my breathing was so compromised, I was afraid to go to bed at night. And all while appearing perfectly healthy on the outside.

On the flip side, I'm also incredibly grateful that my body is resilient. It's astounding to know what my body has handled and survived—to know what it will continue to endure and what I'll adapt to down the road. Loving my body is knowing that every accomplishment is worth celebrating—being able to take my dog for a walk, cook a meal, or even fold my laundry are victories worth celebrating because I've lived seasons where those simple tasks were unattainable.



THE STATE HAS A PLAN FOR YOUR LEGACY. DO YOU?

Fewer than half of Americans have a will. Without a will, the state will decide how to distribute your hard-earned money. Don't lose control of your legacy. Visit an attorney and prepare a will and when you do, remember the MGFA. Make part of your legacy "A world without Myasthenia Gravis"

FAMILY ESTATE
PLANNING



What's Hot Off the Press in Neuromuscular Junction Disorders?

Ikjae Lee, MD, Department of Neurology, University of Alabama at Birmingham

Sarah Jones, MD, Department of Neurology, University of Virginia

New FDA Approved Treatment for Refractory ACh Receptor Positive Myasthenia Gravis

Complement is a part of our immune system that works along with antibodies to protect our body from microorganisms. In the presence of acetylcholine receptor antibody, the activation of complement destroys the neuromuscular junction resulting in symptoms of myasthenia gravis (MG). Dr. Howard and his colleagues¹ successfully completed the phase 3 REGAIN study to show that Eculizumab (brand name Soliris) treatment can improve MG symptoms by inhibiting complement cascade.

This study was designed as a phase 3, randomized, double blinded, placebo-controlled trial in a fashion to eliminate potential biases. Seventy-six Hospitals and specialized clinics in 17 countries across the globe participated in the study. Eligible patients were adults who have generalized MG with positive AChR antibody. Additionally, the study required participants to have refractory disease defined as functional limitation despite of treatment with at least three immunosuppressants including IVIG or plasma exchange. Patients were excluded if they had a thymic tumor, thymectomy in the past year, recent use (less than 4 weeks) of IVIG or plasma exchange, or recent use (less than 6 months) of Rituximab. All participants were required to have been vaccinated against Neisseria meningitidis due to increased risk of severe meningococcal infection with complement inhibition.



One hundred and twenty-six (126) total participants were enrolled in the study: 63 received eculizumab and 63 received placebo medication. Eculizumab was given as intravenous infusion, 900mg weekly for the first month followed by 1200mg every other week thereafter for up to 26 weeks. The authors evaluated disease severity by using patient

reported scales (MG-ADL and MG-QOL15), a physician measured outcome scale (QMG) and a combined scale (MG composite). The change of scores before and after the treatment were compared between eculizumab and placebo treated groups.

Primary analysis did not reveal significant difference in MG-ADL score change between the groups. Additional analyses, however, did show the benefit of

eculizumab as there was an improvement in QMG total score change and also a higher proportion of patients achieving a clinically meaningful response. Authors suggested that the way they were interpreting the data - using a worst-rank analytic approach - likely led to failure in meeting the primary end point. Overall, eculizumab was well tolerated and the safety profile was similar to previously reported. There was no increase in serious infections or reports of meningococcal infections. Participants treated with eculizumab had fewer MG exacerbations, less use of rescue medication, and fewer admissions to hospital.

In summary, the REGAIN study showed the treatment benefit and safety profile of eculizumab

in refractory MG patients with acetylcholine receptor antibody through a rigorous randomized trial. It is important to acknowledge that the trial was funded by Alexion pharmaceutical company, which is a producer of eculizumab.

In nearly all 65 participants with MG or LEMS, a significant increase of the anti-tetanus antibody response was seen.

Tetanus Vaccine Shown to be Safe in patients with MG

Vaccinations are recommended for at risk populations to avoid serious infections that are potentially avoidable. The safety and efficacy of the vaccinations has not been clearly established in MG

patients who are commonly immunosuppressed. Ellen Strijbos and her colleagues² investigated the immune response and safety of tetanus revaccination in patients with MG or Lambert-Eaton myasthenic syndrome (LEMS).

A group of 65 patients with MG or LEMS were given a tetanus revaccination and the response was compared to a historical control group which consisted of 20 healthy individuals (HC group). The immune response was determined by measuring anti-tetanus IgG titer 4 weeks after the revaccination. Disease severity and disease-specific antibodies (AChR, MuSK, or VGCC) were measured prior to and 4 weeks after the revaccination. These results were compared between 65 patients with revaccination and 23 AChR MG patients who had received placebo (saline; placebo AChR MG group).

In nearly all 65 participants with MG or LEMS, a significant increase of the anti-tetanus antibody response was seen. Participants with immunosuppressive medication such as prednisone or azathioprine had overall lower vaccination titers compared to healthy controls, but still the response to vaccination was still determined to be significant. There was no significant worsening of the MG symptoms or increasing disease specific antibody titers associated with tetanus revaccination compared to placebo AChR MG group.

The study result supports that a tetanus revaccination in patients with MG or Lambert-Eaton myasthenic syndrome is safe and induces

significant immune response even when immunosuppressive medications are being taken.

A Tool to predict which myasthenia gravis patients worsen after Thymectomy

Thymectomy has been shown to be an effective treatment option for patients with AChR antibody positive MG. Worsening of the myasthenia symptoms after the surgery, however, has been a concern for both patients and their doctors. Researchers from Japan³ studied MG patients who had thymectomy to find characteristics that help predict myasthenic crisis after thymectomy surgery.

The study authors first reviewed 275 patients with AChR antibody who underwent thymectomy to identify baseline and clinical variables associated with the risk of postoperative myasthenic crisis. Statistical methods were used to establish the scoring system model that fits best with the observed outcome using these identified variables. This scoring system was validated in 118 patients with AChR antibody who underwent thymectomy at 4 centers in Japan.

Factors significantly associated with the occurrence of postoperative crisis included (1) the time between MG onset and thymectomy (particularly less than 3 months), (2) MG severity using the MGFA classification, (3) thymoma associated MG, (4) bulbar symptoms, (5) neck weakness, and (6) Forced Vital Capacity (FVC, a measure of lung function). The Postoperative Myasthenic Crisis Predictive Score was formulated by giving 1 point for preoperative bulbar symptoms, 2 points for duration of MG less or equal to 3 months, and 3 points if preoperative FVC is less than 80% predicted. The results demonstrated that only 1% of the low score (<2) group experienced postoperative crisis compared to 22% of the high score (>=3) group. The scoring system also showed to have a very high negative predictive value. This

Additional analyses, however, did show the benefit of eculizumab as there was an improvement in QMG total score change and also a higher proportion of patients achieving a clinically meaningful response.

continued on page 28



continued from page 27

means postoperative crisis was unlikely if the total score was lower than 2. It is important to note that the study was done in Japanese MG population, where ethnic factors and treatment pattern might not be the same as it is with the US MG population.

Multicenter Review indicates that Rituximab can be an Effective Treatment for MuSK Myasthenia Gravis

The primary outcome target of level 2 or lower (better) was reached significantly more frequently in the rituximab treated group (58%) compared to the control group (16%).

Anti-muscle-specific-kinase (MuSK) antibodies associated MG presents with early involvement of neck, swallowing, speech and breathing muscles often requiring multiple high dose immunosuppressants. Rituximab is a monoclonal antibody targeting B lymphocytes. Rituximab has been shown to be a useful treatment of MuSK MG in case series, but it has

been difficult to conduct randomized controlled trials due to the rarity of MuSK MG. Michael Hehir and his colleagues⁴ performed a multicenter review of anti-MuSK positive MG patients to see if there is a difference in outcome when treated with Rituximab or not (control group). Authors tried to minimize bias by blinding the reviewer from treatment information and obtaining data prospectively.

Authors looked at MG post-intervention status in order to interpret how well the patients are doing after treatment. They evaluated how the patient was doing and what immunosuppressant therapies were being used at follow up visits. Based on this information, patient outcome

was graded into 7 different levels from Level 0 being complete stable remission without immunotherapy to level 6 being symptomatic and requiring recent hospitalization for IVIG or plasma exchange treatment. The primary outcome target of level 2 or lower (better) was reached significantly more frequently in the rituximab treated group (58%) compared to the control group (16%). In addition, at the last visit only 29% of rituximab treated patients were taking prednisone, which was significantly fewer compared to the control group (74%). This study results provide class IV evidence that rituximab treatment increases the probability of good outcomes in patients with anti-MuSK antibody MG.



References

1. Howard JF Jr, Utsugisawa K, Benatar M, Murai H, Barohn RJ, Illa I, Jacob S, Vissing J, Burns TM, Kissel JT, Muppidi S, Nowak RJ, O'Brien F, Wang JJ, Mantegazza R; REGAIN Study Group. Safety and efficacy of eculizumab in anti-acetylcholine receptor antibody-positive refractory generalised myasthenia gravis (REGAIN): a phase 3, randomised, double-blind, placebo-controlled, multicentre study. *Lancet Neurol.* 2017 Dec;16(12):976-986.
2. Strijbos E, Huijbers MG, van Es IE, Alleman I, van Ostaijen-Ten Dam MM, Bakker J, van Zwet EW, Jol-van der Zijde CM, van Tol MD, Verschuuren JJ. A prospective, placebo controlled study on the humoral immune response to and safety of tetanus revaccination in myasthenia gravis. *Vaccine.* 2017 Nov 1;35(46):6290-6296.
3. Kanai T, Uzawa A, Sato Y, Suzuki S, Kawaguchi N, Himuro K, Oda F, Ozawa Y, Nakahara J, Suzuki N, Takahashi YK, Ishibashi S, Yokota T, Ogawa T, Yokoyama K, Hattori N, Izaki S, Oji S, Nomura K, Kaneko J, Nishiyama K, Yoshino I, Kuwabara S. A clinical predictive score for postoperative myasthenic crisis. *Ann Neurol.* 2017 Nov;82(5):841-849.
4. Hehir MK, Hobson-Webb LD, Benatar M, Barnett C, Silvestri NJ, Howard JF Jr, Howard D, Visser A, Crum BA, Nowak R, Beekman R, Kumar A, Ruzhansky K, Chen IA, Pulley MT, LaBoy SM, Fellman MA, Greene SM, Pasnoor M, Burns TM. Rituximab as treatment for anti-MuSK myasthenia gravis: Multicenter blinded prospective review. *Neurology.* 2017 Sep 5;89(10):1069-1077.



EMERGENCY PREPAREDNESS

Sally O'Meara, RN & Bruce Yelverton, EMT Director (Retired)

Every person with MG should be prepared for the possibility of an MG Crisis. Although you may never experience a crisis – being prepared is an essential. Here's what you should know.

Myasthenic crisis can develop slowly or quickly. It is important for patients to get medical care right away when symptoms of myasthenic crisis develop. Sometimes it is hard to tell if shortness of breath is due to anxiety or MG muscle weakness. Patients can do simple assessments at home to check respiratory function.

Signs that breathing function is worsening include:

- Cannot lay flat in bed without feeling short of breath or gasping for air
- Rapid shallow breathing (especially more than 25 breaths/minute)
- Having to pause in the middle of a sentence to take a breath
- Weak cough, especially when mucus/saliva cannot be cleared from the throat
- The muscles between the ribs and around the neck pull in during breathing
- Cannot count out loud past 20 after a full breath of air (single breath count)
- Sweating even when the room is not too warm
- Waking up frequently during the night gasping for air
- Feeling restless, agitated, drowsy or confused
- Breathing worsens even after taking MG medications
- The chest wall moves inward instead of expanding when air is inhaled
- Feeling too tired to keep breathing

Patients should seek immediate medical help when feeling short of breath with the warning signs listed above.

Emergency preparedness plan

Every MG patient should have an emergency plan in place in order to make decisions and provide critical information to healthcare professionals.

Calling for emergency assistance

Some 911 call centers now accept text messaging and some 911 centers can register patient medical information in their database in case of emergency.

Some medical alert systems can

call 911 when the help needed button is pushed.

Patients and caregivers need to check to see what options are available in their area. If the patient is unable to speak when calling 911, the operator will identify the location and send help. An ambulance should be called if the driver is anxious, the patient is too ill to speak, breathing assistance is needed or traffic will cause delays.

First responders must easily be able to see the house address from the street, especially at night. A flashing or colorful front house light can help first responders identify where to go. New electronic light switches can be activated by smart phone apps or voice-activated assistants like Google Home or Amazon Alexa. If the front door

Although you may never experience a crisis – being prepared is an essential.

continued on page 30



Emergency Preparedness continued from page 29

has an electronic key pad lock, the access code should be given to the 911 operator. Some medical alert programs can put a coded lock box on the front door. Lock boxes can also be purchased and mounted. In case of emergency, paramedics will be provided with the access number so that they can enter the home.

MG patients should wear some type of medical alert jewelry at all times to quickly notify healthcare professionals about their health history in case of emergency. Paramedics and hospital personnel are trained to look for medical identification jewelry, especially when caring for patients who are unable to speak. Various types and styles of bracelets and pendants are available. Some have USB chips or QR codes that store important medical information. Others have fall detection sensors and 24/7 emergency response services to provide medical history to providers.

MG patients should also have emergency contacts listed in their cell phones indicated by ICE in the cell phone directory. The ICE and medical history can be added to password-protected phones so that the information can be accessed by emergency room personnel without having to unlock the phone first. Emergency medical information

apps can also be downloaded to smart phones for use by healthcare providers. Patients can use their providers' patient portals to gather pertinent medical history and test results. Patients should check <https://www.healthit.gov/providers-professionals/faqs/what-patient-portal> and <https://www.healthit.gov/patients-families/your-health-data> for more information.

MG patients and their families should be prepared to educate providers about MG as it is a rare illness that presents differently than other types of respiratory failure. Shortness of breath from myasthenia gravis is due to weak respiratory muscles- a concept that many providers are not familiar with. Pulse oximetry is NOT a good test for breathing function in MG patients unless the

All MG patients should have a packet of medical information ready in case of emergency in a packet on the refrigerator and glove box of personal vehicles. The packet must be kept organized and up to date in a brightly colored, clearly labeled envelope. It should include:

- Contact information for all physicians
- Medical history & hospital records
- Emergency contact list
- Health insurance cards
- Information about any implanted devices (i.e. IV port, pacemaker)
- Advance directive papers and medical power of attorney (POA) forms
- Complete medication list – including dosage, frequency and reason for all supplements, vitamins, over-the-counter and prescription medications.
- List of allergies – including medications and other substances (foods, latex, chemicals)
- Medical History – all surgeries, conditions and recent test results
- MG information for providers
<http://www.myasthenia.org/HealthProfessionals/EmergencyManagement.aspx>
- MG medication precautions list
<http://www.myasthenia.org/LinkClick.aspx?fileticket=zmLaFitarOQ%3d&tabid=318>

patient has underlying lung problems such as COPD or pneumonia. It is not uncommon for MG patients in acute respiratory failure/myasthenic crisis to have pulse oximetry levels > 95%. Careful observation of respiration and bedside measurements (forced vital capacity, single breath count) are more reliable indicators of respiratory status than pulse oximetry in MG patients.

The ER physician should call the neurologist to guide treatment decisions. A family member or friend should be present at all times to act as an advocate. If the patient has a BiPAP machine at home, it should be brought to the hospital so that respiratory support will be provided without interruption.

Pulse oximetry is NOT a good test for breathing function in MG patients unless the patient has underlying lung problems such as COPD or pneumonia.

An ambulance should be called if the driver is upset or if the patient is too ill to speak. An ambulance will be able to provide breathing assistance during the trip to the hospital and avoid delays from traffic congestion. Patients should bring their own ventilator or BiPAP to the hospital to ensure continuous breathing support although it may become necessary to switch to a different type of machine depending on the patient's respiratory status. While it may be helpful to go to the emergency room where the patient's neurologist practices, the ambulance may need to go to the nearest hospital to obtain immediate care for the patient.

Once the patient arrives at the emergency room, the patient's advocate should be prepared to communicate effectively with healthcare personnel. Key medical information should be organized and ready for providers. The patient's symptoms and medical history should be related in a concise manner using correct terminology to avoid missed information or errors. The patient's advocate should also be ready to clarify contradictory information between physicians, nurses and other providers.

Having an emergency preparedness plan in place allows MG patients to quickly receive appropriate medical care when myasthenic crisis develops. It also enables MG patients to have some control and direction in what happens during their treatment.

MGFA RESOURCES:

- Emergency Alert Card (1) – Get this card for your wallet, it states that you have MG; gives your details (name, address, etc.); who to contact in an emergency; and First Responder guidance.
- Emergency Alert Card (2) – Provides guide on Drugs to be Avoided as well as your details (name, address, etc.); who to contact in an emergency. Ask for both cards by calling or writing the MGFA office.
- Emergency Management I brochure is directed to medical personnel and gives an overview of the issues – also discussed in this article.
- Emergency Management II is directed to patients, families and caregivers. Get both brochures to share with your family, add to your emergency kit and share with caregivers. ✨

Patients & Doctors continued from page 23

If you've had to work with a doctor who's unfamiliar with MG how did you help him/her become the expert you needed?

Bring a list of the medications that should be avoided or used with caution in patients with MG as well as any medications or treatments you are receiving.

Make sure that he/she understands your history including the signs and symptoms that you have experienced with MG. Give the physician the name and contact information of your neurologist so that he/she can consult with them if there are any concerns regarding your course of treatment.

– Janet Genovese

I explain what MG is, and share the MGFA website. If not a neurologist, provide the number for your neuro, for any needed questions or consultation.

Alert the doctor to resources available on the MGFA website such as the MGFA Manual for Healthcare Providers and the website, www.myasthenia.org/HealthProfessionals. Talk to him/her about the limitations and restrictions of the disorder. If the physician seems unwilling to listen, consider finding a new doctor.

Other Tips & Ideas:

Reduce stress by leaving extra time for traffic and parking challenges.

Find out if you can communicate with your doctor between visits via email or systems used by some medical practices such as "MyChart." Know whether you can ask the doctor's staff questions between visits.

Arrive rested and focused so you can listen effectively. ✨

Resources

NIH – National institute on Aging – What Should I Ask My Doctor during a Checkup?

Go to <https://www.nia.nih.gov/> use the search function to look for Checkup Questions or the title above.

Or, visit www.medlineplus.gov and look for the Talking with Your Doctor section under Health Topics for many articles touching on this issue.

Visit MGFA's website: <http://www.myasthenia.org/CommunitySupport/MGFAConferenceVideos.aspx> and look under 2016-Raleigh for Preparing for a Neurology Appointment presented by Marcia Lorimer, RN, Dr. Allan Weiss & Tommy Santora.



355 Lexington Avenue, 15th Floor
New York, NY 10017-6603
(800) 541-5454
212-297-2158 fax
mgfa@myasthenia.org

www.myasthenia.org   

Myasthenia gravis is an autoimmune neuromuscular disorder. Symptoms may include double vision, drooping eyelids, slurred speech, difficulty chewing and swallowing, weakness in arms and/or legs.

MGFA is committed to finding a cure for myasthenia gravis and closely related disorders, improving treatment options, and providing information and support to people with myasthenia gravis through research, education, community programs, and advocacy.

Focus on MG is published by the Myasthenia Gravis Foundation of America, Inc. If this issue was mailed to you, you are on our subscriber list. If you would like to add, remove or update a subscription, or request that you receive future issues by e-mail, please contact the MGFA home office.

If you would like to receive Foundation Focus by email only, please email mgfa@myasthenia.org.

**THANK
YOU**

**...to all of our
wonderful donors!**

*your generosity
brings us closer to
a world without
MG.*



The goal of the MG Walk Campaign is to expand into new markets where we can bring together patients, create a community of active/engaged MG families and raise vital

awareness & funding for myasthenia gravis! It is crucial that we go where we know we can garner the support needed to ensure success. If you are interested in seeing the MG Walk come to your area and you are excited to play an active part in its planning, promotion and production, we want to hear from you! Please contact the MG Walk Office at 1-855-MG-WALKS or Info@MGWalk.org or fill out our interest form found online at www.MGWalk.org. Thanks so much!