MYASTHENIA GRAVIS FOUNDATION OF AMERICA, INC.

Foundation Focus

News about myasthenia gravis for patients, family and friends

Fall 2013

Join a Fall 2013 MG Walk!

Washington, October 20

Colorado, October 26

Arizona, October 27

Pennsylvania, November 2

Ohio, November 3

Tennessee, November 16

Northern California, November 17

Visit MGWalk.org for details on the MG Walk nearest you or to join the Virtual Walk!

MG walk

For a World Without Myasthenia Gravis

355 Lexington Avenue, 15th Floor
New York, NY 10017-6603
(800) 541-5454 | (212) 370-9047 fax
mgfa@myasthenia.org | www.myasthenia.org

Message from Chairman Sam Schulhof

Dear Friends,

As I sit down to write this letter it's hard to believe the "lazy days of summer" are coming to an end. For MGFA they haven't been lazy but rather a time for productivity and growth.

This year's National Conference in Miami attracted a record number of new and younger attendees as we continue to update both format and programs making them more relevant to the changing demographics. The Foundation's successful and growing awareness programs and a better understanding of the needs of the MG community have enabled these changes.

The MG Walks have been a big part of raising awareness and are attracting new and diverse people to the Foundation. To date the walks have attracted 3000 participants of which 50% have had no previous involvement with the foundation.

The MG Patient Registry, a confidential and patient-driven research project funded by the Foundation was launched at our National Conference and has enrolled 700 plus individuals to date and is growing rapidly. The Registry is an active database of persons with MG developed for the purposes of research, treatment, patient information, and to raise public and medical community awareness of the prevalence of MG. It is expected that this initiative will contribute to a better understanding of MG, patients' needs, improved therapy options and, one day, a cure for MG.

MGFA is launching in this issue of the Foundation Focus a quarterly review and update: "What's Hot off the Press in Neuromuscular Junction Disorders." This review will also be available on the MGFA website.

MGFA announced the funding of two new research projects under its RFA Program at the National Conference:

1. Study of the disease-related quality of life (MG-QOL15) and symptoms and activities (MG-ADL) of patients with MG who download and completed the free "myMG" app.
2. Efficacy of Prednisone in the Treatment of Ocular Myasthenia.

In addition The Board agreed to fund 2 international consensus conferences:

1. Development of Preclinical Research Standards for therapeutic evaluation of agents in animal models of MG. The goal is to achieve international consensus regarding outcome assessments, induction of experimental MG, and assure compliance with NINDS preclinical standards. It is expected that establishment of such standards will aid in therapeutic development.
2. Development of Treatment Guidelines for MG. The goal is to achieve international consensus regarding treatment guidelines.

As you can see, we have been busy over the last several months!

I hope each of you and your families have had a healthy and productive summer and look forward to the fall and the holiday season with as much excitement as I do.

Myasthenia Gravis Patient Registry is "Live" and Open for Enrollment

The long-awaited Myasthenia Gravis Patient Registry has been launched and is "live" and open to patient enrollment. The registry is a confidential means for patients to provide information to an active database of persons with MG and was developed for the purpose of research, treatment and patient information.

It provides a system for researchers to gauge the potential for recruiting patients for clinical trials and communicating with them in a manner that respects their privacy. The registry also can be used to provide education about MG to patients, care givers, non-expert health care providers and funding sources.

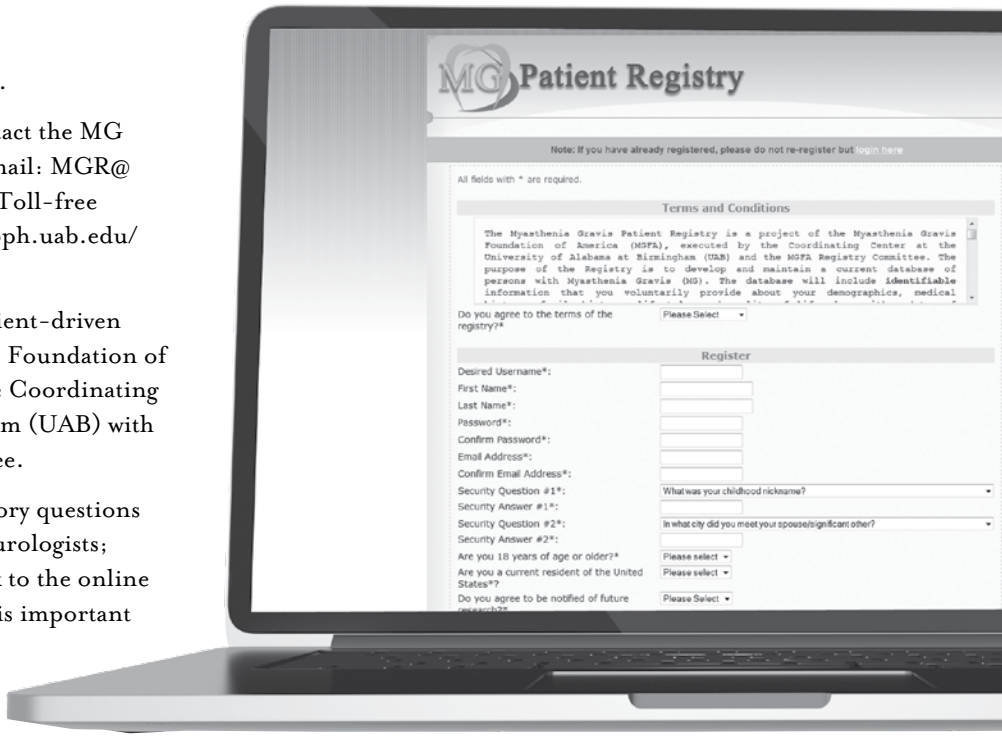
To enroll, follow these steps:

- Click on the following link to go to the "Participant Sign Up" page: https://mgregistry.soph.uab.edu/MGRegistry/SignUp.aspx
- Read and agree to the Terms and Conditions of participation and complete all of the fields under Register. (For future reference, record the username, password, and email address that you use to register, as well as your security questions and answers.)
- Click the button "Sign me up".
- Complete and submit the enrollment survey.
- If you have questions or need assistance contact the MG Patient Registry Coordinating Center at: Email: MGR@MGregistry.org; Phone: 205-975-8633 or Toll-free 855-337-8633; Web address: http://www.soph.uab.edu/mgregistry/

The MG Patient Registry is a confidential and patient-driven research project, funded by the Myasthenia Gravis Foundation of America (MGFA), managed by the MGFA and the Coordinating Center of the University of Alabama at Birmingham (UAB) with oversight by the MGFA Patient Registry Committee.

The registry asks MG patients several medical history questions that may require some fact-finding from their neurologists; patients can save their information and come back to the online survey to complete those questions at any time. It is important for MG patients to answer the questions to the fullest and to the best of their ability to help database researchers gather complete information on their illness. After patients submit the enrollment survey, they will be fully enrolled in the MG Patient Registry and will begin receiving survey updates two times a year. Also, a newsletter with articles about research findings and other topics relevant to individuals with MG will be sent to participants annually.

Gary Cutter, PhD, Managing Director of the MG Registry, and Professor of Biostatistics and Head of the Section on Research Methods and Clinical Trials at the UAB School of Public Health, headed the MG Patient Registry Committee, along with Sam Schulhof, MGFA Chairman; Henry Kaminski, MD, Chair, Department of Neurology, George Washington University School of Medicine and Health Services; Ted Burns, MD, Department of Neurology, University of Virginia; Don Sanders, Division of Neurology, Duke University Medical Center; Gil Wolfe, Irvin and Rosemary Smith Professor and Chair, Department of Neurology/Jacobs Neurological Institute, State University of New York at Buffalo School of Medicine and Biomedical Sciences, Buffalo General Medical Center; and Jennifer Faucett, Esq., Board Member, MGFA, MG Patient and attorney with Waller Lansden Dortch and Davis.



MGFA Closes Out Successful National Conference in Miami, Florida

MGFA's 2013 national conference in Miami was another successful gathering of people who have MG, their families and caregivers, as well as medical professionals and researchers who are treating and trying to conquer MG.

This year's meeting introduced a new feature, interactive sessions. Facilitated by people with experience at several levels, young adults with MG, parents of children with MG, and mature people who have MG, these sessions enabled attendees to tell their stories and discuss their unique concerns with others. Videos of conference sessions will be available at www.myasthenia.org.

A highlight of the conference was the Awards Dinner. MGFA recognized dedication and commitment to the foundation's mission and bestowed the following honors:

Lifetime Achievement: Coleen Shinn, Jack and Elaine Schumacher

Young People of the Year: Kourtney Davis and Jasmine Snow

Caregiver of the Year: Jack Schumacher

Volunteer of the Year: Tommy Santora

Chairman's Award: Robert Pascuzzi, MD

Nurses of the Year: London (Ontario) University 7th Floor Nurses

Doctor of the Year: Janice Massey, MD

Mark your calendars for the 2014 national conference. It will be held in Philadelphia, PA, on April 16-18.

Myasthenia Gravis in Children

by Nancy L. Kuntz, M.D.

Introduction

Anyone who spends time with children realizes that they are delightfully unpredictable and that you frequently don't know what kids will do or say next. Children view the world and think differently than adults. The youngest children think concretely and magically: being mean to their pet or thinking a bad thought around the time that they develop symptoms leads them to believe that those actions caused their illness. Older children lose their magical thinking but become so anxious to be the "same" as all of their peers that they will deny or hide symptoms whenever possible to deflect attention from their problem. In addition to this, adolescents want to be independent to such a degree that they struggle with (and frequently rebel against) the common sense recommendations relating to adequate rest, nutrition and hydration that are part of managing health problems.

Symptoms of myasthenia in children

Most people reading this article understand the core symptoms of myasthenia gravis (MG): episodes of weakness provoked by activity or exercise and improved by rest. This weakness can affect various parts of the body and various activities such as speaking, chewing, swallowing, breathing, using your arms and legs or focusing your eyes so that you see a single object. All of these symptoms can occur in children with the various forms of myasthenia; however, it's sometimes harder to initially detect their problems and definitely harder to monitor the symptoms over time because of the characteristic behaviors just considered.

Symptoms vary between children and over time within an individual child. Symptoms are not always as easy to recognize in children. Ptosis or lowering of the eyelid is a common type of weakness noted in individuals with MG. Young children with ptosis are frequently felt to "look sleepy" and are put down for an early nap. If the ptosis is related to MG, it will likely improve after rest. It can therefore take a period of time before the family recognizes that there is facial weakness rather than a disrupted schedule. If children or teens develop swallowing problems, they may just stop eating or drinking as much and fail to gain weight. Adolescents may stop eating as much and lose weight without much comment and be felt to have body image issues or anorexia. If children develop arm or leg weaknesses, they frequently stop performing functional activities they had previously mastered. For example, they may stop feeding themselves, pouring their own milk or climbing stairs independently. Children who develop weakness with exertion do not have the exercise tolerance they previously had and frequently ask to be carried or stop and complain of leg pains, asking for a rest. Adolescents may just stop participating in social events or withdraw from sports without explaining why. In all of the above circumstances, it can be complicated to differentiate between behavior or mood issues, general issues relating to stamina and energy and fatigable weakness due to MG. These issues can delay initial diagnosis but also can make it complicated to manage the MG over time. As long as the child does not appear to have a crisis with respect to swallowing or breathing pending, it is always reasonable to enforce a rest period and reevaluate the child after the rest.

Forms of myasthenia in children

There are various forms of myasthenia in children which can present differently.

Congenital Myasthenic Syndromes: A rare group of disorders are caused by changes in the genes that code for the proteins controlling transmission of the nerve impulse to the muscle. Several dozen types of mutations in these genes have been identified which cause "congenital myasthenic syndromes" or CMS. The types of muscles involved and the severity of the weakness varies significantly from serious illness with feeding and breathing problems in newborns to milder forms of weakness involving the extremities that are not diagnosed until adult years. CMS is not autoimmune in nature and does not respond to the immunotherapy generally used in autoimmune MG. Not all forms of CMS respond to pyridostigmine (Mestinon™) and some might even worsen in response. Therefore, it is important to have these forms of myasthenia evaluated at a major medical center by specialists so that the appropriate treatment can be initiated. As forms of CMS are caused by genetic mutations, the potential for the disorder to occur in siblings or other family members should be evaluated by geneticists or treating physicians and the family.

Transient Neonatal Myasthenia: Autoimmune MG in mothers can be passively transmitted to some of their newborn babies (about 10% of their babies are affected). This can occur both in mothers with antibodies to acetylcholine receptors or MuSK receptors. With rare exception, the infants have mild to

moderate weakness noted at birth that usually responds to treatment and resolves within days to weeks. This phenomenon is well recognized by the high-risk obstetricians and neonatologists who care for mothers with MG and their babies.

Acquired Autoimmune Myasthenia Gravis: Autoimmune MG causes acquired weakness in children (from toddlers through young adults). Sometimes the symptoms are limited to the eyelids and eye movements and called "ocular MG". In other children, the symptoms are more generalized and involve extremity strength, speaking, swallowing and/or breathing. Some children have antibodies to AchR or MuSK receptors detected in their blood and others don't. However, if they have evidence on examination, on physiologic testing such as repetitive stimulation or electromyography and/or response to pyridostigmine-like medication, the diagnosis of autoimmune MG can be made without antibodies being identified. MG is a chronic autoimmune process and treatment involves both symptomatic and immunomodulatory/immunosuppressive treatment. Each individual's course with myasthenia is unique and their treatment plan needs to be individualized.

Treatment of autoimmune myasthenia gravis in children

Daily Schedule

1. **Regular Sleep:** Everyone's health improves with regular and adequate amounts of sleep. However, this is even more important for individuals with myasthenia gravis.
2. **Regular Activity and Exercise:** Children with myasthenia gravis need to have regular exercise to maintain the best state of fitness possible. It is reasonable to cut back on activities during acute illness or a myasthenic crisis in order to preserve energy. However, marked inactivity leads to poor fitness and increases the work of daily activities. Therefore, planning for regular activity is an important part of good health for children and adolescents with myasthenia gravis.
3. **Scheduled Rest:** Planning for quiet rest periods throughout a busy day or week is very helpful for individuals with myasthenia gravis. For example, not scheduling a birthday party on the same day as other after school activities like soccer practice or karate lessons is very helpful.

Medications

1) Symptomatic medication: Children with weakness due to autoimmune MG generally have improved strength if they take oral pyridostigmine (Mestinon ™) which acts by prolonging the effective life of the neurotransmitter acetylcholine at the neuromuscular junction. This is a short-acting medication and children take between 3-5 doses per day, depending on their symptoms and metabolism. This medication is available in liquid and tablet forms. Some individuals with antibodies to MuSK respond less well to pyridostigmine.

2) Immunomodulating/ Immunosuppressive medication: Symptomatic treatment with pyridostigmine does not influence the underlying cause of acquired autoimmune MG. At times, if the symptoms are restricted to ocular muscles only, start of immune therapy will be delayed while the child is observed to see whether their innate immunity will produce clinical remission. However, if a child or young adult has swallowing difficulties, difficulty breathing or severe extremity weakness, initiating immune treatment is important. There are a number of types of immune therapies. Some, including intravenous immunoglobulin and plasma exchange, may produce benefit within days to weeks. Others, including oral steroids, azathioprine and mycophenolate (among others) may take three or more months to improve symptoms from MG. More information regarding these medications is available on the MGFA website, <myasthenia.org>.

3) General medication principles:

1. **Dose and Timing are important:** Each child's best schedule of medicine for myasthenia depends on their size, their own type and stage of disease and other aspects of their health. However, it is important that once that BEST schedule is identified, that the children receive their medication with the right dose at the right time. If there are issues relating to after school care, parent's work schedule and so forth, it is important to discuss these with your child's doctor so you can develop a schedule for giving medication that can be kept up on a daily basis.
2. **Refills/emergency supply:** It is important to know when current supply of medicines will run out and to plan for refilling medications for your child's myasthenia. The amount of medication given to families at one time depends on the insurance companies who decide whether they will provide one month or up to three months at a time. Don't use medicine after the expiration date on the label. You should discuss the issue of emergency medication supply with your child's doctor: i.e. what phone number to call if the medication is lost or stolen either at home or while travelling. Including a supply of medication in emergency or evacuation plans for threatening weather systems (floods, hurricanes) is important.

3. **New medicines (prescription or over-the-counter):** Some medicines (including herbal medicines and ones available at drug stores without prescription) can affect the transmission of the signal between nerve and muscle. This could worsen a child's control over myasthenia gravis. Therefore, DO NOT give your child a new medication without discussing it with the pharmacist and/or your child's doctor. While there are some lists of medications to avoid in myasthenia, any written list can be quickly outdated. Always ASK before starting a new medication.

Thymectomy

Removal of the thymus gland from behind the sternum (thymectomy) has been shown to increase the longterm likelihood of clinical remission. This can be done by fiberoptic-guided surgery with shortterm recovery in children and young adults. Thymectomy does not improve myasthenic weakness quickly and should never be done in clinically unstable myasthenic patients. There is no evidence that thymectomy improves outcomes for individuals with MuSK-related MG.

Supervision and Support

Need to Know: Parents have the right to protect the privacy of their children, including who knows about the child's medical problem. However, as children grow, parents include other adults (family members, teachers, neighbors, group leaders and coaches) in a larger group of responsible adults who supervise their children during the day. Since myasthenia gravis can be unpredictable, it is important that parents create an environment where all responsible adults know that changes in strength or function in a child with myasthenia gravis are paid attention to. If a child develops double vision and complains of "blurry vision", an adult who doesn't know about the myasthenia may think the child has dust in their eye. An adult might think that a child who suddenly has garbled speech and drooling is "trying to be funny" or taking recreational drugs. If the supervising adults are aware of the MG and have a plan about who should be contacted, any change in function can be handled safely, quickly and with as much privacy as possible.

Accessing Emergency Care

1. **School Emergency Plan:** Schools assign responsibility to someone (generally a nurse) to identify children with various health problems (including asthma, serious allergies, immune suppression as well as myasthenia gravis) and to develop emergency action plans. Participation of parents and a child's doctor allows the school to know what specifically they might expect in terms of symptoms (although they understand that unexpected problems might also occur) and to know what the recommended action should be on their part. While paperwork is always a hassle, this opportunity to make suggestions and requests for action on the part of the school is a very valuable opportunity to protect your child's health.

2. **Authorization to Treat for Coaches, Group Leaders, Family/Friends who provide Supervision:** All people will receive emergency medical care under life-threatening circumstances. However, when the situation is less severe, adults providing supervision to your child needs to be able to either contact a parent or legal guardian immediately and at all moments to get authorization for medical treatment or to have written, notarized permission from you to provide that consent on your behalf. Since it is generally not possible to guarantee that phone lines might not break down or mobile phone transmission might not fail, it is important to think about this issue and consider providing authorization to selected adults. Sometimes having that authorization at the emergency room/institution that will provide the emergency care until you can be reached is most efficient.

3. **Meet/Greet EMS teams:** Myasthenia gravis is a relatively uncommon disorder. Therefore, in large cities, the emergency medical system will be familiar with this disorder. However, in small communities, particularly ones that depend on volunteer EMS teams, the initial responders may not be familiar with myasthenia and the possible, urgent health issues that can arise. In that setting, some myasthenic patients have felt that their care was delayed while an extended history or interview was attempted during which time their shortness of breath or weakness made it difficult to respond. This is an example of why it would be useful to contact the local EMS system and arrange for a scheduled meeting to make certain that the first responders and others are familiar with myasthenia gravis. Written materials can be obtained from the Myasthenia Gravis Foundation of America to provide needed background and a suggested outline of emergency response.

Summary

Myasthenia occurs both due to genetic mutations and due to autoimmune disease. Each child's myasthenia should be evaluated and treated on an individual basis. With diagnosis and careful monitoring and treatment, most children with myasthenia should be able to lead active normal lives.

Telling MY Story

by Don Giammo

I will never forget the monumental moments that have impacted my life… the abrupt announcement over the school intercom that President Kennedy was shot while riding in a motorcade in Dallas, Texas. I wouldn't experience another indelible 'kick in the gut' until the morning of September 11, 2001, when a co-worker ran into my office to announce that New York City was under attack.

I humbly submit the personal event that only I recognize as a life changing occurrence. I compare and categorize the sudden appearance of Myasthenia Gravis into my body with the events in Dallas and Lower Manhattan because everything was fine, and then in an instant, something went horribly wrong.

It was Monday, March 22, 2004. I had coordinated and participated in a tradeshow for my employer while in Los Angeles, CA. I worked hard the previous week to get everything ready for the opening of the show that morning.

At the conclusion of a successful day, we headed to a nearby restaurant. I intended to ask my co-workers to report to the booth 30 minutes before the show in the morning for a brief meeting, but without warning, in mid-sentence, my articulators shut down. I said "Let's plan on meeting at the booth around ninthe erthak er plaaahh…" It was as if a switch in my head was thrown from 'radio-ready diction' to 'slur-babble-implode.' I excused myself from the table and retreated to a neutral corner to analyze what had just happened. By remaining silent while trying to make sense out of my verbal collapse, I inadvertently allowed my articulator muscles to relax and return to normal. Outwardly, I seemed OK, but I wasn't.

The next morning, I was able to speak clearly. I thought the vocal nose-dive the previous evening was a sort of fluke occurrence. Two days later, I set up another show in Long Beach. I welcomed visitors to the booth. The unseen demon struck again. A prospective customer asked for a catalog. I handed one to him and said, "Here is our…" I wanted to say 'Steel Construction Literature' but I became absolutely incoherent. The man asked me if I was OK… I whispered… 'no'

When I returned to my main office in Ohio, I enjoyed about two weeks of 'normalcy'… I thought, 'lousy California smog…' In April, the slurring returned. At first it was a weekly occurrence, then it became daily. My general practitioner referred me to a specialist mainly because he didn't have a clue why I spoke like a drunk, looked like stroke victim and was unable to drink anything without it being rerouted through my nasal passages.

After testing that included MRIs, electronic probes and endless blood work, I was informed by the neurologist that I had a condition known as Myasthenia Gravis. I had never heard of it, my extended family had never heard of it, and 99% of northern Ohio had never heard of it.

Since I'm limited to 599 words, I'll cut to the chase… I took Mestinon, Imuran and Prednisone. Nothing worked to an acceptable level at first, but after more than a year of adjusted doses, I was able to repel the disease enough to maintain some duties from my desk.

My work suffered right along with me. I was no longer able to keep long hours, setup tradeshows or welcome visitors. In 2010, I was replaced by inexperienced people who could speak clearly, handle long hours… and outsource the work I did.

I am now getting my necessary rest because I am home a lot… forced to retire way too soon.

Living with MG

by Barry Levine

In 1975, I started getting MG symptoms: chronic fatigue and shortness of breath, double vision and intolerance to bright light. The symptoms were subtle and for short durations.

In 1993, I developed a brief bout of arm weakness. In 1996, the fatigue worsened for several weeks, and then it went away. In 1997, I experienced severe fatigue and breathing difficulty for six weeks, and then it disappeared for a year.

It returned with a vengeance. The MG generalized and affected my abdomen muscles, eyes, neck, throat, arms, legs and lower back. For the next two years, I went to a different specialist every few months: pulmonary, ENT, heart, and sleep disorder. Medical tests included CAT scan, MRI, Gastric, ultra-sound and etc. Finally, the medical community decided my problems were emotional because all of the testing had negative results. I was sent to a psychologist who found me normal.

In 1998, my arms got so bad that I was sent to a neurologist who diagnosed my MG. Two weeks later, I had my thymus removed.

For the next two years I got worse. To counteract the symptoms I was taking high doses of Imuran, Cellcept and Prednisone. I improved slowly, but my blood counts went sour so I dropped Cellcept.

In April of 2006, I was diagnosed with stage three NH Lymphoma.

The good news is that the type of lymphoma I had was a good cure rate. I started treatments of Chemotherapy on 5/15/06. I receive Rituximab followed by R-ICE a designer chemo cocktail. After a year of chemo, my body could not take any more of the toxic drugs and I still had cancerous tumors.My option was to reduce the treatment to a maintenance chemo until the cancer won or have a stem cell transplant that I was too old for. I was not ready to check out just yet, so I had the stem cell transplant anyway.

In April of 2007, I started preparation for a stem cell transplant using my own stem cells. The problem was my blood only yielded 8,000 of 1.2 million needed for the transplant. There was a trial drug that was for people who could not yield enough stem cells for the transplant. The med center got permission to use the experimental drug on me as a compassionate study. I was the first person to receive the drug outside of the study group.

The drug was successful! A few days later I began the stem cell transplant. A month later, I was discharged. I always will be grateful.

Six months after the transplant, I developed large cell granuloma in both lungs and had great difficulty breathing. I had central oxygen tubes throughout my home.

After a month in the hospital, the doctors could not determine the cause so they advised me to get my affairs in order. I waited for the end to arrive, but I never thought it would happen. Instead of my demise, after six months my lungs normalized.

A year later, I completely recuperated, but my body aged 20 years.

I never accepted the end was near. Maybe I was in denial, but I could not accept that cancer or MG was going to get me. My feelings have not changed, and I have reached my 76th birthday.

I am just getting started with my life!

In Memoriam

Robert (Bob) Howe, a dedicated myasthenia gravis (MG) advocate at the national and chapter levels, died on July 1, in Bowie, MD. Bob and his wife Carol Roman, who had MG, were tireless advocates on behalf of people who have MG. They were active members of the MD/DC/DE Chapter of the MGFA and served on the chapter's board of directors for several years, with Bob serving as the chapter's treasurer. Bob also was active at the national MGFA level as a member of the MGFA Finance Committee. After Carol's untimely death in 2011, Bob remained involved and dedicated to their mutual commitment. The MGFA is grateful for Bob's efforts.

Bob's generosity to non-profit organizations was not limited to MGFA. He was treasurer of the Humanitarian Foundation of International Order of the Blue Gavel; a founding member and president of the Pentagon Sailing Club; former Executive Director of the Naval Sailing Association; and president of Veterans on the Bay, a group of volunteer sailors that took injured vets from Walter Reed Army Medical Center and their families on sailing trips around the Chesapeake Bay.

For additional information and to sign the guest book, go to: www.dignitymemorial.com

MG Walks Raise More Than $1.5 Million Since Launch

Now in its third year, the National MG Walk Campaign's success is unprecedented for the Myasthenia Gravis Foundation of America. The combined efforts of MG Walkers have raised more than $1.5 million to fund critical research and programs of patient support and also raise awareness nationwide of the rarely publicized neuromuscular disease.

The MG Walk has raised awareness, renewed hope and worked to build a connected and caring community of those affected by MG. Through the MG Walk, patients along with families and friends unite their voices to say, "MG must be stopped. We need your help to find a cure."

In 2013, nearly 3,000 participants have already raised close to $500,000 and are approaching their year-end goal of $750,000. There are still several MG Walks remaining on the schedule, including 15 visits to several major cities such as Houston, San Antonio, Seattle, Indianapolis, Nashville and San Francisco. In addition, the "Virtual MG Walk" is ongoing, and anyone, anywhere can participate. Walks for 2014 will also be announced soon at MGWalks.org. Many walks will return to their cities from 2013, and new walks will be decided soon for 2014.
If your local community would benefit from an MG Walk event, contact the Walk Office to discuss a Walk in your area.

From media attention to walkers in their T-shirts to Walk materials in public locations, like in Walgreens pharmacies and doctors' offices, to literally thousands of participants raising funds by talking to everyone about MG and its effects on a person's life, the MG Walk Campaign is a nationwide, awareness-raising machine.

For information on the Walk, to find a Walk near you, and to register, visit MGWalk.org or call 855-MG-Walks (855-649-2557).

Follow the MG Walk Campaign:

Facebook.com/MGWalks

Twitter.com/MG_Walk

Pinterest.com/MGWalk

Congratulations to the Tri-State Mg Walk for Raising More Than $180K In 2013!

MG walk
For a World Without Myasthenia Gravis

Fall 2013 Walks

Register at www.mgwalk.org

September

9/21/2013	**DC Metro/Virginia MG Walk** Springfield, Virginia
9/22/2013	**Delaware Valley MG Walk** Avington Township, Pennsylvania
9/28/2013	**Indiana MG Walk** Indianapolis, Indiana
9/29/2013	**Illinois MG Walk** Napersville, Illinois

October

10/20/2013	**Pacific Northwest MG Walk** Seattle, Washington
10/26/2013	**Colorado MG Walk** Aurora, Colorado
10/27/2013	**Arizona MG Walk** Scottsdale, Arizona
10/27/2013	**New Mexico MG Walk** Albuquerque, New Mexico

November

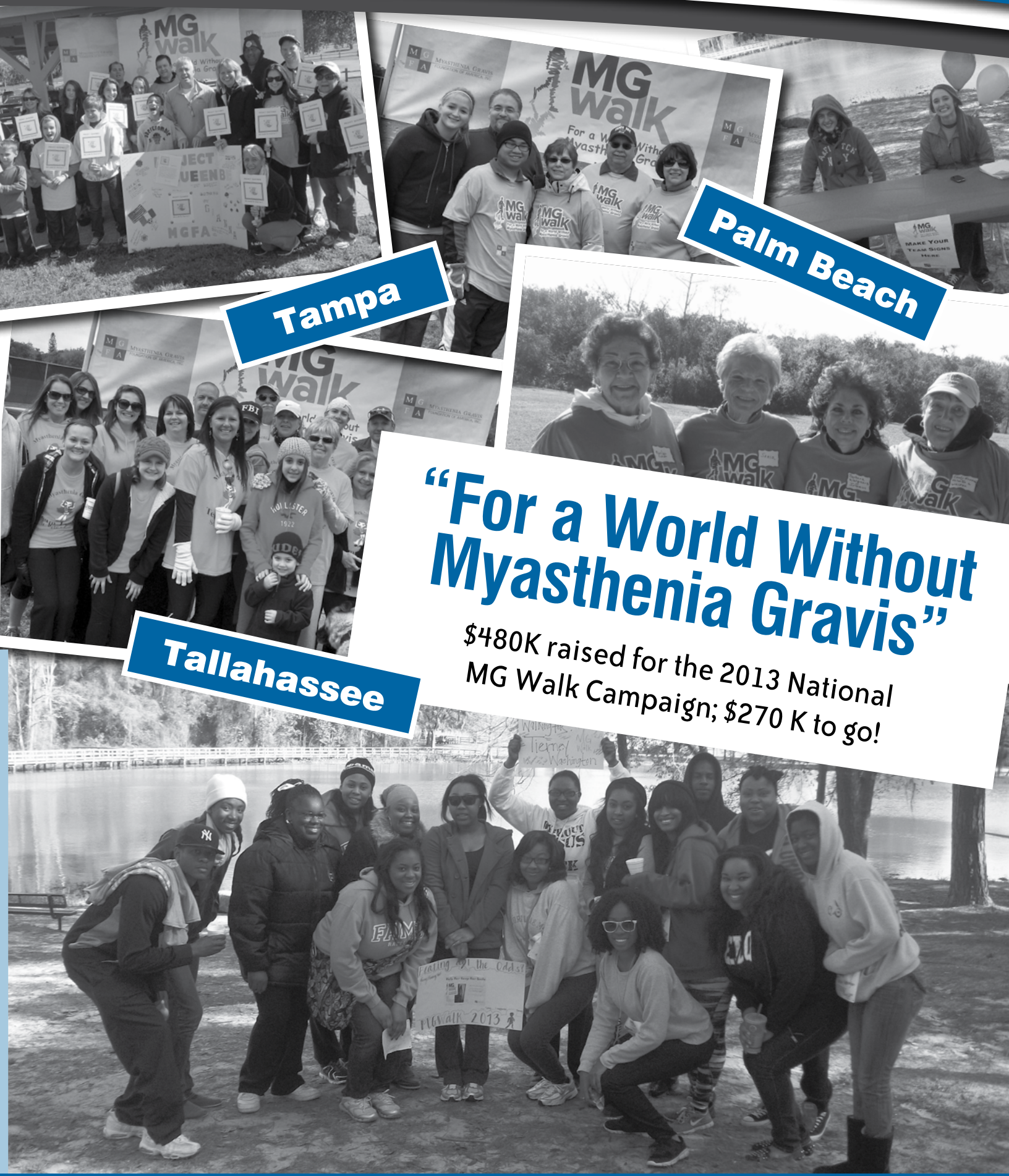
11/2//2013	**Pittsburgh MG Walk** Pittsburgh, Pennsylvania
11/2/2013	**North Texas MG Walk** Grapevine, Texas
11/3/2013	**Ohio MG Walk** Columbus, Ohio
11/3/2013	**Houston MG Walk** Houston, Texas
11/16/2013	**Tennessee MG Walk** Nashville, Tennessee
11/16/2013	**Southern California MG Walk** Santa Monica, California
11/17/2013	**Northern California MG Walk** San Francisco, California

Throughout the Calendar Year

Anywhere, Anytime	**Virtual MG Walk**

Thank You to our National Sponsor Walgreens!

"This is Why We Walk"



"For a World Without Myasthenia Gravis"

$480K raised for the 2013 National MG Walk Campaign; $270 K to go!


MG walk
Minnesota
MG walk
WHOEVER SAID WINNING ISN'T EVERYTHING HAS NEVER FOUGHT WITH MYASTHENIA GRAVIS
MG walk
New England


New Orleans
TEAM
ROSE
WE ♥ U
MG walk
For a World Without Myasthenia Gravis

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

Nicholas J. Silvestri, MD, Gil I. Wolfe, MD

Members of the M/SAB

Department of Neurology, Neuromuscular Division, University at Buffalo School of Medicine and Biomedical Sciences, The State University of New York, Buffalo, NY

Introduction

We would like to welcome MGFA members to a new column that will appear quarterly. Our goal is to highlight some of the latest developments in research related to neuromuscular junction disorders. Every column should include new developments in myasthenia gravis (MG), but we will be covering other disease states as well. For this inaugural column, we will lead off with congenital myasthenic syndromes. We will try to keep things to the point, in language that is understandable. Feedback will be appreciated. Happy reading!

Congenital Myasthenic Syndromes

Congenital myasthenic syndromes (often abbreviated as CMS) represent a wide variety of inherited disorders that compromise in one way or another transmission from the nerve to the muscle.[1] As we think most of you realize, this transmission must negotiate the neuromuscular junction. Most people who have CMS present in the first two years of life, with symptoms varying from problems feeding and breathing, droopy eyelids, reduced eye movement, poor muscle tone, muscle weakness and fatigue. Most forms of CMS arise only if abnormal genes are inherited from both the mother and father. We call this autosomal recessive inheritance. An abnormal gene coming from only one parent will usually not cause any problem. Many of the CMS disorders are caused by gene mutations for proteins that are part of the structural building blocks of the muscle membrane, including the acetylcholine receptor, the protein that is the immune target in most patients with MG. Recently, mutations in two proteins that are not structural but serve as enzymes, glutamine-fructose-6-phosphate transaminase (GFPT1) and dolichyl-phosphate N-acetylglucosaminephosphotransferase 1 (DPAGT1),[2] have been discovered in patients presenting with forms of CMS that impact the limb muscles far more greatly than the ocular muscles. This pattern is often referred to as limb-girdle myasthenia and is seen in several forms of CMS.

Selcen and her coauthors[3] from the Mayo Clinic group led by Dr. Andrew Engel that has defined many forms of CMS over the last 20 years, recently described the clinical features of 11 subjects with GFPT1 mutations. Problems in these individuals began anywhere from the time of birth to 19 years. Fortunately, all but one of these 11 individuals could walk. The weakness observed was in keeping with a limb-girdle pattern. Unlike some patients with CMS and most patients with MG, the authors did observe weakness below the elbows and below the knees involving foot and hand function. For those people who presented in early childhood, there were problems breathing, weak crying, problems eating and marked loss of muscle tone. As far as treatment, ten of the 11 patients had partial responses to pyridostigmine (Mestinon), a drug many of you take for MG. Other drugs such as 3,4-diaminopyridine (used often in Lambert-Eaton myasthenic syndrome), ephedrine and albuterol could also be helpful.

The loss of the GFPT1 enzyme activity caused by the gene mutations is believed to impact the proper structure of several proteins on or associated with the muscle membrane, thereby impairing proper transmission from the nerve to the muscle. What this research article should do is alert neurologists and other physicians to a clinical pattern of limb-girdle myasthenia that responds favorably to pyridostigmine. In addition, both GFPT1 and DPAGT1 mutations produce characteristic features on muscle biopsy that may also raise a clinician's suspicion for these disorders. Of course, these individuals will not harbor the antibodies in the blood that are typical for MG.

A common form of CMS is the slow channel syndrome, caused by mutations in the genes for the acetylcholine receptor.[4] This is the one form of CMS that can be inherited from a single abnormal gene coming from just one parent. So the inheritance pattern is quite different than the disorders we spoke about earlier, and is termed autosomal dominant. Many individuals with slow channel syndrome respond well to fluoxetine (more commonly known as Prozac) and quinidine (a drug used for heart rhythm problems). They get worse with pyridostigmine. Peyer and colleagues[5] recently described a patient

with slow channel syndrome who could not tolerate fluoxetine and was treated successfully with quinine, a medication that has a similar structure to quinidine. So this report suggests another treatment alternative for this group of individuals. Unfortunately, quinine, an old drug that is still sometimes used for malaria, is no longer easy to get your hands on in the United States, due to the FDA's concern about the effects it may have on blood cells and kidney function.

Myasthenia Gravis

Now, let's turn to MG. There have been several recent articles describing people with MG who often struggled with other treatments but had impressive responses to rituximab. Favorable responses have been seen in different types of MG, particularly in MuSK MG. In MuSK MG, an initial series of infusions of this manufactured antibody over a period of about 2 months can lead to a positive treatment response lasting years.[6] In a brief report, Catzola and coauthors from Italy[7] analyzed the effects of rituximab on regulatory T cells, white blood cells that are known to play a crucial role in preventing the host from attacking itself. There is good evidence that this class of white blood cells do not function properly in individuals with MG. The authors studied two patients with MG who were refractory to standard therapy. One had antibodies to the acetylcholine receptor and the other to MuSK. The MuSK MG patient had a dramatic response to rituximab; an increase in the regulatory T cells was also observed. The same clinical improvement and regulatory T cell boost were not observed in the other patient. Although limited by a single patient in each group, this report confirms prior observations that rituximab seems to work particularly well in MuSK MG and suggests that the drug's impact on these regulatory T cells may be largely responsible for the improvement. In this case, good disease control lasted for over 2 years after an initial series of the rituximab in the first month of treatment and a single dose 3 months later. Other medications were stopped and prednisone could be lowered. We think that the growing number of favorable reports for rituximab in MG begs for a formal drug trial. Efforts, in fact, are underway to conduct such a study through the NeuroNEXT clinical trials network funded by the NIH and all of our tax dollars.

Finally, steroid-sparing immunosuppressant agents are commonly used in MG. Black box warnings that alert doctors and patients to serious side effect for this class of medications typically include a risk for cancer, mainly lymphoma. But the data behind such warnings has largely been derived from individuals who have received these drugs to prevent rejection of transplanted organs such as kidneys and hearts. Actual studies in patients with neurologic disorders are few and far between. Pedersen and colleagues[8] recently performed a careful study on the risk of cancer in Danish MG patients treated with azathioprine (Imuran). Using several national databases in Denmark, they identified 89 patients treated with azathioprine who developed cancer and 873 controls (MG patients who also received azathioprine but did not develop cancer). They found a slightly higher rate of cancer with long-term use of azathioprine, defined as duration of treatment for 5 years or more. The increase in the rate of cancer over the control group was less than 25%, so considerably less than even a two-fold increase. The patients who developed cancer tended to be older men. The total number of cancer cases was too small to estimate the increased risk of specific types of cancer, but lymphoma was more common for those patients receiving longer treatments and higher doses. Larger studies will be necessary to clarify what the risk is for specific cancer types. But for now, it does appear the increased risk of cancer with azathioprine -- one of the oldest and most commonly used steroid-sparing agents in MG -- is fairly modest.

See you next time.

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Former MasterChef Winner Continues to Raise Awareness, Funds for MG

FOX Television MasterChef Season One winner and cookbook author Whitney Miller, who has been an advocate of raising awareness and funds for MG since her father John Miller was diagnosed, has been active in June and July, promoting the MGFA's partnership with ZOKU and Miller's popsicle maker fundraiser.

Whitney, a resident of Poplarville, Mississippi, has appeared in the media on ABC Birmingham News, Mississippi Public Broadcasting, Fox 8 New Orleans and in a story in the Picayune Item.

ZOKU donated to MGFA 20% of each of their popsicle makers sold on their site, www.zokuhome.com, when someone uses the code MGFA during checkout.

Whitney reports that the fundraiser has sold more than 100 popsicle makers for a fundraising total of close to $500.

You can view Whitney's "Cooking Tips if You Have MG" on her web site at:

http://whitneymiller.net/blog/cooking-tips-for-those-with-myasthenia-gravis

MGFA Welcomes New Board Members

Tommy Santora

Tommy is in public relations/marketing for the law firm of Adams and Reese, one of the largest law firms in the Southeast with more than 330 attorneys and advisors in 16 offices in 15 markets throughout the Gulf Coast and Washington, DC. A New Orleans native, Tommy has close to 15 years of experience in the journalism and communications field, formerly working for The Times-Picayune, CityBusiness and The Daily-Review. In 2011, Tommy founded the New Orleans Myasthenia Gravis Support Group, and in two years, the group has grown to more than 100 MG patients, family members and friends. The group has also raised more than $70,000 in two MG fundraising Walks, most recently raising $45,000 with more than 20 corporate sponsors and gathering more 350 people at the 2013 New Orleans MG Walk. Tommy has had MG for 22 years since the age of 12. In 2013, Tommy was recognized among the Top 50 "Health Care Heroes" in the Volunteer category by New Orleans CityBusiness magazine for his professional contributions and community service. In 2012, he was named among Gambit Magazine's "Top 40 Under 40," and also selected among the HUGS (Hope, Understanding, Giving and Support) volunteers of the year by his law firm for community service. Tommy earned his Bachelor of Arts in Mass Communications from Louisiana State University in 2002. In his spare time, Tommy is a freelance reporter/photographer, part-time ACT/SAT teacher for The Princeton Review, and plays softball three times a week and bowls. Tommy is the current Chair of the Communications Committee and serves on the Outreach and Resource Development Committees.

Michael Lifshitz

Michael has served on the Boards of Turtle Nest Village and Kids@Home, two programs designed to help young people transition from Foster Care to successful adulthood. He was a member of the UJA (now JFNA) National Young Leadership Cabinet, serving on the Executive Committee, as well as the regional and national committees for Oversees Allocations. With UJA, he was trained as a solicitation trainer, and has traveled to communities throughout the country teaching lay leadership effective fundraising skills and techniques. He has also been honored to work with the Peter and Vicki Halmos Family Foundation, helping them to promote opportunities in the arts for young people. Where most people have no idea what MG is, Michael comes to MGFA as the nephew and the cousin of two people fighting MG.

Jerry Faught

Jerry is a Director at Glendon Todd Capital and is part of the executive leadership team at Aztec Systems. His career has encompassed different leadership roles in the IT and technology infrastructure sectors with his expertise being revenue generation, change management, mergers and business transformations and building startup organizations. Shortly after his wife was diagnosed with MG in 2009 he became involved with the NW Texas Chapter of the MGFA where he serves as Chairman. Jerry earned his BS in International Business at Tarleton State University. Jerry serves on the Board Development/ Nominating Committee as well as the Chapter Relations Committee.

June Awareness Month Features Several MG Awareness Proclamations by Major U.S. Cities

During MGFA's official "June is MG Awareness Month," the national office, as well as chapters and individuals throughout the country, were involved in a variety of activities to promote awareness. These activities included obtaining proclamations from local and state officials, providing information about MG to the media, holding MG awareness events, disseminating information via social networks, and distributing MGFA's "The Faces of MG" June Awareness Month poster. Several areas, including New York, Boston and Nevada, issued proclamations, recognizing the month of June as Myasthenia Gravis Awareness Month.

This year, MGFA introduced two new awareness initiatives. The national office mailed the poster to over 2,000 neurologists throughout the country with a cover letter signed by MGFA's Chief Executive, Medical/Scientific Advisory Board chair, and Nurses Advisory Board chair. In the letter, they asked the neurologists to help expand awareness about MG by displaying the poster in their offices, informing their patients, and using any other means that they chose. MGFA invited all participants at the Miami annual conference to attend a new roundtable discussion entitled, "How to Energize Your Campaign and Get Media Attention." Attendees shared ideas and successes and received tips on how to get their message to the public.

Although the annual "month" has ended, awareness efforts at all levels will continue throughout the year in fulfillment of MGFA's mission to provide information and support to people with myasthenia gravis through research, education, community programs, and advocacy.

Chapters Corner

MGFA Upstate New York Chapter Support Group

- For June Awareness Month, Benita Zahn, WNYT Channel 13, interviewed Lynn Stone, one of our members. The interview was shown on June 18, 2013, during the 5 p.m. news.
- Posters, media letters and MGFA pamphlets were distributed to doctor's offices and pharmacies.
- Dr. Sheldon Staunton, Schenectady Neurologic Consultants, was a guest speaker at our Support Group on September 8, 2012. He was dynamic – he spoke with each person present. He is advising his myasthenia gravis patients to join the support group.
- An Amsterdam officer, Michael Palmerino, overcame myasthenia gravis to begin a career in law enforcement. When we saw the notice in the Albany Times Union in January 2013, we gave a donation in his name to the National MGFA.
- On April 28, 2012, two of our members traveled to Cortland, NY, to the New York Statewide Grange meeting to give a talk on Myasthenia Gravis. Of the 80 - 100 people in attendance, there were three people that had relatives or friends with Myasthenia Gravis. Pamphlets, neurology magazines and newsletters were available for handouts.
- Insisting on monthly meetings, rather than four a year, has been a valuable building tool. The friendships, cohesiveness, camaraderie among the members who attend the meetings is exceptional. The longevity of some of the members, the dedication of the newer members (from Board members to the refreshment committee) has sustained our group. MG has gripped all of us and to conquer the daily impact of this snowflake disease, we in turn are united in the fact that it takes all of us working together to manage the daily effects of this disease.

The overwhelming feeling of a need to tell how important this group was to each member or family member was clear. Finding others that look better/worse than you, sound like they have gone through the same things as you, truly do understand what you mean and feel, was amazing for each of us. It created connections, as well as close friendships, within the group. Our Support Group does that WELL! And we all seem to want to be there for newly diagnosed myasthenics and their families. We hope to take this opportunity to "Blow our own Horn," but also to really let others know the VALUE of a Support Group!

– Written by Helen Shufon, Support Group Co-Facilitator for the MGFA Upstate New York Chapter Support Group

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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.

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...to all of our wonderful donors!

Your generosity brings us closer to a world without MG.

MG walk
For a World Without Myasthenia Gravis

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!