# MG MATTERS

MYASTHENIA GRAVIS MID-ATLANTIC REGION

SUMMER, 2018

***June is Myasthenia Gravis (MG) Awareness month.*** Now and in the past, this means that we try to get the word out about MG. We distribute materials at health fairs, mail brochures to medical facilities for them to give MG patients, address nursing schools about our special needs. But where awareness of MG means the most is in emergency situations such as ambulances or hospital emergency rooms.

It is difficult, if not impossible, to include MG in medical school programs, nor can we influence the content of hospital databases about MG. In many cases we can't speak up for ourselves on our own behalf because if our breathing is compromised, so is our speech. What do we do, then?

Once again, we are providing emergency medical cards. When emergency providers arrive at your house, the second thing they look for, yourself being the first, is the card either on the refrigerator or in it. You probably don't even have to waste your precious breath trying to tell them where it is. It is also wise to have a copy at your bedside, in the box or on the shelf where you keep your medications, or in your purse, if you carry one. Having the card in your car is a good idea, too.

In addition to a medical alert bracelet you should be wearing, and the small card you should have in your wallet with MG information on it, you have the completed larger card with the medications you take and should avoid taking along with the phone number of your neurologist. You are well armed, so you think. What if your speech is impaired and you can't advocate for yourself? Or, worse, if medical personnel won't listen to you! That's when you really need the help of a someone who knows you, the problems your mg presents and the best way to help.

*Marika Bates*
*Patient Advocate*

*This issue is devoted to different kinds of "awareness". Awareness of how we present ourselves. Awareness of how to stay active and mobile, avoiding isolation. Does your voice sound different? Do you try to find the humor in living with MG? Are you aware of the support options available? Do you stay abreast of the latest research and new treatments and do you discuss all your options with your doctor?*

*Have a great summer and stay cool!*

*Lynn Waltz*
*Publisher*

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## SUPPORT GROUPS

**Glen Burnie SG**
**1st Saturday of each month**
**10:00 to noon**
Baltimore Washington Medical Center
Glen Burnie, MD
Marika Bates
410-507-3980 / marika_bates@gmail.com
Wanda Danney
443-742-4895 / wanda.avonlady@gmail.com

**Eastern Shore SG**
As requested. Contact Sandy Turner, RN
443-880-8271 / saundra.rn@gmail.com

**Bethesda SG**
**3rd Saturday of each month**
**10:00 to noon**
Suburban Hospital
Bethesda, MD.
William DeLorenzo
301-384-1299 / delorenz@umd.edu
Alan Sinclair
301-474-7745 / alsin6206@verizon.net

**Office & Helpline**
Lynn Waltz

**Phone Buddy**
**Pasadena, MD –**
Butch Jones @ 443-306-0252

**Washington, DC –** Betty @ 202-203-8696

**Teens – 30'ish –** Crysta @ 301-741-2747

**Support group leaders are also available as phone buddies**

**Website Manager – www.mgmar.org**
Jessica Dodson

**"MG MATTERS" Newsletter (quarterly)**
Publisher – Lynn Waltz
Editor – Johanna Monka, RN

**Mark your calendar for our**
**Annual Meeting on September 8th**
10 – Noon
Baltimore Washington Medical Center

**Speakers from CSI Pharmacy will discuss**

*Picking the Right Healthcare Plan

* Access to Affordable Health Insurance

* Ensuring that your medications are covered in the best & most affordable site of care for you

* How to get equipment you need covered

* How to ensure you are getting the best care in the home

* How to get your own patient advocate

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***Thank you for your support***

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***In honor of Dr. Charlene Hafer-Macko***

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MYASTHENIA GRAVIS

***Order this car magnet and help raise awareness. $10 includes postage!***

***Any medical views expressed in this newsletter are those of the individual author and do not reflect any official position of MGMAR or MG MATTERS publisher/editor.***

***Always contact your own physician who knows your situation best.***

InfuCare Rx

# Proudly Supports Myasthenia Gravis Awareness Month

Myasthenia gravis (MG) is a chronic neuromuscular, autoimmune disorder that causes varying degrees of weakness involving the voluntary muscles of the body, specifically those that control eye movements, eyelids, chewing, swallowing, coughing, facial expression, breathing and movements of the arms and legs. At present, the cause of Myasthenia gravis is unknown. Myasthenia gravis in the United States affects approximately 20 out of 100,000 people in the population. However, myasthenia gravis is probably under diagnosed and the prevalence may be higher. Myasthenia gravis occurs in all races, both genders, and at any age.

InfuCare Rx is a leading provider of home immnuoglobulin therapy (IVIG) therapy for autoimmune disorders, including myasthenia gravis. Reimbursement for IVIG can be complex for the treatment of myasthenia gravis. Our expert staff of reimbursement specialists completely handle the authorization process and counsel patients prior to therapy.

- All supplies, medication and equipment are shipped directly to you before the treatment date.
- An experienced, highly skilled infusion nurse will be with you throughout your home treatment.
- Infusion dates and times are scheduled according to your needs, including evenings and weekends.
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- Our patient-centric care model offers all brands of IVIG, while focusing on education and communication.
- Our expert staff of pharmacists, nurses and reimbursement specialists closely monitor patient's response to therapy.

Please contact our office to determine if home IVIG is right for you.
Please call us toll free at (877) 828-3940 or visit www.infucarerx.com.

MYASTHENIA GRAVIS

## *People* React *to the Reality You Present*

*There are few things as complicated and challenging as interpersonal relationships. The older I get, the more difficult it is to stay in touch with those I once considered to be friends. Supporting someone with a chronic illness will never be an easy task, just as living with one is not.*

*After falling ill, I soon realized that losing some friends along the way would be inevitable, yet there were still a few who stuck around. A couple of friends accepted the challenge of having a partially disabled friend and seemed to even excel at it. They seemed to accept the new sick version of me, but not without some hiccups along the way, of course. There were more than a few awkward situations my health elicited, but the difference between the friends who stayed and the ones who faded away was that we approached these situations together as a team. They offered to help and (this part is important)* ***I accepted their offer****. Friendships are a two-way deal: You have to give a little to take a little.*

*Maintaining friendships with those around you while constantly feeling like a stranger in your own body can be quite daunting. The thought process that has vastly helped me is reminding myself that people around me can only respond to the reality I present them with. Most myasthenia gravis patients have probably been told, "You don't look sick." That statement is important to remember, as infuriating as you may find it. We hardly ever look as bad as we feel. I've often thought on bad days that it is impossible for my looks to accurately represent how horrible I truly feel.*

*If you saw a fruit that looked ripe, you would realize within the first bite that it was not. This does not change the fact that the fruit appeared to be ripe, but we first need evidence to the contrary to change our minds. So, I have learned to respond to the infamous line, "You don't look sick," with, "I feel really tired today, but I am glad I have you fooled!" As Katharine Hepburn said, "If you want to change attitudes, start with a change in behavior". I may not be able to control the behavior of those around me, but I can control how I behave and respond.*

*I have lost more than a handful of people I once considered friends when my health started fading away. However, I can honestly say that the lasting friendships are the ones that have sometimes brought me to tears in awe of their abundance of selflessness and love.*

*https://myastheniagravisnews.com/2018/05/18/myasthenia-gravis-affects-relationships-control-your-reaction/*

## With Myasthenia Gravis, It's Important to Be Aware of Your Voice

We express ourselves in many ways: through our hairstyle, our clothing, our facial gestures, our voice, and how we choose to articulate our thoughts. One symptom of myasthenia gravis is dysphonia, which results in an unreliable and fading quality of voice. Another symptom is having difficulty with speech, which can sound like drunken slurring.

Before my diagnosis, I wanted to pursue a career in speech therapy. I didn't know how myasthenia gravis could be affecting my voice, because I only developed noticeable bulbar symptoms after starting university. Initially, my main concerns were my difficulty with breathing and weak arms and legs. I did not even think about the quality of my voice until we discussed the effect of myasthenia gravis on voice in our academic modules.

Suddenly, I became very aware of my voice, and I realized that my symptoms were not isolated to my larger extremities. At first, this realization left me feeling angsty about how effective I was at monitoring my symptoms. I felt that my health was completely out of my control.

Eventually, I abandoned the self-pity and anxiety-driven thoughts that were preventing me from thinking logically about the situation. The varying quality of my voice was not a new symptom. I found that being aware of the change in the quality of my voice and articulation was a way to monitor my symptoms. After all, these are not new symptoms; they are merely clues that my body gives me to solve the ongoing mystery of "how much can my body do today?"

I learned that when I was feeling short of breath, I could avoid speaking in long sentences. When I was able to speak in longer sentences, I would know that my breathing had improved. If I could not speak at a normal loudness and people had difficulty understanding me against background noise, I knew that I would have to take it easy that day. I realized my voice symptoms could be used as tools to help me to stay in control of my health. I saw an opportunity to empower myself and become an educated patient.

It is important never to lose your inner voice, even when you are not physically able to speak. Never stop fighting.

https://myastheniagravisnews.com/

## MOBILITY MANAGEMENT

By Trudie Mitschang

WHILE MOBILITY issues are often associated with aging, these difficulties can also be the result of many types of chronic illness. Mobility impairment refers to the inability to use one or more of a person's extremities. It may also involve a lack of muscle strength to walk, grasp or lift objects. Obviously, the inability to move freely and the subsequent loss of independence can have both a physical and emotional impact. The good news is, thanks to technology and innovations, a wealth of products are available to assist those who find themselves mobility impaired.

Mobile technologies and apps can offer life-changing support for individuals with physical disabilities. Mobile devices can be used as remote controls for any number of gadgets, while wearable sensors can make it easy to summon help or track a person's location. Some apps and texting services can help users maintain medication regimens, and various hands-free devices, including smart keyboards and dictation apps, can help those with hand strength and dexterity issues.

Walking canes are designed to help people with mobility issues improve balance and walk safely. One innovation in the pipeline is the new Dring Smartcane (dring.io). The device debuted in France last year and features sensors that detect any unusual situation (fall detection, diminished activity, etc.). If that happens, the cane automatically alerts caregivers and family, without any action required by the user.

For a more low-tech solution, there are canes designed to meet very specific needs. Some offer adjustable features and can be customized based on height and left or right handedness. Others fold compactly for travel and come equipped with built-in LED lights. Many canes come in a rainbow of color options and with ergonomically designed grips for added comfort.

A transport wheelchair is a mobility chair designed for convenience, short-distance use and easy handling by a caregiver. Some come equipped with rugged wheels for outdoor use, while others are specially designed for use in the bath or shower. Lightweight and foldable, these wheelchairs are easily moved and typically fit in the trunk of most vehicles. There is even a model designed to fold into a bag to be carried over the shoulder.

Travel scooters not only provide powered mobility, but also transportability. Many are compact, lightweight and can easily be disassembled into separate components to load into a vehicle. Four-wheeled models allow for enhanced stability on rough terrain, and an ergonomic delta tiller can provide increased usability for those with limited hand strength. For all models, consideration must be given to how long the scooter can operate on a single battery charge.

For those who have difficulty traversing household stairs, a stairlift could offer the ideal solution. The most recognizable type is the straight stairlift that attaches to the stair treads by way of a rail on which a chair can glide up and down. These lifts are suitable for those who can walk, but not climb, and who don't need to transport anything up and down except themselves and small items that can be held on a lap. A variation would be a curved model that can be utilized with a winding staircase.

Those with a chronic illness do not need to let mobility issues prevent them from doing the things they love. Freedom in movement is possible with the right device. For help making the best device choice, consult with a physician or physical therapist who can offer recommendations.

TRUDIE MITSCHANG is a contributing writer for IG Living magazine. This article is reprinted with their permission.

## Study to Evaluate New Drug in Myasthenia Gravis Patients

A Phase II proof-of-concept study will evaluate the safety, tolerability, efficacy, impact of quality-of-life and assessment of pharmacokinetics and pharmacodynamic markers of ARGX-113 on up to 24 myasthenia gravis (MG) patients. ARGX-113 will be dosed on top of current standard of care, corticosteroids and/or immunomodulatory agents. Topline data from the study is expected in the second half of 2018.

ARGX-113 has the potential to eliminate patient symptoms while minimizing common side effects seen with current treatments by reducing the pathogenic IgG levels," said Nicolas Leupin, CMO at argenx. "The initiation of this Phase II study is an important milestone in understanding how ARGX-113 can be effective in a wide range of IgG-mediated autoimmune diseases, including additional orphan indications and larger indications like multiple sclerosis and lupus."

## Preventing Isolation

**By Abbie Cornett**

IF YOU HAVE been diagnosed with chronic illness, chances are that diagnosis didn't come overnight and it came with a big price tag. Chronic illness imposes a heavy toll of challenges that affect all parts of patients' lives, from simply accessing appropriate care to paying for it. Unfortunately, one of the most common challenges chronically ill patients face is also frequently overlooked by family and the medical community: isolation!

Isolation is most often a consequence of no longer being able to work or interact with family and friends as you have in the past. Actually, the sense of isolation doesn't happen overnight; it is a gradual process. Over time, you may receive fewer and fewer invitations from friends and family because your illness or physical limitations have caused you to frequently cancel plans in the past. Further, because of medical and other costs, you may not be able to afford to go out and do things you enjoyed before, even if you feel like it. Experts agree, it is extremely important for you and your loved ones to find ways for you to stay connected. If isolation is not dealt with, it can lead to loneliness and depression.

Here are some suggestions for preventing isolation:
Find a support group. Support groups, gatherings of people who share common illness, offer many benefits. They provide a great way to learn more about your illness, connect with other people who are dealing with the same issues, and learn what resources are available your particular disability. Oftentimes, these organizations hold in-person meetings and mixers, but there are also online support groups for times when you don't feel like leaving the house.

Use technology. Technology has created social networks that span the globe. The upside of technology is you don't have to leave the house to stay connected with family, friends and the world through text, Facebook, Twitter and Instagram, to name a few.

Don't give up on friends and family. Talk with them about your illness, and plan events that make allowances for your limitations. Be creative. Instead of going out to dinner and a movie, ask them to watch a movie with you at home and order takeout. Remember, it's about the quality of the time you spend together versus the quantity.

Start writing a blog. Blogging is a great way to express yourself and share your experiences. Keep in mind you may be able to impart valuable information to another person suffering from the same condition.

If you are having trouble staying connected, find a therapist who specializes in patients with chronic illness. A therapist can help you find ways to cope with your illness and the changes it has caused in your life. If you are housebound, many therapists offer sessions via Skype or FaceTime.

Having a chronic illness doesn't mean you are doomed to isolation. With a little work, preplanning and creativity, you can make lasting connections. The benefits are truly worth the effort.

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## Legislation

### 2018 Budget Act Provision Narrows the Gap for IG Home Infusion Therapy Reimbursement

In February, the Bipartisan Budget Act of 2018 was signed into law by President Trump that includes a provision to more adequately fund immune globulin (IG) infusion therapy in the home. Specifically, the provision creates a temporary transition service and education Medicare payment for home infusion beginning in 2019, remaining in place until the permanent benefit for home infusions established with the passage of the 21st Century Cures Act in December 2016 is implemented in 2021.

In section 5004 of the 21st Century Cures Act, reimbursement for IG infused in the home was changed from 95 percent of the first published average wholesale price to the average sales price (ASP) plus 6 percent, less a 2 percent reduction of payment due to the federal sequestration required under the Budget Control Act of 2011. As legislated, applying sequestration of 2 percent to 80 percent of Medicare payment portions changes the actual reimbursement to ASP plus 4.3 percent. As a result, the cost to purchase an IG product exceeds reimbursement for many specialty pharmacies.

Section 5012 of the 21st Century Cures Act allows the Centers for Medicare and Medicaid Services (CMS) to reimburse "qualified home infusion therapy suppliers" for providing infusion therapy services at home to beneficiaries covered under Medicare Parts B and D. This provision comes with a broad list of new requirements and standards for suppliers of home infusion, and it requires Medicare to reimburse home infusion therapy suppliers based on a single, all-inclusive payment. But, the act's effective date left a four year gap in adequate reimbursement. Therefore, with the passage of the Bipartisan Budget Act of 2018, that gap is reduced by two years.

CMS has until 2019 to determine what the payment rate will be.

## Using Humor in the Battle Against Myasthenia Gravis

**By Retha De Wit**

There are not many perks to living with myasthenia gravis, especially since it can be difficult for others to understand the fatigability and variation in our symptoms. However, there are also not many things that do not benefit from a having a good sense of humor.

The symptoms of MG and the associated medications and their side effects can often become rather overwhelming. This is especially problematic when you do not know if your current discomfort stems from a disease symptom or a new side effect from one of your many medications. Side effects can range from something mild like headaches or stomach cramps to something more life-altering like neuropathic pain, hair loss, or anaphylactic shock. The reality for those suffering from a rare disease is that the treatment options are limited. This means trying to live with the side effects are sometimes better than the alternative, which could be no treatment at all.

That is not exactly a comforting thought. Honestly, most of life with a rare disease is not a comforting thought. We are often so busy fighting our battle for survival or a decent quality of life with illness, that we forget to laugh a little. We forget to live a joyous life too — which we certainly deserve to do. Especially since we are more aware of the vulnerability of life than most of our peers.

Finding humor in your battle can be done in any way you feel comfortable. A good friend of mine decided she was going to name her symptoms “Susan,” and inform all of those around her of her current state of health by describing how “Susan” was feeling that day. I am fairly certain most of us can relate our unexpectedly flaring symptoms to the statement, “Susan is being such a bummer today.” My friend found a way to make relaying the state of her health a little more fun and a little less clinical.

Although I wish I could claim that idea as my own, I have my own less creative way of using humor to cope with life and my disease. Instead of being embarrassed when I lose my balance, or my foot drops and I stumble, I like to make jokes about wishing I was falling due to being intoxicated. In doing this, I make those around me aware that this happens because of my disease and not merely public intoxication. I acknowledge the presence of my fall, but I get more control in determining how they will react to this physical representation of my disease.

By embracing humor, I am more confident in explaining my disease. By embracing humor, people usually feel more at ease while dealing with challenging situations — like having a friend with a muscle disease fall in a public place. Humor tends to make things less intimidating to talk about, and casual conversation is a good way to start educating those around you about the reality of the struggles you face.

In my opinion, the key to happiness is to never stop laughing. Laugh at yourself, make a joke, or name your symptoms something ridiculous. Do whatever you can to find the humor in your battle. You are a warrior. Never stop fighting. Life is beautiful.

***If you haven’t already discovered the Myasthenia Gravis News website we highly recommend it…***

***https://myastheniagravisnews.com/***

## 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 colleagues 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.

## Catalyst Enrolls First MuSK MG Patient in Phase 3 Trial Evaluating Firdapse

Firdapse is a neuronal potassium channel blocker that works to improve nerve impulses to muscle. In MG, the body attacks special receptors on nerve cells that are sensitive to an important chemical called acetylcholine (ACh), This chemical is responsible for the communication across the neuromuscular junction to muscles, where acetylcholine receptors (AChR) interpret the message and cause muscles to contract.

A second chemical that is needed for healthy nerve-muscle communication is called muscle-specific kinase, or MuSK. Unlike AChR-MG, where the body attacks the receptor, in patients with MuSK-MG, their bodies attack cells that produce the MuSK chemical.

MuSK MG is a particularly severe form of myasthenia gravis and is estimated to affect between 3,000 and 4,800 people in the United States.

The MSK-002 trial (NCIQ33.mQ54) is now recruiting up to 60 people diagnosed with MuSK MG in the U.S. and Italy. The study's primary endpoint is the measurement of Myasthenia Gravis Activities of Daily Living (MG-ADL). The secondary endpoint is to measure Quantitative Myasthenia Gravis Score (QMG).

"By conducting this Phase 3 study in patients with MuSK-MG, we hope to provide a potential treatment option for people suffering from this rare condition," Patrick J. McEnany, chairman and CEO of Catalyst, said in a press release.

"There is a significant unmet medical need to treat the symptoms of MuSK-MG, and these patients are eagerly awaiting a new treatment option," said Gary Ingenito, an MD and PhD, and chief medical officer at Catalyst. "The previous Catalyst supported, proof-of-concept investigator-sponsored study in MuSKMG patients showed impressive clinical improvement in multiple measures.

"We're pleased to have begun enrolling patients in this study and look forward to working closely with the MG community to advance Firdapse through this Phase 3 trial," he added.

Firdapse was granted FDA orphan drug status for the treatment of MG and Lambert-Eaton Myasthenic Syndrome (LEMS). Firdapse in oral tablet form is approved in the European Union to treat LEMS.

Two other studies testing Firdapse are now underway in people with MG The first now completed, was a Phase 3 trial designed to evaluate the effect of withdrawing Firdapse from patients with I-EMS in a 50/50 ratio.

A second trial (NCI.Q2562Q66), now recruiting patients with congenital myasthenic syndromes (CMS), is evaluating the efficacy and safety of Firdapse in patients diagnosed with certain types of CMS.

Catalyst has filed a new drug application (NDA) requesting FDA approval of Firdapse as a LEMS treatment. The NDA could open a regulatory pathway for additional approvals in MG and MuSK MG.

## New theory on why more women than men develop autoimmune diseases

UNIVERSITY OF GOTHENBURG

New findings are now being presented on possible mechanisms behind gender differences in the occurrence of rheumatism and other autoimmune diseases. The study, published in Nature Communications, can be of significance for the future treatment of diseases.

"It's very important to understand what causes these diseases to be so much more common among women," says Asa Tivesten, professor of medicine at Sahlgrenska Academy, Sweden, a chief physician and one of the authors of the study. "In this way, we can eventually provide better treatment for the diseases."

In autoimmune diseases, the immune system creates antibodies that attack the body's own tissue. Almost all autoimmune diseases affect women more often than men. The gender difference is especially great in the case of lupus, a serious disease also known as systemic lupus erythematosus or SLE. Nine out of ten of those afflicted are women.

It has been known that there is a link between the male sex hormone testosterone and protection against autoimmune diseases. Men are generally more protected than women, who only have one tenth as much testosterone.

Testosterone reduces the number of B cells, a type of lymphocyte that releases harmful antibodies. The researchers behind the study were trying to understand what the connection between testosterone and the production of B cells in the spleen actually looks like, mechanisms that have so far been unknown.

After numerous experiments on mice and studies of blood samples from 128 men, the researchers were able to conclude that the critical connection is the protein BAFF, which makes the B cells more viable.

"We have concluded that testosterone suppresses BAFF. If you eliminate testosterone, you get more BAFF and thereby more B cells in the spleen because they survive to a greater extent. Recognition of the link between testosterone and BAFF is completely new. No one has reported this in the past," says Asa Tivesten.

The results correlate well with a previous study showing that genetic variations in BAFF can be linked to the risk of diseases such as lupus. That disease is treated with BAFF inhibitors, a medicine that has not, however, really lived up to expectations.

"That's why this information about how the body regulates the levels of BAFF is extremely important, so that we can continue to put the pieces together and try to understand which patients should have BAFF inhibitors and which should not. Accordingly, our study serves as a basis for further research on how the medicine can be used in a better way."

https://www.eurekalert.org/pub_releases/2018-06/uog-nto060518.php

## Rituxan Improves Muscle Strength, Allows Refractory MG Patients to Reduce Steroids, Study Says

Treatment with Rituxan (rituximab) provided sustained clinical improvement, increased muscle strength, and extended the time before disease worsening in myasthenia gravis, according to researchers.

It also allows myasthenia gravis (MG) patients who are resistant to conventional treatment to reduce their dose level of steroids, they said.

Their study, "Rituximab in refractory myasthenia gravis: extended prospective study results," was published in the journal Muscle & Nerve.

Rituxan is an antibody targeted to a protein called CD-20, present in B-cells, a type of immune cell that plays important roles in inflammation and involved in the development of MG.

When Rituxan binds to CD-20, it lowers the levels of B-cells, which may slow the progression of disease and reduce the need for other medications. Although this treatment seems beneficial in treatment-resistant (refractory) myasthenia gravis, long-term data was needed.

Researchers from the University of Alberta, Canada, performed a prospective, open-label study from 2012 to 2018 to evaluate the outcomes of treatment with Rituxan in patients with treatment-resistant MG.

A total of 22 patients received one of two rituximab regimens, followed by repeated maintenance cycles in the case of disease worsening, or relapses.

In regimen one (nine patients), Rituxan infusions of 375 mg/m2 were administered once a week for four weeks, then once every four weeks for two additional infusions.

In regimen two (13 patients), infusions of 750 mg/m2 (up to a maximum of 1 g per dose) were given twice, with two weeks between infusions. No serious adverse events occurred with infusions.

Patients were followed-up for a median time of 28.8 months; the primary outcome measure was the change in manual muscle testing (MMT) score, a test for evaluating the strength of muscles, from study initiation to the most recent follow-up.

Complete blood cell counts, liver enzymes and B-cell counts were monitored throughout the study.

Rituxan was associated with a significant improvement in muscle strength in patients positive for AChR (acetylcholine receptor) antibodies or MuSK (muscle-specific tyrosine kinase) antibodies.

No changes were observed in seronegative patients.

MuSK-positive patients showed the most improvement, with a mean 89% reduction in MMT scores, while AChR-positive patients demonstrated a 47% reduction.

Regimens one and two proved equally effective, leading to comparable improvements in MMT scores.

Treatment with Rituxan also enabled those patients taking prednisone (an immunosuppressant steroid) to significantly reduce their daily dose of the drug.

Another important effect seen in the treated patients was a significant extension in the time patients were without relapses, following a single cycle of Rituxan.

The average time to first relapse was 17.1 months and the average time to second relapse following a second cycle was 13.5 months.

Ten relapses occurred, with the average time to first relapse of 17.1 months; these were not associated with recovery of B-cell counts.

Three patients experienced prolonged B-cell depletion after one cycle of Rituxan, representing less than 1 percent of CD19-expressing B-cells for 24 to 34 months.

All other patients recovered their B-cell population in an anticipated time period of 12 months.

Rituxan may be a promising therapy option in patients with refractory myasthenia gravis, but researchers caution physicians to closely monitor patients' B-cells.

"Given the risk of infection inherent with prolonged B-cell depletion, clinicians must be aware of this possibility, and we would advocate continued monitoring of B-cell counts in all patients receiving rituximab," researchers wrote.

https://myastheniagravisnews.com/2018/05/29/myasthenia-gravis-rituxan-improves-muscle-strength-long-term-data/

## A Patient's Guide to Nonprofit Support

By Trudie Mitschang

**PEOPLE LIVING WITH** rare diseases need a variety of people on their healthcare and support teams to help them navigate the ups and downs of symptoms and treatment strategies. Statistically, these patients are not alone; more than 30 million Americans have been diagnosed with some type of rare or chronic illness. But, that doesn't mean having a diagnosis isn't extremely lonely, and knowing where to seek help when problems arise can offer tremendous peace of mind.

### Finding Needed Help

Even a decade ago, only a handful of nonprofits specialized in rare diseases. Today, there is a dedicated group for almost every need and diagnosis. The first thing to know is that, by definition, a nonprofit simply refers to the business structure of the entity itself, but the organizations offering assistance to patients fall under several categories:

**Disease-specific groups**

Finding a group that specializes in a specific diagnosis can offer tremendous practical and emotional support. Once a group is found, it's important to read its mission statement to make sure the services it offers are in line with individual needs. For example, some groups exist to drive research and education, while others offer services that range from finding healthcare providers, lobbying with state legislators or hosting patient events and conferences.

**Alliances**

Sometimes, nonprofit advocacy groups partner with one another to form an alliance. An alliance exists to tackle large issues affecting patients and, in some instances, to serve as an umbrella group for the advocacy of all patients with rare diseases. These groups may also offer resources and support to individuals with specific medical conditions, but their primary focus tends to be aimed at assisting the rare disease community as a whole.

**Support groups**

Support groups can help patients deal with the larger issues related to a chronic illness or may focus on symptoms related to the disease such as loss of mobility, chronic pain or vision impairment. They may have regional and local chapters, and many have online forums. When considering joining a group, testing the waters online may provide opportunities to meet others who share symptoms and challenges. Whether in person or online, support groups can empower patients to become their own healthcare advocate.

**Advocacy groups**

Patient advocacy groups provide many helpful resources for patients struggling to adjust to life with a rare disease. In many cases, financial assistance, access to clinical trials or assistance in attaining special medical equipment is offered. Some groups offer counseling and assistance for patients dealing with school or employment issues or health insurance concerns. Another helpful resource often found on advocacy sites are physician directories that are typically searchable by specialty and/or state and ZIP code. Because advocacy groups typically have a medical advisory board made up of experts in the field, patients can consider their recommendations trustworthy.

**Educational groups**

Once patients receive a diagnosis, the first thing they are likely to do is "Google" information about their disease. The problem is, many online "experts" are anything but, and much health-related information online is unreliable at best. A nonprofit educational research group may provide medical information in easy to understand terms to help patients learn more about their condition, while also providing insights on available treatment options and trending research. Larger, national nonprofits are considered a trusted resource for up-to-date information regarding even the most rare diseases.

**Living with a rare disease presents many hurdles, but help is available.** Whether patients want to connect with other people who understand how having a chronic condition affects their quality of life, need medical and treatment information, would like to participate in a clinical trial, or want to access resources for financial assistance, a nonprofit organization dedicated to a specific disease can offer a wealth of much-needed support and guidance.

TRUDIE MITSCHANG is a contributing writer for IG Living magazine.

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**Myasthenia Gravis Research & Abstracts**

The following items are summaries for abstracts of professional research studies relating to MG. They are for your information only and the intention is to keep patients and providers up-to-date with current MG research.

To read the complete report, go to the link following the abstract.

## Viral Infection Could Be Contributing Factor for Myasthenia Gravis, Study Says

Infection of the thymus by the virus that causes fifth disease — the human parvovirus B19 — can induce thymus overgrowth (hyperplasia) contributing to the development of myasthenia gravis, researchers have found.

Reported in a study published in the journal Clinical Microbiology and Infection, this finding adds new insights on the possible causes of myasthenia gravis. The study is titled "Detection of human parvovirus B19 infection in the thymus of patients with thymic hyperplasia-associated myasthenia gravis."

Myasthenia gravis is characterized by the increased production of antibodies that prevent the proper function of the AChR protein, which is essential for normal nerve-muscle cell communication.

Although it's still unclear what causes this disease, thymus overgrowth has been shown to play a role. More than 80 percent of patients with early-onset myasthenia gravis have thymic hyperplasia and 10-15 percent of patients also have thymoma, a benign tumor in the thymus.

The thymus is a small organ responsible for the normal development of immune B- and T-cells, playing a key role in regulation of the immune system.

It's been proposed that increased thymus inflammation caused by viral infections may contribute to autoimmunity, where the immune system attacks healthy cells in the body. In particular, B19 virus infection has been associated with the production of several autoantibodies — the antibodies that attack the body.

Based on these previous findings, researchers in China proposed evaluating the impact of this virus on the thymus of myasthenia gravis patients.

They analyzed 138 thymus tissue samples, of which 68 were representative of thymic hyperplasia, 58 of thymomas, and 12 were collected from healthy volunteers. Among these, 39 thymic hyperplasia and 23 thymoma samples were from myasthenia gravis patients. They identified 28 cases of thymic hyperplasia and two cases of thymomas infected with the B19 virus. Of these, 13 were from myasthenia gravis patients. None of the healthy thymus samples were positive for the infection. Interestingly, the frequency of infected thymic hyperplasias was higher in the non-myasthenia gravis group.

When the team looked at the presence of viral proteins — a measure of active infection — they found that thymic hyperplasia with myasthenia gravis samples were more affected than those without the disease (74.36% versus 48.28%). Additionally, the proteins were mainly found in the sites where B-cells are, which could directly impact antibody production.

"Since the presence of viral protein indicates viral replication in tissues, it may also simultaneously imply a causal link with the disease," the researchers wrote. They also believe that these "findings revealed a previously unrecognized" cause of thymic hyperplasia-associated myasthenia gravis, "evoking numerous questions that require further investigation."

They are planning to evaluate the impact of B19 virus infection on B-cell expansion and activation in the context of myasthenia gravis in a future study.

https://myastheniagravisnews.com/2018/04/12/b19-viral-infection-possible-contributor-myasthenia-gravis-study/

## *Enrollment Is Open for MG Trial*

Investigators assess the safety and drug effects of RA101495

Researchers are looking for people with MG to participate in a phase 2 clinical trial, sponsored by Ra Pharmaceuticals, to test the experimental drug RA101495 in people with generalized MG who have tested positive for acetylcholine receptor (AChR) autoantibodies.

Administered subcutaneously (an injection under the skin), RA101495 is designed to prevent the body's attack on the space across which nerve fibers transmit signals to muscle fibers, called the neuromuscular junction (NMJ).

RA101495 acts by targeting and blocking a part of the immune system called the complement system, which is responsible for helping antibodies clear damaged cells and potentially toxic microbes that could cause infections. In patients with AChR autoantibody positive generalized MG, the body's own immune system turns on itself to produce antibodies against the AChR (a receptor located on muscle cells at the NMJ), activating the complement system.

Goals of the trial are to assess the safety of RA101495 and determine whether the treatment is able to reduce muscle weakness in people with generalized MG.

To be eligible to participate, individuals must have a diagnosis of generalized MG, test positive for AChR autoantibodies and meet additional criteria.

The trial is taking place at 27 trial sites across the United State and support for travel costs may be available. Trial length is approximately three months, during which participants will visit with study investigators in person seven times. At in-person visits, treatment effects will be measured across two MG-specific assessment scales, along with other clinical tests.

To learn more about this trial, visit clinicaltrials.gov and enter NCT03315130 in the "Other Terms" search box

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