



MYASTHENIA GRAVIS ASSOCIATION OF NEW ENGLAND

SPRING 2018 – Newsletter Volume 3

Welcome to the Spring edition of the MGANE Newsletter. We hope that you will find this information interesting and helpful. If you have any suggestions for our newsletter or would like to contribute “your story”, please contact Denise L. Trombly @ Trombly1@myfairpoint.net

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What is the Myasthenia Gravis Association of New England – MGANE

MGANE is a volunteer organization whose mission is to improve the quality of life for all people affected by the neuromuscular disease, Myasthenia Gravis. Our intent is to raise public awareness and promote education of patients and their families, and health care professionals. We welcome participants from throughout New England. Meetings of MGANE are held at the Newton-Wellesley Hospital in the Allen Riddle Building. For more information, or to join, please go to our website at www.mgane.org or 508-435-3808.

The MGANE Board of Directors:

Executive Committee: Pam Cerullo, President; Glenda Thomas, Vice-President; Jill Robbins, Clerk; Jim Buckner, Assistant Clerk

At Large Members: Tom Atherton, Jim Cerullo, Jeff Palmer & Denise Trombly

PATIENT ADVOCACY

At our last meeting, Dr. Seward Rutkove and Board member Glenda Thomas presented information on how to effectively communicate with your neurologist and physicians as you navigate through MG. Their presentation emphasized the importance of the patient taking an active role in his or her health care. In that way, the physicians can increase the effectiveness of therapeutic activities by incorporating patient input into the decision making. He customizes his approach for each patient based upon their personality and medical condition. He believes that a key component to a good and long lasting relationship is trust and respect for both parties.



Dr. Rutkove is a Neurologist affiliated with Beth Israel Deaconess Medical Center in Boston. He received the Americas Top Doctors award and has over 100 publications and presentations to his credit

NEWS YOU CAN USE

Phone App - The MyMG phone app contains information about MG and drugs that are inadvisable and possibly dangerous for MG patients. This is especially important in emergency situations.

Myasthenia Gravis Foundation of America, Inc. (MGFA) --- Information nationwide regarding MG research, helpful podcasts by physicians in the field of MG research, and additional support information for those affected with MG, including the Patient Registry for MG patients. In November there was an excellent Webinar on the new drugs that are on the horizon for MG patients. You can access through: <http://www.myasthenia.org>
MGFA also has an E- Newsletter to which you can subscribe.

MGANE --- Our local organization is here to help support those in the New England area. Please visit our website at www.MGANE.org, our Facebook page at Myasthenia Gravis Association of New England MGANE, or our email contact at Support@MGANE.org. There is also an MGFA support group that meetings Rochester NH contact Deb Correia (deblcorreia@yahoo.com) Meetings are in Rochester NH Every 3rd Thursday at 6PM.

Maintaining
Your
Attitude
Sense of humor and
Timing
Hope
Enthusiasm and
Normal life
Is
Absolutely essential

The Assistance Fund Opens Copay Program for Individuals with Myasthenia Gravis

The Assistance Fund, an independent charitable patient assistance foundation that helps patients and families facing high medical out-of-pocket costs, announced the launch of a program to provide financial support to patients with myasthenia gravis. “Perhaps there is nothing more frustrating for patients with a life altering disease like myasthenia gravis (MG) than knowing that there are treatments that can help, but not being able to afford them,” said Nancy Law, Chief Executive Officer for the Myasthenia Gravis Foundation of America (MGFA). Support for medication/treatment only includes FDA approved therapies, that is Edrophonium chloride; Enlon; Mestinon; Neostigmine bromide; Prostigmin; Pyridostigmine bromide; and Soliris. Treatments such as IVig; PLEX; Cellcept and others cannot be covered. Contact MGFA for more details.

Interview with Dr. Pushpa Narayanaswami



Dr. Narayanaswami is affiliated with Beth Israel Hospital and has received a grant from the Patient Centered Outcomes Research Institute (PICORI)

DT: Can you tell us a bit about your current research project?

Dr. N: The focus of this grant is on the reported outcomes by actual patients as measured by Quality of Life surveys and clinical outcomes. It will compare medications such as microphenolate, azathioprin etc with the real world experience of our patients. It will examine side effects, effectiveness (minimal manifestation) of the patient population. It is a real world trial (in contrast to randomized clinical trials) in 20 different centers. This is an observational study.

DT: Who will benefit from this research?

Dr. N: Since this will provide insight into actual MG patients (as reported by them), it has the potential to benefit many.

DT: Who qualifies for the study?

Dr. N: Newly diagnosed MG patients will be engaged in the study through their physicians. There are several centers throughout the Boston area.

CURRENT RESEARCH

There is a considerable amount of good news these days for MG patients as you will see in the following newsworthy pharmaceutical report courtesy of MGFA E newsletter:

MG Treatment Gets First FDA Approval Since 1950s

For more than 60 years, steroids and acetylcholinesterase inhibitors have been widely used as therapeutic options for patients with myasthenia gravis (MG), mostly because alternative means of immunotherapy have always been associated with serious adverse events (AEs). Alexion Pharmaceuticals, Inc. announced that the U.S. Food and Drug Administration (FDA) has approved Soliris (eculizumab) as a new treatment for adult patients with generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody-positive. The drug exhibited clinical benefit for these patients who had previously failed immunosuppressive treatment and continued to suffer from significant unresolved disease.

Clinical Trials:

Clinical Trial Begins for Firdapse A new clinical trial to determine the efficacy and safety of amifampridine phosphate in improving the activities of daily living for patients with antibody positive MuSK myasthenia gravis has opened.

Belgian Antibody Proves Effective to Treat Myasthenia Gravis Argenx has released the results from a Phase II trial with ARGX-113 (efgartigimod), in which the antibody therapy has proved to provide a significant improvement in patients with generalized myasthenia gravis. A total of 24 patients were recruited for the trial. They all received the standard care for myasthenia gravis (corticosteroids and/or immunomodulatory agents) in addition to four weekly doses of either ARGX-113 or placebo. After 6 weeks, 75% of patients treated with ARGX-113 showed a significant improvement in their symptoms, compared to just 25% of those receiving the placebo. A clear improvement was seen starting from the first week after the first injection.

Phase 1 Trial of GTP-004 Initiated as Therapy for Myasthenia Gravis Treatment Side Effects GT Biopharma has launched proof-of-concept Phase 1 trial of its investigational therapy GTP-004 for the treatment of MG. The trial's primary goal is to demonstrate the therapy improves the gastrointestinal side effects of current treatments. GTP-004 is a fixed-dose combination tablet of two approved therapies – Mestinon (pyridostigmine) and an antagonist to Mestinon's gastrointestinal side effects.

New RA 101495 Clinical Trial

RA Pharmaceuticals is recruiting participants for a new clinical trial. This Phase 2 Study seeks to evaluate the safety, tolerability, and preliminary efficacy of RA101495 in subjects with generalized myasthenia gravis. 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. The trial will last approximately 3 months and will include approximately 7 visits. It is taking place at 21 sites across the country. Patients will also have the possibility to continue receiving the study drug after they complete the study. For more information about the trial, visit [MGFA Clinical Trials](#) listed on the myasthenia.org website and/or visit clinicaltrials.gov.

Department of Neurology, St Elizabeths Hospital



What are the side effects of rituximab?

Rituximab is an intravenous treatment designed initially for cancer treatment and used more recently for immune diseases such as rheumatoid arthritis or multiple sclerosis. It has been used in recent years for patients with refractory MG and the results have been encouraging. A well-designed and relatively large trial of Rituximab in MG has recently completed enrolling patients and the results should be available in the next year.

Rituximab has a number of side effects and potential risks. Before treatment even starts with this drug, it is important to check for evidence of prior or active hepatitis B viral infections. Reactivation of the virus can occur with Rituximab and can cause a serious reaction. The presence of the virus would be a reason to try and avoid Rituximab.

The most common side effects during treatment are infusion reactions. During the infusion of the medication, a variety of different typically mild and unpleasant but not dangerous symptoms such as fever, headache, chills, nausea, itching, rash, fatigue and/or dizziness (with a drop in blood pressure). These symptoms can be reduced by taking acetaminophen +/- or diphenhydramine prior to treatment and this is usually done. Rarely, the infusion reactions can be severe and dangerous but fortunately this is extremely uncommon. This type of infusion reaction typically occurs during the first infusion and is less likely thereafter. Another very rare but severe complication is the development of a brain infection referred to as PML.

Following Rituximab, there is an increased risk of more common infections besides the rare PML described above. These include urinary tract infections, sinus infections or pneumonia for example. Some patients also experience drops in their blood counts that typically are not a problem but need to be followed.

Upcoming Events

2018 MGANE Spring meetings (held at Newton Wellesley Hospital, Allen Riddle Bldg.)

March 4th Nutrition with Liisa Jackson
May 6th Topic TBD

MGFA Annual Conference in Kansas City, MO: April 15-17, 2018 -- Check the MGFA web site as more information is being posted every day.

Why attend?

The MG National Conference is a must for anyone in the MG Community – with presentations and sessions on all aspects of MG from the medical to the personal and the social. MG experts, fellow patients and family members share their knowledge and experience.

MGFA Annual New England Walk: Saturday, May 5th at [Pope John Paul II Park, Boston, MA](#) Rain or shine we are committed!

JUNE is MG Awareness Month: Let's make our voices heard...TV, Radio, Physicians, friends and neighbors

SUNDAY, NOVEMBER 4: Is our annual meeting at the Crown Plaza (The VERVE) in Natick, Massachusetts. **Save the date: Keynote speaker will be Nancy Law, CEO of MGFA**