

**MG  
QUARTERLY**



## **RESEARCH**

### **Ra Pharmaceuticals Announces Positive Top-Line Results from Phase 2 Clinical Trial for generalized MG**

[Click here for full press release](#)

MGFA is excited to share that Ra Pharmaceuticals has announced positive top-line results from the Phase 2 clinical trial of their drug, Zilucoplan (formerly called RA101495) in treating myasthenia gravis, for people with AChR+ antibodies. The company also announced plans to engage with regulatory agencies, including the U.S. FDA, regarding the design of a Phase 3 trial in early 2019 for people with generalized MG.

“Zilucoplan has the potential to become the first convenient, self-administered, complement inhibitor expanding access for patients living with this chronic, debilitating, neuromuscular disease” “said James F. Howard, MD, Distinguished Professor of Neuromuscular Disease and Chief of the Neuromuscular Disorders Section, Department of Neurology, University of North Carolina School of Medicine.

“This represents a potential breakthrough for all patients who are struggling every day with their MG, and seeking more effective and convenient treatment options,” said Nancy Law, Chief Executive Officer of the MGFA. “This unmet need was highlighted in a recent survey of patients from the MGFA database, where we learned that a majority of patients with MG are not satisfied with their current treatments and are interested in effective, at home, self---injectable treatment options.”

### **Catalyst Pharmaceuticals Announces Approval of Firdapse for Treatment of LEMS**

[Click here for full press release](#)

The U.S. Food and Drug Administration (FDA) has approved FIRDAPSE<sup>®</sup> (amifampridine) 10mg tablets for the treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults 18 years of age or older. LEMS is a rare, severe neuromuscular disease that causes debilitating, progressive muscle weakness and fatigue.

Catalyst will work to ensure access to FIRDAPSE<sup>®</sup> for those diagnosed with LEMS with as little out-of-pocket burden as possible. The Catalyst Pathways program can support patients in many ways along their treatment journey, including treatment support, understanding insurance coverage, and identifying potential financial assistance. Patients and families can learn more by visiting [YourCatalystPathways.com](http://YourCatalystPathways.com) or by calling 1-833-4-Catalyst (1-833-422-8259).

MGFA is collaborating with the LEMS community to ensure that patients continue to receive access to cost-free, or low-cost, treatment options.

## Be Part of the MG Patient Registry



[Click here for more information](#)

As the MGFA community continues to grow, our organization asks each one of you that resides in the U.S., that is 18 or older, to think about joining the MG Patient Registry. The registry has already made a huge difference for so many people with MG. The information you provide allows researchers to start and continue clinical trials, which lead to increased treatment options. The Myasthenia Gravis Patient Registry is an active database of individuals with myasthenia gravis (MG), developed for the purposes of research, treatment, and patient information. The registry is free to join and all data is protected by federal privacy laws.

# ADVOCACY

## Join MGFA at Rare Disease Week!

MGFA will be at the 2019 Rare Disease Week, taking place from February 24 to 28, in Washington, D.C. While MGFA continues to advocate on behalf of all of those with MG, we know that there is strength in numbers. That's why we want YOU to join us! Raise your voice on Capitol Hill and help us make a difference for those with rare diseases.

The [EveryLife Foundation](#) has generously offered a travel stipend to help you bring your message to D.C. (without breaking the bank!) [Apply today](#) for the travel stipend, which has a deadline of **December 15<sup>th</sup>**! Let Congress know we matter.

Rare Disease Week on Capitol Hill brings the rare disease community and their caregivers together to advocate on federal healthcare issues. This is a great opportunity to meet other advocates, share your unique stories, and urge the importance of passing key healthcare legislation.

For any questions, contact Lisa Douthit at [lisa@LisaDouthit.com](mailto:lisa@LisaDouthit.com). We hope to see you there!

## **MGFA Represented by Board Member, Dr. Hafer-Macko, at 17<sup>th</sup> Annual Neurology on the Hill!**

Dr. Charlene Hafer-Macko will represent MGFA in the [17<sup>th</sup> Annual Neurology on the Hill Summit](#) in February 2019. Her work around increasing access to critical treatments for MG patients has helped moved MGFA advocacy efforts forward. During the Neurology on the Hill summit, Dr. Hafer-Macko will meet with senators and congressional representatives in conjunction with advocates from the rare disease community. Dr. Hafer-Macko serves as a member of MGFA's Board of Directors, Medical and Scientific Advisory Board as well as the Interim Chair for our Advocacy Committee.

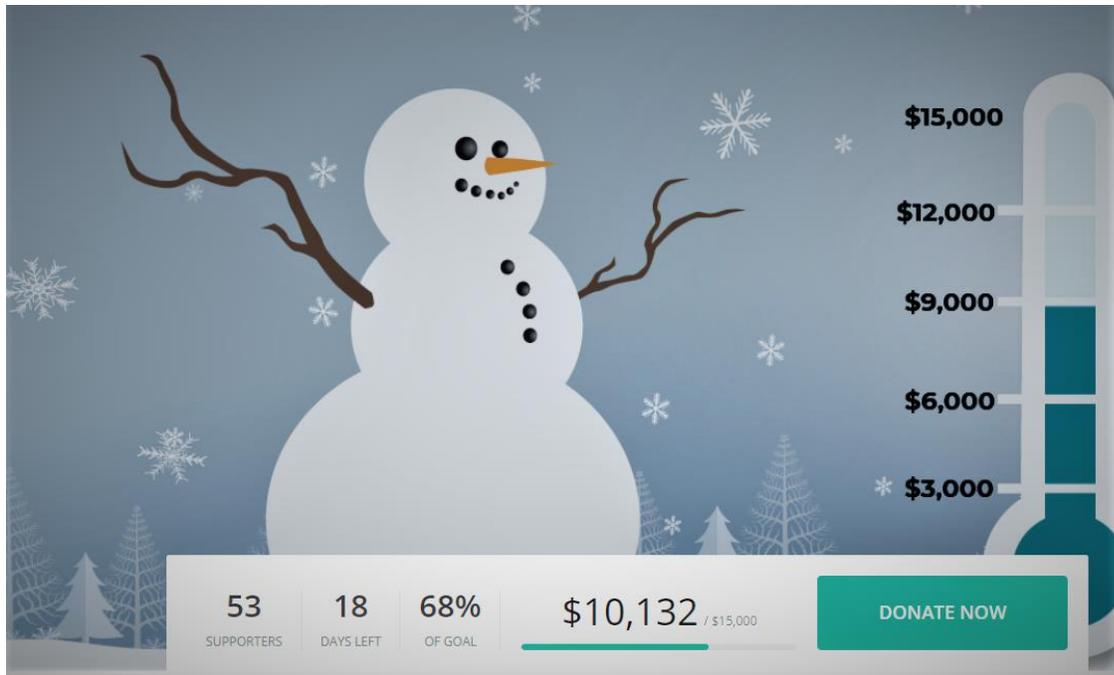


## **AWARENESS**

### **#GivingTuesday Campaign Creates Blizzard of Support with “Do You Want to Build a Snowman?”**

On Giving Tuesday, November 27, 2018, MGFA embarked on an ambitious awareness and fundraising campaign, titled “Do You Want to Build a Snowman?” This unique fundraising campaign was designed to honor someone you know affected by MG, whether it’s a person living with MG, a family member or a caregiver. As donations came in, our snowman came to life! Even better, our CEO, Nancy Law, matched donations up to 5K on Giving Tuesday!

There is still time to be part of our Blizzard of Support, which runs until December 31<sup>st</sup>. We’re at 68% of our goal and would love your help to get us to 100%! Please donate or spread the word on social media: [www.classy.org/campaign/do-you-want-to-build-a-snowman/c212079](http://www.classy.org/campaign/do-you-want-to-build-a-snowman/c212079)



## Help Raise Critical Awareness About MG Emergency Management



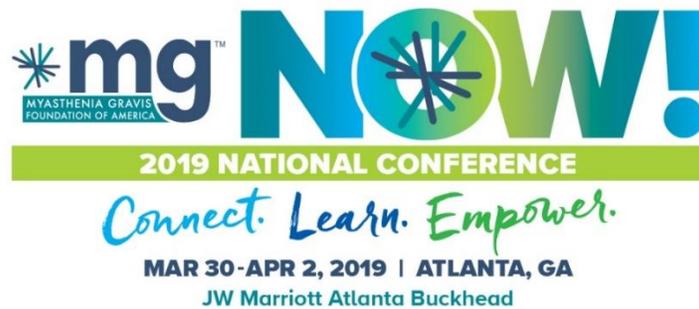
MGFA has produced an educational training video for first responders and emergency health providers. MG may be easily mistaken for other neuromuscular diseases or the effects of substance abuse among other diagnoses. A myasthenic crisis- a life-threatening situation for someone with MG- can go unrecognized and possibly exacerbated by a medical professional unfamiliar with MG. This presentation helps educate nurses, first responders and emergency room personnel about how to identify a myasthenic crisis and what to do.

Help spread this important information by contacting the hospital administration office of your local Emergency Room. Offer a meeting to show this video to first responders, nurses and emergency room personnel. Nurses will also earn CNE credits after completing this training.

The training is available [here](#).

# MGFA COMMUNITY NEWS

**SAVE THE DATE!**  
**2019 National Conference in Atlanta**



The **2019 National Conference** and **National MG Walk** will be held at the JW Marriott in Atlanta, Georgia from **March 30 through April 2**. Registration coming soon, please stay tuned via our [website](#)!

# Stay Tuned For 2019 Webinars!

## Webinar on Seronegative MG

Our upcoming webinar on seronegative MG teams up both medical professional and patient perspectives on seronegative MG. This webinar will be presented by Dr. Charlene Hafer-Macko and Cheryl Heitman, RN.

Charlene Hafer-Macko, M.D. is the Associate Professor of Neurology, clinician and researcher affiliated with the University of Maryland School of Medicine, Department of Neurology and the Baltimore Veterans Administration Medical Center. She holds a MS in Biomedical Engineering from Case Western University, MD from Ohio State University, School of Medicine, Neurology Residency at University of California Los Angeles (UCLA), and Neuromuscular Disease Fellowships at UCLA and Johns Hopkins University. She has an active myasthenia gravis practice at both the University of Maryland and Baltimore VA. Her research interest focuses on promotion health and wellness for individuals with MG, other neurologic diseases and aging. Charlene is a member of MGFA's Medical / Scientific Advisory Board and Board of Directors. Charlene serves as Chair to the MGFA Advocacy Committee and will represent MGFA in the 17<sup>th</sup> Annual Neurology on the Hill Summit in February 2019. She is also an active member of the Mid-Atlantic MG support groups.



Cheryl Heitman has been an RN for 30 years and was diagnosed with MG in 2015 after having symptoms for over 15 years. She graduated Magna Cum Laude from the University of Missouri, Columbia School of Nursing in 1987. Her work included many areas of nursing including OB, geriatrics, staff development, medical surgical, and most recently, 20 years in cardiopulmonary rehabilitation and diagnostics. She enjoyed opportunities for patient and family education. Cheryl has served on numerous committees and councils, including hospital-wide nurse practice council and as secretary for the Missouri Kansas Association of Cardiovascular and Pulmonary Rehabilitation. Cheryl enjoys spending time with her husband, her two grown children and two grandsons. She is also an advocate for myasthenia gravis awareness, especially for those with seronegative myasthenia gravis. She promotes use of the MG Patient Registry and looks forward to new advances in the care of MG patients. Both Charlene and Cheryl team up during our National MG Walk as part of Team Duck Tapers, one of our top fundraising teams of all time!

## Webinar on Respiratory Issues & MG



Our upcoming webinar on respiratory issues and MG will be presented by Dr. Nicholas Silvestri.

Nicholas J. Silvestri, M.D. is the Clinical Associate Professor of Neurology and Assistant Dean for Graduate Medical Education at the Jacobs School of Medicine & Biomedical Sciences, University at Buffalo. He currently practices at Jacobs Neurological Institute and is affiliated with Kaleida Health Buffalo General Hospital. Dr. Silvestri accepts multiple insurance plans including Aetna, MVP Health Plan and Empire Blue Cross Blue Shield. Dr. Silvestri also practices at UBMD Neurology, Buffalo General Medical Center (BGMC) and at the Muscular Dystrophy Association clinic at BGMC, where he serves as co-director.

Dr. Silvestri has specialized training in clinical neuromuscular medicine, with expertise in clinical care and in performing electrodiagnostic studies (nerve conduction and electromyography). Dr. Silvestri conducts both clinical research studies and basic science research in collaboration with physician-scientists from multiple disciplines, including neurology. His clinical research is focused on several studies that address illnesses such as Guillain-Barre syndrome, myasthenia gravis and polyneuropathy. These studies include both treatment trials--to find more effective treatments for these diseases--as well as research investigating the impact of the diseases on patients' quality of life.

Stay tuned for more details about our [webinars](#) coming in 2019!