

Myasthenia Gravis Support Group of Central Texas

March 11, 2019

Linda Ann Joslin Facilitator

Lee Higgins MGFA Rep

Susan Larkin Treasurer

Rachel Stewart MGFA Rep

Disclaimer: The following MG Support, Central Texas notes are to provide the reader with general information to be used solely for educational purposes. As such, it does not address individual patient needs, and should not be used as a basis for decision making concerning diagnosis, care, or treatment of any condition. Instead, such decisions should be based upon the advice of a physician or health care professional that is directly familiar with the patient. Each member has different ideas, opinions and manners in which they deal with MG. **This group was established to help people with Myasthenia Gravis.**

Members in Attendance:

Linda Ann Joslin David Renfro

Rachel Higgins

Sandra Johnston

Joyce Brown

Jonathan Simmons

Don Jones

Richard Armor

Dan Bealko

Joni Kendrick

Kevin Kendrick

Daniel Bealko

Anita Isaac

Marcy Baird

Toby Kinslow (new)

Scott Curtis (new)

Our 2019 Schedule: Spicewood Springs Library Branch 8637 Spicewood Springs Rd. 78759

Monday	April 15, 2019 at 7:00 pm to 8:00	Dr. Husain	Topic: New Trials in the area
Wednesday	May 15, 2019 at 7:00 pm to 8:00		
Wednesday	June 12, 2019 at 7:00 pm to 8:00		
Wednesday	July 10, 2019 at 7:00 pm to 8:00		
Wednesday	August 21, 2019 at 7:00 pm to 8:00		
Wednesday	September 11, 2019 at 7:00 pm to 8:00		
Wednesday	October 9, 2019 at 7:00 pm to 8:00		
Thursday	November 14, 2019 at 7:00 pm to 8:00		
Thursday	December 12, 2019 at 7:00 pm to 8:00		

DR. ADAM HORVIT

Dr. Horvit visited our group and told us about the new approved drug for MG, Soliris.

Guys, this sounds boring, but every time Dr. Horvit visits, we have a blast.

Be sure and come to his next visit.

FDA Approves Soliris® (Eculizumab) Treatment of Patients with Generalized Myasthenia Gravis (gMG)

First FDA-Approved Treatment in More Than 60 Years for Patients with gMG, a Chronic and Debilitating Neuromuscular Disorder

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"I am pleased that the FDA recognized the comprehensive clinical data supporting the benefits of Soliris for patients with anti-AchR antibody-positive gMG"

NEW HAVEN, Conn.--([BUSINESS WIRE](#))--Alexion Pharmaceuticals, Inc. (NASDAQ:ALXN) announced today that the U.S. Food and Drug Administration (FDA) has approved Soliris® (eculizumab) as a treatment for adult patients with generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AchR) antibody-positive. In the Phase 3 REGAIN study and its ongoing open-label extension study, Soliris demonstrated treatment benefits for patients with anti-AchR antibody-positive gMG who had previously failed immunosuppressive treatment and continued to suffer from significant unresolved disease symptoms, which can include difficulties seeing, walking, talking, swallowing and breathing. These patients are at an increased risk of disease exacerbations and crises that may require hospitalization and intensive care and may be life-threatening. These patients represent approximately 5-10% of all patients with MG.¹⁻⁶

“Today’s approval is a significant milestone for Alexion and, more importantly, for the subset of patients with anti-AchR antibody-positive gMG who continue to suffer from significant unresolved disease symptoms despite existing treatment options,” said John Orloff, M.D., Executive Vice President and Head of Research & Development at Alexion. “We are proud that we could apply our deep understanding of complement biology to develop Soliris for the treatment of patients with this debilitating neuromuscular disorder.”

Chronic uncontrolled activation of the complement system, a part of the immune system, plays a major role in the debilitating symptoms and potentially life-threatening complications for patients with gMG who are anti-AchR antibody-positive.¹⁻⁴ By selectively and effectively inhibiting the terminal complement cascade, Soliris targets a critical underlying cause of the disease.

“I am pleased that the FDA recognized the comprehensive clinical data supporting the benefits of Soliris for patients with anti-AchR antibody-positive gMG,” said Professor James F. Howard, M.D., Department of Neurology at the University of North Carolina, Chapel Hill, and lead investigator in the clinical development of this new indication. “It is exciting that patients who have not responded adequately to existing therapies will now have a new treatment option that was shown in clinical studies to improve patients’ symptoms, their ability to carry out activities of daily living and their quality of life.”

“This is a landmark day for the members of the U.S. myasthenia gravis community, who have not seen a therapy approved for generalized myasthenia gravis in more than 60 years,” said Nancy Law, Chief Executive Officer of the Myasthenia Gravis Foundation of America (MGFA). “It is particularly significant that this approval of Soliris will provide a new option for those with gMG and especially for those who do not respond adequately to or cannot tolerate standard treatment options.”

The FDA based its approval of the extended indication for Soliris on comprehensive clinical data from the Phase 3, randomized, double-blind, placebo-controlled, multicenter REGAIN study (ECU-MG-301).

Soliris is approved in the European Union (EU) for the treatment of refractory gMG in adults who are anti-AchR antibody-positive. Alexion’s new drug application in Japan for Soliris as a treatment for patients with anti-AchR antibody-positive refractory gMG has been accepted for review by the Japanese Ministry of Health, Labour and Welfare (MHLW). Soliris has received Orphan Drug Designation (ODD) for the treatment of patients with MG in the U.S. and EU, and for the treatment of patients with refractory gMG in Japan.

About Generalized Myasthenia Gravis

Myasthenia gravis (MG) is a debilitating, chronic and progressive autoimmune neuromuscular disease that can occur at any age but most commonly begins for women before the age of 40 and men after the age of 60.⁷⁻¹⁰ It typically begins with weakness in the muscles that control the movements of the eyes and eyelids, and often progresses to the more severe and generalized form, known as gMG, with weakness of the head, neck, trunk, limb and respiratory muscles.¹⁰

While most patients with gMG can be managed with current therapies for MG, 10-15% of patients fail to respond adequately to or cannot tolerate multiple therapies for MG and continue to suffer profound muscle weakness, and severe disease symptoms that limit function.^{1,11,12} These patients can suffer from slurred speech, choking, impaired swallowing, double or blurred vision, disabling fatigue, immobility requiring assistance, shortness of breath, and episodes of respiratory failure. Complications, exacerbations and myasthenic crises can require hospital and intensive care unit admissions with prolonged stays and can be life-threatening.^{8,9,13}

In patients with anti-AchR antibody-positive MG, the body’s own immune system turns on itself to produce antibodies against AchR, a receptor located on muscle cells at the neuromuscular junction (NMJ) and used by nerve cells to communicate with the muscles these nerves control.^{8,9} The binding of these antibodies to AchR activates the complement cascade, another part of the immune system, which leads to a localized inflammation

and destruction of the muscle membrane at the NMJ.¹⁴⁻¹⁶ As a result, the communication between nerve and muscle is impaired, which in turn leads to a loss of normal muscle function.^{8,9}

Patients with anti-AchR antibody-positive gMG who continue to suffer from severe disease symptoms and complications despite current therapies for MG represent approximately 5-10% of all patients with MG.⁷⁻¹⁰

About Soliris® (eculizumab)

Soliris® is a first-in-class complement inhibitor that works by inhibiting the terminal part of the complement cascade, a part of the immune system that, when activated in an uncontrolled manner, plays a role in serious ultra-rare disorders like paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS) and anti-acetylcholine receptor (AChR) antibody-positive myasthenia gravis (MG).

Soliris is approved in the U.S., EU, Japan and other countries as the first and only treatment for patients with PNH and aHUS, in the EU as the first and only treatment of refractory gMG in adults who are anti-AchR antibody-positive, and in the U.S. for the treatment of adult patients with gMG who are anti-AchR antibody-positive. Soliris is not indicated for the treatment of patients with Shiga-toxin E. coli-related hemolytic uremic syndrome (STEC-HUS). Alexion and Soliris have received some of the pharmaceutical industry's highest honors for the medical innovation in complement inhibition: the Prix Galien USA (2008, Best Biotechnology Product) and France (2009, Rare Disease Treatment).

For more information on Soliris, please see full prescribing information for Soliris, including BOXED WARNING regarding risk of serious meningococcal infection, available at www.soliris.net.

Important Soliris Safety Information

The U.S. prescribing information for Soliris includes the following warnings and precautions: Life-threatening and fatal meningococcal infections have occurred in patients treated with Soliris. Meningococcal infection may become rapidly life-threatening or fatal if not recognized and treated early. Comply with the most current Centers for Disease Control (CDC)'s Advisory Committee on Immunization Practices (ACIP) recommendations for meningococcal vaccination in patients with complement deficiencies. Immunize patients with meningococcal vaccines at least two weeks prior to administering the first dose of Soliris, unless the risks of delaying Soliris therapy outweigh the risk of developing a meningococcal infection. Monitor patients for early signs of meningococcal infections and evaluate immediately if infection is suspected. Soliris is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS). Under the Soliris REMS, prescribers must enroll in the program. Enrollment in the Soliris REMS program and additional information are available by telephone: 1-888-SOLIRIS (1-888-765-4747) or at www.solirisrems.com.

Patients may have increased susceptibility to infections, especially with encapsulated bacteria. Aspergillus infections have occurred in immunocompromised and neutropenic patients. Children treated with Soliris may be at increased risk of developing serious infections due to *Streptococcus pneumoniae* and *Haemophilus influenzae* type b (Hib). Soliris treatment of patients with PNH should not alter anticoagulant management because the effect of withdrawal of anticoagulant therapy during Soliris treatment has not been established. Administration of Soliris may result in infusion reactions, including anaphylaxis or other hypersensitivity reactions.

In patients with PNH, the most frequently reported adverse events observed with Soliris treatment in clinical studies were headache, nasopharyngitis, back pain and nausea. In patients with aHUS, the most frequently reported adverse events observed with Soliris treatment in clinical studies were headache, diarrhea, hypertension, upper respiratory infection, abdominal pain, vomiting, nasopharyngitis, anemia, cough, peripheral edema, nausea, urinary tract infections, and pyrexia. In patients with gMG who are anti-AchR antibody-positive, the most frequently reported adverse reaction observed with Soliris treatment in the placebo-controlled clinical study ($\geq 10\%$) was musculoskeletal pain.

About Alexion

Alexion is a global biopharmaceutical company focused on serving patients and families affected by rare diseases through the innovation, development and commercialization of life-changing therapies. Alexion is the global leader in complement inhibition and has developed and commercializes the first and only approved complement inhibitor to treat patients with paroxysmal nocturnal hemoglobinuria (PNH), atypical hemolytic uremic syndrome (aHUS), and anti-acetylcholine receptor (AChR) antibody-positive generalized myasthenia gravis (gMG). In addition, Alexion has two highly innovative enzyme replacement therapies for patients with life-threatening and ultra-rare metabolic disorders, hypophosphatasia (HPP) and lysosomal acid lipase deficiency (LAL-D). As the leader in complement biology for over 20 years, Alexion focuses its research efforts on novel molecules and targets in the complement cascade, and its development efforts on the core therapeutic areas of hematology, nephrology, neurology, and metabolic disorders. This press release and further information about Alexion can be found at: www.alexion.com.

[ALXN-G]

Forward-Looking Statement

This news release contains forward-looking statements, including statements related to the potential medical benefits of Soliris[®] (eculizumab) for the treatment of generalized myasthenia gravis (gMG), and Alexion's future clinical, regulatory and commercial plans for Soliris for the treatment of myasthenia gravis. Forward-looking statements are subject to factors that may cause Alexion's results and plans to differ from those expected, including for example, the risks and uncertainties of drug development, decisions of regulatory authorities regarding the adequacy of our research, marketing approval or material limitations on the marketing of eculizumab for the treatment of gMG, delays, interruptions or failures in the manufacture and supply of our products and our product candidates, failure to satisfactorily address matters raised by the FDA and other regulatory agencies, the possibility that results of clinical trials are not predictive of safety and efficacy results of our products in broader patient populations, the possibility that clinical trials of our product candidates could be delayed, the adequacy of our pharmacovigilance and drug safety reporting processes, the risk that third party payers (including governmental agencies) will not reimburse or continue to reimburse for the use of our products at acceptable rates or at all, the outcome of challenges and opposition proceedings to our intellectual property, assertion or potential assertion by third parties that the manufacture, use or sale of our products infringes their intellectual property, uncertainties surrounding legal proceedings, company investigations and government investigations, including investigations of Alexion by the SEC and DOJ, the risk that anticipated regulatory filings are delayed, the risk that estimates regarding the number of patients with gMG are inaccurate, and a variety of other risks set forth from time to time in Alexion's filings with the U.S. Securities and Exchange Commission, including but not limited to the risks discussed in Alexion's Quarterly Report on Form 10-Q for the period ended June 30, 2017 and in our other filings with the U.S. Securities and Exchange Commission. Alexion does not intend to update any of these forward-looking statements to reflect events or circumstances after the date hereof, except when a duty arises under law.

2019 MGFA National Conference

March 31 - April 2, 2019
JW Marriott Buckhead
3300 Lenox Road, NE, Atlanta, GA 30326
+1 (404) 262-3344

[CLICK HERE TO REGISTER](#)

For more information about the hotel, please visit the Hotel & Travel page. Please note that the **MGFA room block cutoff date is Friday, March 8.**

The National Conference is the largest gathering of the myasthenia gravis (MG) community in the U.S. The conference is a place for people to connect, learn and share their personal and professional experience with MG. We welcome people with MG, their caregivers and health professionals to attend.

Why attend?



The National Conference offers a wealth of information for everyone in the MG community, whether newly diagnosed or having lived with MG for years. Presentations and breakout sessions cover all aspects of MG, from medical to personal to social. MG experts, MG community leaders, people with MG and family members come together to share their experience. Join us to gain knowledge, share resources and speak

with others "who understand!"

Join the National MG Walk!

Kick off the conference with the National MG Walk on March 30th! This is a great chance for those who may not have a Walk in their own community to form or join a team. Team Duck Tapers, comprised of Facebook friends from around the country, was the top team at our New Orleans Walk in 2017, and again in Kansas City for the 2018 walk, and they are looking for a three-peat! So, plan to come for both the MG Walk and the National Conference. Start now to form your own MG Walk team and give the Duck Tapers a 'walk' for their money!



Support Groups in Texas ----- LET'S GO TEXAS!!!!!!!!!!!!!!

Central Texas MG Support Group meets at the Spicewood Springs Library 8637 Spicewood Springs Rd Austin 78759
Linda Ann & Larry Joslin, Facilitators Started in February 2007 www.mg-centraltexas.org

Alamo MG Support Group meets in San Antonio on the 2nd Health Link Fitness Center, 288 W. Bitters Rd San Antonio 78216
Elroy and Gail Tschirhart, Facilitators Started in February 2007 www.mgsouthtexas.org

Houston MG Support meets in Houston every 2nd Saturday. Trini Mendenhall Community Center, 1414 Wirt Rd. Houston 77055
Meena Outlaw or Sarah Ricks, Facilitators Started January 2017 <https://mghoustontx.org/>

Northwest TX/DFW Support meets in Dallas Every 2nd Sat contact Facilitator to confirm location
Karon & Jerry Faught, Facilitator Facebook: [DFW Myasthenia Gravis Support Group](https://www.facebook.com/DFW-Myasthenia-Gravis-Support-Group)

Southeast Texas MG Support (also servicing Southwest Louisiana) 2nd Thurs in Beaumont - Howell's Furniture Community Rm
Tracey Young, Facilitator Started November Facebook Page

Corpus Christi Texas MG Support Meets 3rd Saturday confirm location Robert Harvey, Facilitator Started January 2017
https://www.facebook.com/Myasthenia-Gravis-Support-Group-of-Corpus-Christi-Texas-630868910390981/?ref=page_internal&qsefr=1

Deep South Texas MG Support Harlingen Karen Mau "Karen Mau" <maubillkaren@live.com>
San Angelo Texas MG Support (<https://www.facebook.com/sanangelomg/>) or contact Robert Rumph at rrumph@me.com.

Linda Ann Joslin,
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www.mg-centraltexas.org