Myasthenia Gravis Support Group of Central Texas

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Linda Ann Joslin	Facilitator	Lee Higgins	MGFA Rep	Susan Larkin	Treasurer
Karen Davis	Web Mgr	Rachel Higgins	MGFA Rep	Keith Pflieger	Secretary

Members in Attendance:

Linda Ann Joslin	Larry Joslin	David Renfro	Rachel Higgins	Sallie Sassoon	Joni Kendrick	Kevin Kendrick
Ron Estes	Pauline Estes	Dan Bealko	Sandra Johnston	Richard Armor	Dan McSpadden	Jackie McSpadden
Atis Jurbergs	Joyce Brown	Jonathan Simmons				

Dr. Horvit's Visit

FDA Grants Orphan Drug Designation to Soliris® (eculizumab) for the Treatment of Patients with Myasthenia Gravis (MG)

"By specifically inhibiting the terminal complement pathway, which is believed to play a pivotal role in the pathophysiology of MG, we believe that eculizumab has the potential to help patients living with this devastating rare disorder."

CHESHIRE, Conn.--(BUSINESS WIRE)--Alexion Pharmaceuticals (Nasdaq:ALXN) today announced that the U.S. Food and Drug Administration (FDA) has granted orphan drug designation (ODD) to Soliris® (eculizumab) for the treatment of patients with Myasthenia Gravis (MG), a rare, debilitating neurologic disorder caused by uncontrolled complement activation. In patients with MG, uncontrolled complement activation due to antibodies directed at the neuromuscular junction can ultimately lead to profound and debilitating weakness of various muscle groups throughout the body.

"Patients with MG develop debilitating muscle weakness, impairing their ability to walk, speak clearly, swallow and, in some cases, to breathe normally, which could lead to a life-threatening myasthenic crisis," said Martin Mackay, Ph.D., Executive Vice President, Global Head of R&D at Alexion. "By specifically inhibiting the terminal complement pathway, which is believed to play a pivotal role in the pathophysiology of MG, we believe that eculizumab has the potential to help patients living with this devastating rare disorder."

Soliris is a first-in-class terminal complement inhibitor and is currently approved for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH) and atypical hemolytic uremic syndrome (aHUS), two debilitating, ultra-rare and life-threatening disorders caused by chronic uncontrolled complement activation. Soliris is not approved in any country to treat MG. Alexion is enrolling patients in a multinational, placebo-controlled registration trial of eculizumab in patients with refractory generalized MG. More information on this trial is available at www.clinicaltrials.gov under the identifier NCT01997229.

The FDA, through its Office of Orphan Products Development (OOPD), grants orphan status to drugs and biologic products that are intended for the safe and effective treatment, diagnosis, or prevention of rare diseases or disorders that affect fewer than 200,000 people in the U.S. Orphan drug designation provides a drug developer with certain benefits and incentives, including a period of marketing exclusivity if regulatory approval is ultimately received for the designated indication.

About Myasthenia Gravis (MG)

Myasthenia gravis is a rare, debilitating neurologic disorder caused by auto-antibodies that recognize a specific target in the nerve-muscle junction, which results in life-long uncontrolled terminal complement activation causing tissue damage and interference with signalling between nerve and muscle fibers.[1,2] Patients with MG initially experience weakness in their ocular (eye) muscles, and the disease typically progresses to the more severe and generalized form to include weakness of head, trunk, limb and respiratory muscles. Symptoms can include drooping eyelid, weakness in the arms and legs, slurred speech, difficulty chewing or swallowing, and difficulty breathing, which could lead to a life-threatening myasthenic crisis.

About Soliris

Soliris is a first-in-class terminal complement inhibitor developed from the laboratory through regulatory approval and commercialization by Alexion. Soliris is approved in the U.S. (2007), European Union (2007), Japan (2010) and other countries as the first and only treatment for patients with paroxysmal nocturnal hemoglobinuria (PNH), a debilitating, ultra-rare and life-threatening blood disorder, characterized by complement-mediated hemolysis (destruction of red blood cells). Soliris is indicated to reduce hemolysis. Soliris is also approved in the U.S. (2011), the European Union (2011), Japan

(2013) and other countries as the first and only treatment for patients with atypical hemolytic uremic syndrome (aHUS), a debilitating, ultra-rare and life-threatening genetic disorder characterized by complement-mediated thrombotic microangiopathy, or TMA (blood clots in small vessels). Soliris is indicated to inhibit complement-mediated TMA. Soliris is not indicated for the treatment of patients with Shiga-toxin *E. coli*-related hemolytic uremic syndrome (STEC-HUS). For the breakthrough innovation in complement inhibition, Alexion and Soliris have received the pharmaceutical industry's highest honors: the 2008 Prix Galien USA Award for Best Biotechnology Product with broad implications for future biomedical research and the 2009 Prix Galien France Award in the category of Drugs for Rare Diseases.

More information including the full U.S. prescribing information on Soliris is available at www.soliris.net.

Important Safety Information

The U.S. product label for Soliris includes a boxed warning: "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 [see Warnings and Precautions (5.1)]. Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for meningococcal vaccination in patients with complement deficiencies. Immunize patients with a meningococcal vaccine 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. [See Warnings and Precautions (5.1) for additional guidance on the management of the risk of 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 [see Warnings and Precautions (5.2)]. Enrollment in the Soliris REMS program and additional information are available by telephone: 1-888-SOLIRIS (1-888-765-4747)."

In patients with PNH, the most frequently reported adverse events observed with Soliris treatment in clinical studies were headache, nasopharyngitis (runny nose), back pain and nausea. 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. 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, pyrexia. Soliris is not indicated for the treatment of patients with Shiga toxin *E. coli* related hemolytic uremic syndrome (STEC-HUS). Please see full prescribing information for Soliris, including BOXED WARNING regarding risk of serious meningococcal infection.
http://soliris.net/sites/default/files/assets/soliris_pi.pdf

What is Soliris? 

Soliris (eculizumab) is a monoclonal antibody. Eculizumab binds to proteins in the blood that can destroy red blood cells in people with genetic conditions that affect the natural defenses of red blood cells.

Soliris is used to prevent the breakdown of red blood cells in people with paroxysmal nocturnal hemoglobinuria (PNH).

Soliris is also used to treat a rare chronic blood disease called atypical hemolytic uremic syndrome (aHUS)

Soliris is also used to treat myasthenia gravis in adults.

Soliris is available only under a special program. You must be registered in the program and understand the risks and benefits of this medicine.

Important Information

You should not use Soliris if you have bacterial meningitis or if you have not been vaccinated against meningitis.

You must be vaccinated against meningococcal infection at least 2 weeks before treatment with Soliris. If you have been vaccinated in the past, you may need a booster dose.

Seek emergency medical attention or call your doctor right away if you have **symptoms of meningitis**: headache and fever with nausea or vomiting, skin rash, body aches, flu symptoms, confusion, sensitivity to light, stiffness in your neck or back.

You will receive a card listing the symptoms of meningococcal infection. Read this information and learn what symptoms to watch for. Keep the card with you at all times while using Soliris and for at least 3 months after your last dose.

Do not stop receiving Soliris without first talking to your doctor. Stopping or interrupting your treatment could cause sudden and serious effects on your red blood cells.

Before taking this medicine

You should not use Soliris if you are allergic to eculizumab, or if:

- you have bacterial meningitis; or
- you have not been vaccinated against meningitis.

Tell your doctor if you have a fever or any type of infection.

You must be vaccinated against meningococcal infection at least 2 weeks before you start treatment with Soliris. If you were vaccinated in the past, you may need a booster dose.

Your doctor may also recommend other vaccines while you are using Soliris.

Follow your doctor's instructions about using Soliris **if you are pregnant.** It is very important to control your blood disorder during pregnancy to avoid unwanted effects in you or the baby.

It may not be safe to breast-feed a baby while you are using this medicine. Ask your doctor about any risks.

6 Diet Tips for Myasthenia Gravis Patients (***Activebeat***)

By: Jeff Hayward on Tuesday, June 6th

1. Make it Easier to Swallow

Myastheniagravis.org (Conquer MG) recognizes that some solid foods can be difficult to get down, and notes you can use gravy, sauce, butter, mayo or yogurt to help them slide down your throat more easily with fewer residues.

2. Boost Potassium

DrWeil.com notes that potassium, along with rest, can be a useful tool to help you maintain muscle strength (or at least minimize the symptoms). However, not every meal is packed with potassium, so you may need to make some dietary changes to ensure you're getting enough.

Foods naturally high in potassium include oranges, tomatoes, apricots (solids or as juices), bananas and broccoli, all of which contain other essential vitamins and nutrients your body needs to function.

Meanwhile, you can choose foods that are fairly easy to chew and swallow to begin with, while also offering some nutritional value – such as egg salad, canned salmon, and even fruit smoothies. It suggests looking for "soft diet" recipe books from your local bookstore for more ideas.

3. Douse Inflammation

Certain foods can aggravate inflammation, which is closely related to autoimmune disease flare-ups. The list of foods that can cause inflammation is quite extensive, and may take some getting used to, but might be worth it in the end.

For example, animal proteins and foods with trans fats are to be avoided (try getting protein from plant sources like beans and nuts), according to Livestrong.com. You should also cut out polyunsaturated oils including corn and sunflower oil, and try not to consume items high in “table sugar” such as cookies and candy.

4. Reduce Meal Sizes

Instead of following the “three square meals per day” rule for the majority of people, it’s wiser for those with myasthenia gravis to follow a schedule of eating smaller meals throughout the day (with the largest meal early in the day when you have the most energy), according to Myastheniagravis.org.

If you’re choosing to eat meat or other solid foods and don’t fancy using sauces to help them go down easier, then just cut them into much smaller pieces or mince the meal, it adds.

5. Avoid Aspiration

Another problem for those with the disease is thinner liquids ending up in the lungs rather than the stomach, which is known as aspiration. The same source notes that some foods actually need to be thickened to help avoid this problem.

“Remember that ice cream and popsicles melt into a thin fluid in your mouth, and that once you chew fruits, the juice released is also a thin liquid,” notes Conquer MG. Be more way when eating foods that have a “mixed consistency,” such as cereal with milk, it adds.

6. Consume Vitamin D and Calcium

Sources notes that steroid drugs (prednisone in particular) used to help treat autoimmune diseases such as myasthenia gravis can promote loss of bone or bone thinning, so you should consulting your doctor about Vitamin D and calcium supplements to offset this side effect.

Meanwhile, other drugs called acetyl cholinesterase inhibitors can cause diarrhea, and in this case you should avoid foods that are spicy or greasy, as well as cutting out caffeine and chocolate, according to LifeExtension.com.

Support Groups in Texas **------ LET’S GO TEXAS!!!!!!!!!!!!**

Central Texas MG Support Group meets every 2nd Wed. 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***

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 Planning

Linda Ann Joslin, Facilitator, MG Support Central Texas www.mg-centraltexas.org