Alexion aims to advance NMOSD treatment landscape with exceptional ULTOMIRIS® (ravulizumab-cwvz) efficacy data at ECTRIMS 2022

October 12, 2022

Results from CHAMPION-NMOSD trial demonstrated zero relapses with a median treatment duration of 73 weeks

Additional presentations will underscore debilitating emotional and physical toll of NMOSD on patients and caregivers, and highlight urgency to reduce disease burden

WILMINGTON, Del., October 12, 2022 – Alexion, AstraZeneca Rare Disease, will present new data showing significant advances for the treatment of anti-aquaporin-4 (AQP4) antibody-positive (Ab+) neuromyelitis optica spectrum disorder (NMOSD) at the European Committee for Treatment and Research in Multiple Sclerosis (ECTRIMS) Congress, October 26 to 28, 2022.

Data presented at the meeting will feature new insights from Alexion's complement portfolio illustrating the critical role of C5 inhibition in treating AQP4 Ab+ NMOSD and significantly reducing the risk of relapses, including findings on the first and only long-acting C5 complement inhibitor, ULTOMIRIS® (ravulizumab-cwvz).

Marc Dunoyer, Chief Executive Officer, Alexion, said: "NMOSD relapses are unpredictable and often cause permanent disability, including blindness, paralysis and even premature death. Our data build on the established role of complement inhibition in managing NMOSD and reinforce our commitment to patient-centered innovation. Results from the CHAMPION-NMOSD trial highlight the potential for ULTOMIRIS to substantially reduce the risk of relapse and ease treatment burden for the community."

Redefining NMOSD disease management

An oral presentation will showcase results of the CHAMPION-NMOSD trial, which evaluated the safety and efficacy of ULTOMIRIS in adults with AQP4 Ab+ NMOSD, compared to the external placebo arm from the pivotal SOLIRIS® PREVENT clinical trial.

The data will show there were zero adjudicated on-trial relapses observed in patients with AQP4 Ab+ NMOSD with a median treatment duration of 73 weeks, representing a relapse risk reduction of 98.6% ($p<0.0001$) compared to the external placebo arm. The safety and tolerability of ULTOMIRIS in the CHAMPION-NMOSD trial were consistent with previous clinical studies and other approved indications.

Improving understanding of the NMOSD patient experience

Two poster presentations detail findings from qualitative interviews with NMOSD patients. This work helps to inform the scientific community about the immediate and lasting impact of relapses, including ability to work and participate in daily activities, mobility and pain as well as reliance on caregiver support.

Alexion presentations during ECTRIMS 2022

Lead author	Abstract title	Presentation details
Pittock, SJ	Efficacy and safety of ravulizumab-cwvz in adults with anti-aquaporin-4 antibody-positive neuromyelitis optica spectrum disorder: outcomes from the phase 3 CHAMPION-NMOSD trial	Oral Presentation O051 October 27, 2022 10:05 CEST (4:05 EDT)
Pittock, SJ	Efficacy subgroup analyzes from the phase 3 CHAMPION-NMOSD trial in adults with anti-aquaporin-4 antibody-positive neuromyelitis optica spectrum disorder	Poster Presentation P010 October 26, 2022 16:30 CEST (10:30 EDT) ePoster Tour 5 October 28, 2022 14:00 CEST (8:00 EDT)
Allen, K	Sensitivity analysis using propensity score methods for primary efficacy outcome in the CHAMPION-NMOSD trial	Poster Presentation P012 October 26, 2022

		16:30 CEST (10:30 EDT)
Palace, J	Mortality estimates in patients with anti-aquaporin-4 autoantibody positive neuromyelitis optica spectrum disorder	Poster Presentation P018 October 26, 2022 16:30 CEST (10:30 EDT)
Kielhorn, A	Long-term burden of relapse in patients with AQP4+ NMOSD: simulation study based on network meta-analysis	Poster Presentation P019 October 26, 2022 16:30 CEST (10:30 EDT)
Osborne, B	Activity impairment and support needs in patients with neuromyelitis optica spectrum disorder	Poster Presentation P418 October 27, 2022 17:00 CEST (11:00 EDT)
Bernitsas, E	Understanding the symptoms of patients with neuromyelitis optica spectrum disorder and their impact on patient's lives: a qualitative interview study	ePoster Presentation EP0901 October 26, 2022 8:00 CEST (2:00 EDT)

INDICATION(S) & IMPORTANT SAFETY INFORMATION for ULTOMIRIS® (ravulizumab-cwvz)

What is ULTOMIRIS?

ULTOMIRIS is a prescription medicine used to treat:

- adults and children 1 month of age and older with a disease called Paroxysmal Nocturnal Hemoglobinuria (PNH).
- adults and children 1 month of age and older with a disease called atypical Hemolytic Uremic Syndrome (aHUS). ULTOMIRIS is not used in treating people with Shiga toxin E. coli related hemolytic uremic syndrome (STEC-HUS).
- adults with a disease called generalized myasthenia gravis (gMG) who are anti-acetylcholine receptor (AChR) antibody positive.

It is not known if ULTOMIRIS is safe and effective in children younger than 1 month of age.

It is not known if ULTOMIRIS is safe and effective for the treatment of gMG in children.

Subcutaneous administration of ULTOMIRIS has not been evaluated and is not approved for use in children.

IMPORTANT SAFETY INFORMATION

What is the most important information I should know about ULTOMIRIS?

ULTOMIRIS is a medicine that affects your immune system and can lower the ability of your immune system to fight infections.

- ULTOMIRIS increases your chance of getting serious and life-threatening meningococcal infections that may quickly become life-threatening and cause death if not recognized and treated early.

1. You must receive meningococcal vaccines at least 2 weeks before your first dose of ULTOMIRIS if you are not vaccinated.
2. If your doctor decided that urgent treatment with ULTOMIRIS is needed, you should receive meningococcal vaccination as soon as possible.
3. If you have not been vaccinated and ULTOMIRIS therapy must be initiated immediately, you should also receive 2 weeks of antibiotics with your vaccinations.
4. If you had a meningococcal vaccine in the past, you might need additional vaccination. Your doctor will decide if you need additional vaccination.
5. Meningococcal vaccines reduce but do not prevent all meningococcal infections. Call your doctor or get emergency medical care right away if you get any of these signs and symptoms of a meningococcal infection: headache with nausea or vomiting, headache and fever, headache with a stiff neck or stiff back, fever, fever and a rash, confusion, muscle aches with flu-like symptoms and eyes sensitive to light.

Your doctor will give you a Patient Safety Card about the risk of meningococcal infection. Carry it with you at all times during treatment and for 8 months after your last ULTOMIRIS dose. It is important to show this card to any doctor or nurse to help them diagnose and treat you quickly.

ULTOMIRIS is only available through a program called the ULTOMIRIS REMS. Before you can receive ULTOMIRIS, your doctor must: enroll in the ULTOMIRIS REMS program; counsel you about the risk of meningococcal infection; give you information and a Patient Safety Card about the symptoms and your risk of meningococcal infection (as discussed above); and make sure that you are vaccinated with a meningococcal vaccine, and if needed, get revaccinated with the meningococcal vaccine. Ask your doctor if you are not sure if you need to be revaccinated.

ULTOMIRIS may also increase the risk of other types of serious infections. Make sure your child receives vaccinations against *Streptococcus pneumoniae* and *Haemophilis influenzae* type b (Hib) if treated with ULTOMIRIS. Call your doctor right away if you have any new signs or symptoms of infection.

Who should not receive ULTOMIRIS?

Do not receive ULTOMIRIS if you have a meningococcal infection or have not been vaccinated against meningococcal infection unless your doctor decides that urgent treatment with ULTOMIRIS is needed.

Before you receive ULTOMIRIS, tell your doctor about all of your medical conditions, including if you: have an infection or fever, are pregnant or plan to become pregnant, and are breastfeeding or plan to breastfeed. It is not known if ULTOMIRIS will harm your unborn baby or if it passes into your breast milk. You should not breastfeed during treatment and for 8 months after your final dose of ULTOMIRIS.

Tell your doctor about all the vaccines you receive and medicines you take, including prescription and over-the-counter medicines, vitamins, and herbal supplements which could affect your treatment.

If you have PNH and you stop receiving ULTOMIRIS, your doctor will need to monitor you closely for at least 16 weeks after you stop ULTOMIRIS. Stopping ULTOMIRIS may cause breakdown of your red blood cells due to PNH. Symptoms or problems that can happen due to red blood cell breakdown include: drop in your red blood cell count, tiredness, blood in your urine, stomach-area (abdomen) pain, shortness of breath, blood clots, trouble swallowing, and erectile dysfunction (ED) in males.

If you have aHUS, your doctor will need to monitor you closely for at least 12 months after stopping treatment for signs of worsening aHUS or problems related to a type of abnormal clotting and breakdown of your red blood cells called thrombotic microangiopathy (TMA). Symptoms or problems that can happen with TMA may include: confusion or loss of consciousness, seizures, chest pain (angina), difficulty breathing and blood clots or stroke.

ULTOMIRIS can cause serious side effects including allergic reactions to acrylic adhesive. Allergic reactions to the acrylic adhesive may happen with your subcutaneous ULTOMIRIS treatment. If you have an allergic reaction during the delivery of subcutaneous ULTOMIRIS, remove the on-body injector and get medical help right away. Your healthcare provider may treat you with medicines to help prevent or treat allergic reaction symptoms as needed.

What are the possible side effects of ULTOMIRIS?

ULTOMIRIS can cause serious side effects including infusion-related reactions. Symptoms of an infusion-related reaction with ULTOMIRIS may include lower back pain, tiredness, feeling faint, discomfort in your arms or legs, or bad taste. Tell your doctor or nurse right away if you develop these symptoms, or any other symptoms during your ULTOMIRIS infusion that may mean you are having a serious infusion reaction, including: chest pain, trouble breathing or shortness of breath, swelling of your face, tongue, or throat, and feel faint or pass out.

The most common side effects of ULTOMIRIS in people treated for PNH are upper respiratory tract infection and headache.

The most common side effects of ULTOMIRIS in people with aHUS are upper respiratory tract infection, diarrhea, nausea, vomiting, headache, high blood pressure and fever.

The most common side effects of ULTOMIRIS in people with gMG are diarrhea and upper respiratory tract infection.

The most common side effects of subcutaneous administration of ULTOMIRIS in adults treated for PNH and aHUS are local injection site reactions.

Tell your doctor about any side effect that bothers you or that does not go away. These are not all the possible side effects of ULTOMIRIS. For more information, ask your doctor or pharmacist. Call your doctor right away if you miss an ULTOMIRIS infusion or for medical advice about side effects. You may report side effects to FDA at 1-800-FDA-1088.

Read the Instructions for Use that comes with subcutaneous ULTOMIRIS for instructions about the right way to prepare and give your subcutaneous ULTOMIRIS injections through an on-body injector.

Please see the accompanying full Prescribing Information and Medication Guide for ULTOMIRIS, including Boxed WARNING regarding serious and life-threatening meningococcal infections/sepsis. Please see the accompanying Instructions for Use for the ULTOMIRIS On Body Delivery System.

Notes

Alexion

Alexion, AstraZeneca Rare Disease, is the group within AstraZeneca focused on rare diseases, created following the 2021 acquisition of Alexion Pharmaceuticals, Inc. As a leader in rare diseases for 30 years, Alexion is focused on serving patients and families affected by rare diseases and devastating conditions through the discovery, development and commercialization of life-changing medicines. Alexion focuses its research efforts on novel molecules and targets in the complement cascade and its development efforts on hematology, nephrology, neurology, metabolic disorders, cardiology and ophthalmology. Headquartered in Boston, Massachusetts, Alexion has offices around the globe and serves patients in more than 50 countries. For more information, please visit www.alexion.com.

AstraZeneca

AstraZeneca is a global, science-led biopharmaceutical company that focuses on the discovery, development and commercialization of prescription medicines in Oncology, Rare Diseases, and BioPharmaceuticals, including Cardiovascular, Renal & Metabolism, and Respiratory & Immunology. Based in Cambridge, UK, AstraZeneca operates in over 100 countries and its innovative medicines are used by millions of patients worldwide. Please visit www.astrazeneca-us.com and follow us on Twitter @AstraZenecaUS.

ULTOMIRIS

ULTOMIRIS (ravulizumab-cwvz), the first and only long-acting C5 complement inhibitor, offers immediate, complete and sustained complement inhibition. The medication works by inhibiting the C5 protein in the terminal complement cascade, a part of the body's immune system. When activated in an uncontrolled manner, the complement cascade over-responds, leading the body to attack its own healthy cells. ULTOMIRIS is administered intravenously every eight weeks in adult patients, following a loading dose.

ULTOMIRIS is approved in the US, EU and Japan for the treatment of certain adults with gMG.

ULTOMIRIS is also approved in the US, EU and Japan for the treatment of certain adults with PNH and for certain children with PNH in the US and EU.

Additionally, ULTOMIRIS is approved in the US, EU and Japan for certain adults and children with aHUS to inhibit complement-mediated thrombotic microangiopathy.

As part of a broad development program, ULTOMIRIS is being assessed for the treatment of additional hematology and neurology indications.

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