

Danicopan as add-on to ULTOMIRIS® or SOLIRIS® improved hemoglobin levels and maintained disease control in patients with PNH experiencing signs or symptoms of clinically significant extravascular hemolysis

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Results from ALPHA Phase III trial demonstrated statistically significant increase compared to placebo in mean hemoglobin from baseline to week 12

All key secondary endpoints met statistical superiority, showing danicopan reduced fatigue and anemia as well as the need for transfusions

Positive results from the pivotal Phase III ALPHA trial showed that investigational, first-in-class oral Factor D inhibitor danicopan as add-on to standard of care C5 inhibitor therapy ULTOMIRIS® (ravulizumab-cwvz) or SOLIRIS® (eculizumab) demonstrated a statistically significant and clinically meaningful increase in hemoglobin levels and maintained disease control in patients with paroxysmal nocturnal hemoglobinuria (PNH) who experience clinically significant extravascular hemolysis (EVH), compared to placebo plus established C5 inhibitor therapy.¹

The data were presented today at the European Hematology Association (EHA) Annual Meeting in Frankfurt, Germany.

PNH is a rare and severe blood disorder characterized by the destruction of red blood cells within blood vessels, known as intravascular hemolysis (IVH), and white blood cell and platelet activation that can cause thrombosis (blood clots) and result in organ damage and potentially premature death.²⁻⁴ Approximately 10-20% of people living with PNH who are treated with a C5 inhibitor experience clinically significant EVH, which can result in continued symptoms of anemia and require blood transfusions.^{1,5,6}

Professor Jong Wook Lee, MD, PhD, Department of Hematology at Seoul St. Mary's Hospital of The Catholic University of Korea, and investigator in the ALPHA trial, said: "While EVH is not life-threatening, its manifestations can be burdensome for patients. The ALPHA trial demonstrated that adding danicopan to standard of care with eculizumab or ravulizumab significantly improved fatigue and anemia and reduced transfusion dependence, while still allowing for sustained control of IVH with terminal complement inhibition addressing the thrombotic risks associated with PNH. These results suggest danicopan has the potential to be an important option for the small subset of patients with PNH who experience clinically significant EVH while being treated with eculizumab or ravulizumab."

Gianluca Pirozzi, Senior Vice President, Head of Development, Regulatory and Safety, Alexion, said: "As a leader in PNH for decades, Alexion has transformed the treatment landscape by developing the first approved medicine for this rare disease and establishing C5 complement inhibition as standard of care. These promising results presented at the EHA Annual Meeting underscore the potential for targeted Factor D inhibition with danicopan as an add-on to ULTOMIRIS or SOLIRIS to address clinically significant EVH while allowing patients to maintain disease control with established C5 complement inhibitors."

At the prespecified interim analysis for the ALPHA trial – occurring after 63 study participants either completed or discontinued from the primary treatment period of 12 weeks – danicopan met the primary efficacy endpoint. In patients managing PNH with ULTOMIRIS or SOLIRIS, add-on treatment with danicopan was superior to placebo plus ULTOMIRIS or SOLIRIS based on change in hemoglobin from baseline to week 12, reported as least squares mean change from baseline and standard error of the mean (2.94 [0.211] g/dL vs 0.50 [0.313] g/dL; $p < 0.0001$). Further, significant improvements in hemoglobin were observed with danicopan by week 2 and maintained through week 12.¹

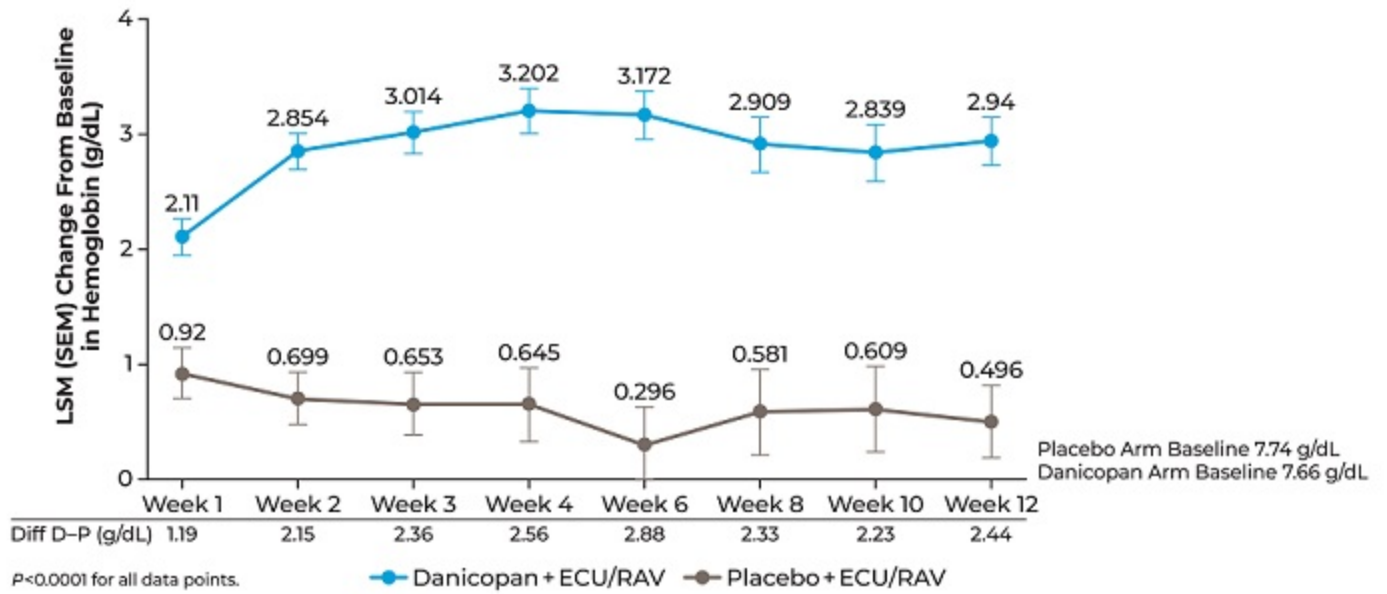
All key secondary endpoints also met statistical superiority in favor of danicopan plus ULTOMIRIS or SOLIRIS, compared to placebo plus C5 inhibition. Results showed significantly more patients treated with danicopan (59.5%) versus placebo (0%) experienced an improvement in hemoglobin of ≥ 2 g/dL at week 12 in the absence of transfusion (difference in danicopan-placebo: 46.9, 95% CI: 29.2–64.7, $p < 0.0001$).¹

Additionally, significantly more patients treated with danicopan avoided transfusion (remaining transfusion-free and not requiring a transfusion as per protocol) through week 12, versus the comparator arm. Improvements in fatigue, as measured by Functional Assessment of Chronic Illness Therapy – Fatigue (FACIT-Fatigue) score, and absolute reticulocyte count, another indicator of clinically significant EVH, were also shown.¹

Further, change from baseline in lactate dehydrogenase (LDH) at Week 12 was -23.49 [8.29] U/L in the danicopan plus ULTOMIRIS or SOLIRIS arm and -2.92 [11.91] U/L in the placebo arm ($p = 0.1569$), demonstrating effective control of IVH was maintained with C5 inhibition in both arms.

Summary of efficacy results^{i,ii}

Danicopan add-on treatment to SOLIRIS or ULTOMIRIS resulted in a statistically significant increase in hemoglobin from baseline to week 12 versus placebo



Endpoint	Statistic	Danicopan + ULTOMIRIS/SOLIRIS (N = 42)	Placebo + ULTOMIRIS/SOLIRIS (N = 21)
Primary Endpoint			
Change in Hgb from baseline at Week 12	LSM (SEM) Difference (D–P) (SEM) P value	2.94 (0.211)	0.50 (0.313) 2.44 <0.0001
Key Secondary Endpoints			
Percentage of Participants with Hgb Increase ≥2 g/dL at Week 12 in the Absence of Blood Transfusion	Percentage (%) Difference (D–P)† 95% CI P value	59.5	0 46.9 29.2–64.7 <0.0001
Percentage of Participants with Transfusion Avoidance‡ Through Week 12	Percentage (%) Difference (D–P)† 95% CI P value	83.3	38.1 41.7 22.7–60.8 0.0004
Change From Baseline to Week 12 in FACIT-Fatigue Score	LSM (SEM) Difference (D–P) 95% CI P value	7.97	1.85 6.12 2.33–9.91 0.0021
Change From Baseline to Week 12 in ARC	LSM (SEM) Difference (D–P) 95% CI P value	–83.8	3.5 –87.2 –117.7 to –56.7 <0.0001

i. Results analyzed comparing danicopan plus ULTOMIRIS or SOLIRIS to placebo plus ULTOMIRIS or SOLIRIS from the Phase III ALPHA trial.

ii. ARC, absolute reticulocyte count; D, danicopan; FACIT-Fatigue, Functional Assessment of Chronic Illness Therapy-Fatigue scale (version 4.0); Hgb, hemoglobin; LSM, least squares mean; P, placebo; SEM, standard error of the mean.

†Difference in rates and corresponding 95% CI values were calculated using Miettinen and Nurminen method adjusting for stratification factors.

‡Defined as patients who remain transfusion-free and do not require a transfusion per protocol-specified guidelines.

Results from this Phase III trial showed danicopan is generally well tolerated, and no new safety concerns were identified. The safety analysis set included 49 and 24 participants in the danicopan and placebo arms, respectively. No treatment-emergent adverse events (TEAEs) were Grade 4 or 5. The most common TEAEs were headache (10.2%), nausea (8.2%), arthralgia (8.2%) and diarrhea (8.2%) in the treatment arm. There were no reported deaths, meningococcal infections or discontinuations due to hemolysis.¹

Regulatory submissions for danicopan for adults with PNH experiencing clinically significant EVH are currently under review with multiple global health authorities.

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.

- adults with PNH or aHUS when administered subcutaneously (under your skin).

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 healthcare provider 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 healthcare provider will decide if you need additional vaccination.
 5. Meningococcal vaccines reduce but do not prevent all meningococcal infections. Call your healthcare provider 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 healthcare provider 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 healthcare provider 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 healthcare provider 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 healthcare provider 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 *Haemophilus influenzae* type b (Hib) if treated with ULTOMIRIS. Call your healthcare provider 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 healthcare provider decides that urgent treatment with ULTOMIRIS is needed.

Before you receive ULTOMIRIS, tell your healthcare provider 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 healthcare provider 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 healthcare provider 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 healthcare provider 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, bad taste, or drowsiness. Stop treatment of ULTOMIRIS and tell your healthcare provider 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 treated for 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 infections.

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

Tell your healthcare provider 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 healthcare provider or pharmacist. Call your healthcare provider 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.

INDICATIONS & IMPORTANT SAFETY INFORMATION FOR SOLIRIS[®] (eculizumab) [injection for intravenous use 300mg/30mL vial]

What is SOLIRIS?

SOLIRIS is a prescription medicine used to treat:

- patients with a disease called Paroxysmal Nocturnal Hemoglobinuria (PNH).
- adults and children with a disease called atypical Hemolytic Uremic Syndrome (aHUS). SOLIRIS is not for use 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.
- adults with a disease called neuromyelitis optica spectrum disorder (NMOSD) who are anti-aquaporin-4 (AQP4) antibody positive.

It is not known if SOLIRIS is safe and effective in children with PNH, gMG, or NMOSD.

IMPORTANT SAFETY INFORMATION

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

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

- **SOLIRIS 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.**
 - You must receive meningococcal vaccines at least 2 weeks before your first dose of SOLIRIS if you are not vaccinated.
 - If your doctor decided that urgent treatment with SOLIRIS is needed, you should receive meningococcal vaccination as soon as possible.
 - If you have not been vaccinated and SOLIRIS therapy must be initiated immediately, you should also receive 2 weeks of antibiotics with your vaccinations.
 - If you had a meningococcal vaccine in the past, you might need additional vaccination. Your doctor will decide if you need additional vaccination.
 - 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 3 months after your last SOLIRIS dose. It is important to show this card to any doctor or nurse to help them diagnose and treat you quickly.

SOLIRIS is only available through a program called the SOLIRIS REMS. Before you can receive SOLIRIS, your doctor must enroll in the SOLIRIS 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 the 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.

SOLIRIS may also increase the risk of other types of serious infections. Make sure your child receives vaccinations against *Streptococcus pneumoniae* and *Haemophilus influenzae* type b (Hib) if treated with SOLIRIS. Certain people may be at risk of serious infections with gonorrhea. Certain fungal infections (*Aspergillus*) may occur if you take SOLIRIS and have a weak immune system or a low white blood cell count.

Who should not receive SOLIRIS?

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

Before you receive SOLIRIS, 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 SOLIRIS will harm your unborn baby or if it passes into your breast milk.

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. It is important that you have all recommended vaccinations before you start SOLIRIS, receive 2 weeks of antibiotics if you immediately start SOLIRIS, and stay up-to-date with all recommended vaccinations during treatment with SOLIRIS.

If you have PNH, your doctor will need to monitor you closely for at least 8 weeks after stopping SOLIRIS. Stopping treatment with SOLIRIS 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 the number of your red blood cell count, drop in your platelet count, confusion, kidney problems, blood clots, difficulty breathing, and chest pain.

If you have aHUS, your doctor will need to monitor you closely during and for at least 12 weeks after stopping treatment for signs of worsening aHUS symptoms or problems related to abnormal clotting (thrombotic microangiopathy). Symptoms or problems that can happen with abnormal clotting may include: stroke, confusion, seizure, chest pain (angina), difficulty breathing, kidney problems, swelling in arms or legs, and a drop in your platelet count.

What are the possible side effects of SOLIRIS?

SOLIRIS can cause serious side effects including serious infusion-related reactions. Tell your doctor or nurse right away if you get any of these symptoms during your SOLIRIS infusion: chest pain; trouble breathing or shortness of breath; swelling of your face, tongue, or throat; and feel faint or pass out. If you have an infusion-related reaction to SOLIRIS, your doctor may need to infuse SOLIRIS more slowly, or stop SOLIRIS.

The most common side effects in people with PNH treated with SOLIRIS include: headache, pain or swelling of your nose or throat (nasopharyngitis), back pain, and nausea.

The most common side effects in people with aHUS treated with SOLIRIS include: headache, diarrhea, high blood pressure (hypertension), common cold (upper respiratory infection), stomach-area (abdominal) pain, vomiting, pain or swelling of your nose or throat (nasopharyngitis), low red blood cell count (anemia), cough, swelling of legs or feet (peripheral edema), nausea, urinary tract infections, and fever.

The most common side effects in people with gMG treated with SOLIRIS include: muscle and joint (musculoskeletal) pain.

The most common side effects in people with NMOSD treated with SOLIRIS include: common cold (upper respiratory infection); pain or swelling of your nose or throat (nasopharyngitis); diarrhea; back pain; dizziness; flu-like symptoms (influenza), including fever, headache, tiredness, cough, sore throat, and body aches; joint pain (arthralgia); throat irritation (pharyngitis); and bruising (contusion).

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 SOLIRIS. For more information, ask your doctor or pharmacist. Call your doctor for medical advice about side effects. You are encouraged to report negative side effects of prescription drugs to the FDA. Visit MedWatch, or call 1-800-FDA-1088.

Please see the full [Prescribing Information](#) and [Medication Guide](#) for SOLIRIS, including **Boxed WARNING regarding serious and life-threatening meningococcal infections.**

Notes

PNH

PNH is a rare, chronic, progressive and potentially life-threatening blood disorder. It is characterized by red blood cell destruction within blood vessels (also known as intravascular hemolysis) and white blood cell and platelet activation, which can result in thrombosis (blood clots).²⁻⁴

PNH is caused by an acquired genetic mutation that may happen any time after birth and results in the production of abnormal blood cells that are missing important protective blood cell surface proteins. These missing proteins enable the complement system, which is part of the immune system and is essential to the body's defense against infection, to 'attack' and destroy or activate these abnormal blood cells.² Living with PNH can be debilitating, and signs and symptoms may include blood clots, abdominal pain, difficulty swallowing, erectile dysfunction, shortness of breath, excessive fatigue, anemia and dark-colored urine.^{2,7,8}

Clinically Significant EVH

EVH, the removal of red blood cells outside of the blood vessels, can sometimes occur in PNH patients who are treated with C5 inhibitors.^{5,9} Since C5 inhibition enables PNH red blood cells to survive and circulate, EVH may occur when these now surviving PNH red blood cells are marked by proteins in the complement system for removal by the spleen and liver.^{2,4,5} Patients with PNH with EVH may continue to experience anemia, which can have various causes, and may require blood transfusions.^{5,9-11} A small subset of people living with PNH who are treated with a C5 inhibitor

experience clinically significant EVH, which can result in continued symptoms of anemia and require blood transfusions.^{2,8,12,13}

ALPHA

ALPHA is a pivotal, global Phase III trial designed as a superiority study to evaluate the efficacy and safety of danicopan as an add-on to C5 inhibitor therapy eculizumab or ravulizumab-cwvz in patients with PNH who experience clinically significant EVH. In the double-blind, placebo-controlled, multiple-dose trial, patients were enrolled and randomized to receive danicopan or placebo (2:1) in addition to their ongoing eculizumab or ravulizumab-cwvz therapy for 12 weeks. A prespecified interim analysis was performed once 63 randomized patients had completed 12 weeks of the primary evaluation period or discontinued treatment as of June 28, 2022. At 12 weeks, patients on placebo plus a C5 inhibitor were switched to danicopan plus eculizumab or ravulizumab-cwvz, and patients on danicopan plus eculizumab or ravulizumab-cwvz remained on treatment for an additional 12 weeks. Patients who completed both treatment periods (24 weeks) had the option to participate in a two-year long-term extension period and continue to receive danicopan in addition to eculizumab or ravulizumab-cwvz. The open-label period of the study is still ongoing.^{1,14}

Danicopan

Danicopan is an investigational oral medicine in development as an add-on to C5 inhibitor therapy eculizumab or ravulizumab-cwvz for patients with PNH who experience clinically significant EVH. It is designed to selectively inhibit factor D, a complement system protein that plays a key role in the amplification of the complement system response. Danicopan has been granted Breakthrough Therapy designation by the US Food and Drug Administration and PRiority Medicines (PRIME) status by the European Medicines Agency. Danicopan has also been granted Orphan Drug Designation in the US, EU and Japan for the treatment of PNH. Alexion is also evaluating danicopan as a potential monotherapy for geographic atrophy in a Phase II clinical trial.

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.

SOLIRIS

SOLIRIS (eculizumab) is a first-in-class C5 complement inhibitor. 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 terminal complement cascade over-responds, leading the body to attack its own healthy cells. SOLIRIS is administered intravenously every two weeks, following an introductory dosing period.

SOLIRIS is approved in the US, EU and Japan for the treatment of PNH, aHUS, certain adults with gMG and certain adults with NMOSD.

SOLIRIS is not indicated for the treatment of patients with STEC-HUS.

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 more than 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. For more information, please visit <http://www.astrazeneca-us.com/> and follow us on Twitter [@AstraZenecaUS](https://twitter.com/AstraZenecaUS).

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