ULTOMIRIS® (ravulizumab-cwvz) FDA APPROVAL FOR CHILDREN[a] WITH PNH

FREQUENTLY ASKED QUESTIONS

[a]Aged one month and older.

Alexion is committed to bringing therapies to patients with rare diseases. Through our work with patients, we know that living with PNH involves a lot of time for disease management and care. **We recognize how important it is that we keep our patients up to date on any and all changes to our treatments, so we are making every effort to provide this information to you as quickly as possible.**

The following Frequently Asked Questions may not answer all of your questions, so for any additional information, please reach out to our OneSource™ Patient Services team at 1-888-765-4747.

INDICATION

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

Please see Important Safety Information on pages 4 and 5, full Prescribing Information, and Medication Guide for ULTOMIRIS, including Boxed WARNING regarding serious and life-threatening meningococcal infections/sepsis.

Children of what age can now be treated for PNH with ULTOMIRIS?

ULTOMIRIS is now FDA approved for the treatment of children with PNH aged 1 month and older. Findings from an ongoing clinical study that included 13 children (ages 9 to 17 years) contributed to the approval. Of the 13 children, 8 had been treated with another complement inhibitor (eculizumab) and 5 had never been treated with a complement inhibitor.

Are there any other FDA approved medications for children with PNH?

No. ULTOMIRIS (ravulizumab-cwvz) is currently the only FDA approved medication for children 1 month of age and older with PNH.

How does the safety of ULTOMIRIS in children with PNH compare to the safety that has been established in adults?

The types and rates of side effects experienced by children with PNH in the ULTOMIRIS clinical study were similar to those seen in adults. The most common side effects of ULTOMIRIS among the children in the study were upper respiratory tract infection, anemia, abdominal pain, and headache.

For more information, please see Important Safety Information on pages 4 and 5, full Prescribing Information, and Medication Guide for ULTOMIRIS, including Boxed WARNING regarding serious and life-threatening meningococcal infections/sepsis.

How does the efficacy of ULTOMIRIS in children with PNH compare to the efficacy that has been established in adults?

The efficacy of ULTOMIRIS in children with PNH appeared to be similar to that seen in adults. Children treated with ULTOMIRIS in the clinical study experienced improvements in lab tests measuring PNH activity and in PNH symptoms. For more information, please review the full Prescribing Information for ULTOMIRIS or go to ULTOMIRIS.com/pnh.

What is the ULTOMIRIS dose for children with PNH, and how often is it given?

The ULTOMIRIS dose and treatment schedule are determined by the child's body weight. All people with PNH starting ULTOMIRIS receive a starting dose, followed 2 weeks later by the first maintenance dose. Maintenance doses are given every 4 weeks for people weighing less than 20 kg (44 lbs) and every 8 weeks for people weighing 20 kg or more. ULTOMIRIS is given by intravenous (IV) infusion.

What will be my out-of-pocket cost and insurance coverage for ULTOMIRIS?

If you choose to enroll in Alexion OneSource, we can help review your health insurance coverage and specific out-of-pocket costs for ULTOMIRIS therapy. We can also provide information about available financial assistance programs that may help cover some or all of your out-of-pocket costs.

Can children with PNH and their caregivers enroll in OneSource?

Yes. Today, we can provide the full suite of OneSource services for children with PNH who are starting or already on treatment with ULTOMIRIS and their caregivers. OneSource services include education about PNH and ULTOMIRIS, health insurance navigation, information on available financial assistance resources, connections to the PNH community, and ongoing support to help you maintain PNH treatment through life changes, such as a move or travel.

My child used to be enrolled in OneSource; can I re-enroll now? Will I have the same Case Manager?

OneSource can now provide patient support services to children with PNH who are starting or already on treatment with ULTOMIRIS (ravulizumab-cwvz) and their caregivers. We continue to evolve our OneSource services to ensure a personalized experience. Since you last connected with OneSource, we have made some changes that let us provide a more seamless and specialized experience.

As a result of these changes, you may have a new contact at OneSource. Your Patient Navigator will be focused on providing support while your child is starting treatment with ULTOMIRIS. After a few infusions, your Patient Liaison will provide ongoing support.

While the titles of your contacts at OneSource have changed, our focus remains the same: providing you with the best support services possible. We look forward to helping you.

My child is currently on SOLIRIS® (eculizumab) for PNH. Can I enroll in OneSource?

OneSource can provide support services to people who are being treated with an Alexion therapy for an FDA-approved indication, including children with PNH aged 1 month and older.

Because SOLIRIS is not FDA approved for the treatment of children with PNH, OneSource cannot provide services to children who are receiving SOLIRIS for PNH at this time, although Alexion can continue to provide support to your child's healthcare provider and their office. Please speak with your child's healthcare provider if you have any additional questions.

Who can I talk to about treating my child's PNH with ULTOMIRIS?

Please speak with your healthcare provider about the treatment choices for your child. At any time, if you and your healthcare provider decide to start ULTOMIRIS treatment, you can choose to enroll in OneSource for patient support services.

ONESOURCE®

Personalized Patient Support from Alexion

Call 1-888-765-4747, email OneSource@Alexion.com, or visit www.AlexionOneSource.com

IMPORTANT SAFETY INFORMATION for ULTOMIRIS® (ravulizumab-cwvz)

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 *Haemophilus 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.

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, feeling faint or discomfort in your arms or legs. 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.

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.

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