Indications and usage

Soliris® is indicated for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH) to reduce hemolysis.

Soliris is indicated for the treatment of patients with atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy.

The effectiveness of Soliris in aHUS is based on the effects on thrombotic microangiopathy (TMA) and renal function. Prospective clinical trials in additional patients are ongoing to confirm the benefit of Soliris in patients with aHUS.

Limitation of Use

Soliris is not indicated for the treatment of patients with Shiga toxin E. coli related hemolytic uremic syndrome (STEC-HUS).
Atypical Hemolytic Uremic Syndrome (aHUS) is a rare and complex disease, and you probably have many questions about it. It is important to learn about your disease, how to live with it, and your treatment options. The more you know, the better you can help your doctor to manage your care.

This brochure will help you understand more about your diagnosis and give you answers to your questions. You can find the definition of words that are underlined in the Glossary on pages 20-21.

Questions that are answered in this brochure:

- What is aHUS?
- What is Soliris®?
- What do I need to know before taking Soliris?
- How is Soliris given?
- Where can I find out more?
- What should I know about the risk of infection?
- Important information about Soliris

Since you have been diagnosed with atypical Hemolytic Uremic Syndrome (aHUS), you most likely have a lot of questions.

Please see accompanying full Prescribing Information and Medication Guide for Soliris, including Boxed WARNING regarding serious meningococcal infection.

Please see Important Safety Information on pages 16-19.
What are the serious complications of aHUS?

aHUS affects the blood system, kidney, and sometimes other body organs. When left untreated, aHUS can cause vital organs to either suddenly fail, or slowly lose their ability to function over time.

What is aHUS?

aHUS is a rare, genetic, chronic disease that can damage vital organs such as the kidneys, heart, and brain. This disease can develop at any age, and if left untreated, aHUS may lead to serious complications.

aHUS affects a part of the body’s immune system called complement. When the immune system is working normally, complement is usually controlled by natural inhibitors that keep the complement system activity in check.

In aHUS, changes in certain genes cause a loss of natural inhibitors, which results in uncontrolled complement activation. This inability to control complement, or keep the system in check, causes damage to those cells in the body that the immune system usually protects. This leads to blood clots. Clots can block blood flow and trap platelets, create inflammation, and travel to other organs, causing further damage.

It’s important that you understand your diagnosis of aHUS, so that you can take an active role in your treatment.

“I was hospitalized and doctors diagnosed me with atypical Hemolytic Uremic Syndrome (aHUS). I had no idea what it meant, but I soon realized this disease was very serious.”

— Patient with aHUS

Please see accompanying full Prescribing Information and Medication Guide for Soliris, including Boxed WARNING regarding serious meningococcal infection.

Please see Important Safety Information on pages 16-19.
Soliris® was shown to be effective in two 26-week studies
Your doctor may recommend ongoing treatment with Soliris as an important part of helping to manage aHUS.

- Many patients taking Soliris had an increase in their platelet counts, which indicates that the small clots that had been trapping the platelets are no longer being formed
- Many patients no longer needed plasma exchange or plasma infusion (PE/PI). Many patients did not need dialysis for at least 12 consecutive weeks
- Soliris prevented, and sometimes eliminated, the need for dialysis treatment in some patients who had reduced kidney function

Speak with your doctor about how Soliris can help in the treatment of aHUS.

As demonstrated in clinical trials
Common side effects in people with aHUS treated with Soliris include:
- High blood pressure
- Common cold (upper respiratory infection)
- Diarrhea
- Headache
- Nausea and vomiting
- Low red blood cell count
- Low white blood cell count
- Urinary tract infection

Soliris is the first and only drug approved by the FDA to treat patients with aHUS.
Soliris is a medicine that affects your immune system. Soliris can lower the ability of your immune system to fight infections.

"My 18-year-old daughter was diagnosed with aHUS and was approved for Soliris treatment. She has received eight infusions. Her labs show that Soliris is working. We understand how truly lucky we are."
—Parent of a patient taking Soliris

Please see accompanying full Prescribing Information and Medication Guide for Soliris, including Boxed WARNING regarding serious meningococcal infection.
Please see Important Safety Information on pages 16-19.
If you stop or interrupt Soliris, aHUS symptoms may come back.

In aHUS, the risk of disease complications continues throughout your life because of an abnormality in your genes. The body cannot naturally regulate part of its own immune system. Ongoing treatment is an important part of managing aHUS, so it is important to speak with your doctor if you have questions about it.

Your doctor may discontinue Soliris® if you are undergoing treatment for serious meningococcal infection. If you have chest pain or shortness of breath, your doctor will interrupt the Soliris infusion and monitor you closely.

In aHUS, the risk of complement-mediated thrombotic microangiopathy (TMA) is always present so if you interrupt or stop Soliris treatment, symptoms may return and you may be at risk for TMA complications. This can cause renewed damage to the cells and vessels of the body, especially in the kidney. Clotting problems and other complications can return.

Important Safety Information if You Stop Treatment

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 (TMA).

Symptoms or problems that can happen with abnormal clotting may include:

- Stroke
- Confusion
- Seizures
- Chest pain (angina)
- Difficulty breathing
- Kidney problems
- Swelling in arms or legs
- A drop in your platelet count

Please see accompanying full Prescribing Information and Medication Guide for Soliris, including Boxed WARNING regarding serious meningococcal infection. Please see Important Safety Information on pages 16-19.

Soliris is a therapy for aHUS—an ongoing disease needing ongoing treatment.

Keep track of your health.

Ask your doctor or your OneSource Nurse Case Manager for a copy of the Patient aHUS Symptom Tracker. It is also available through OneSource. Do your best to keep track of your signs and symptoms with the aHUS Symptom Tracker checklist. Share it with your doctor.

If you experience any of these symptoms, it’s important that you seek immediate medical care. And remember to keep your Patient Safety Information Card with you at all times.
**Important Safety Information**

Soliris increases your risk of getting serious and life-threatening meningococcal infections. A meningococcal vaccine does 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 a fever
- Headache with a stiff neck or stiff back
- Fever of 103°F (39.4°C) or higher

Get IMMEDIATE medical attention if you experience any signs or symptoms of a meningococcal infection.

Please see accompanying full Prescribing Information and Medication Guide for Soliris, including Boxed WARNING regarding serious meningococcal infection.

Please see Important Safety Information on pages 16-19.
Dosing considerations

Soliris® is given through a vein as an intravenous (IV) infusion, usually over 35 minutes.

If you are over 18 years of age, you will usually receive an infusion
— Every week for 5 weeks; then
— Every 2 weeks thereafter

If you are under 18 years of age, your doctor will decide your dose and frequency of treatment based on your age and weight.

If you are also receiving PE/PI, additional doses of Soliris are required based on the Prescribing Information.

If you have an allergic reaction during your Soliris infusion, your doctor may decide to give you Soliris more slowly or stop your infusion. After each infusion, you should be monitored for 1 hour for allergic reactions.

Serious allergic reactions can happen during your Soliris infusion. Tell your doctor or nurse right away if you get any of these symptoms during Soliris infusion:
— Chest pain
— Trouble breathing or shortness of breath
— Swelling of your face, tongue, or throat
— Feel faint or pass out

Tell your doctor right away if you have any of these symptoms during your Soliris infusion.

Please see accompanying full Prescribing Information and Medication Guide for Soliris, including Boxed WARNING regarding serious meningococcal infection.
Please see Important Safety Information on pages 16-19.
Patient Safety Card

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. Your risk of meningococcal infection may continue for several weeks after your last dose of Soliris. It is important to show this card to any doctor or nurse who treats you. This will help them diagnose and treat you quickly.

The Patient Safety Card on this page contains Important Safety Information that you should know while receiving Soliris therapy. Because Soliris may reduce your natural resistance to infections, it is important to recognize the signs and symptoms of infections, including those of meningitis. This card includes a list of the signs and symptoms of these infections, so you can recognize an infection, including meningitis, and seek IMMEDIATE medical attention.

Remember to keep your Patient Safety Card with you during treatment and for 3 months after your last Soliris dose.

Soliris is only available through a program called the Soliris Risk Evaluation and Mitigation Strategy (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 about the symptoms of meningococcal infection
☑ Give you a Patient Safety Card about your risk of meningococcal infection
☑ Make sure that you are vaccinated with a meningococcal vaccine

Soliris may also increase the risk of other types of serious infections. If your child is treated with Soliris, make sure that your child receives vaccinations against Streptococcus pneumonia and Haemophilus influenza type b (Hib).

Remember to keep your Patient Safety Card with you during treatment and for 3 months after your last Soliris dose.

Please see accompanying full Prescribing Information and Medication Guide for Soliris, including Boxed WARNING regarding serious meningococcal infection.

Please see Important Safety Information on pages 16-19.
Indications and Important Safety Information

**WARNIMG: SERIOUS MENINGOCOCCAL INFECTIONS**

*See full prescribing information for complete boxed warning*

Life-threatening and fatal meningococcal infections have occurred in patients treated with Soliris and may become rapidly life-threatening or fatal if not recognized and treated early (5.1).

- Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for meningococcal vaccination in patients with complement deficiencies (5.1).
- Immunize patients with a meningococcal vaccine at least 2 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 Serious Meningococcal Infections (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 (5.2).

**Indications and usage**

Soliris® is indicated for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH) to reduce hemolysis.

Soliris is indicated for the treatment of patients with atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy. The effectiveness of Soliris in aHUS is based on the effects on thrombotic microangiopathy (TMA) and renal function. Prospective clinical trials in additional patients are ongoing to confirm the benefit of Soliris in patients with aHUS.

**Limitation of Use**

Soliris should not be used to treat patients with Shiga toxin *E. coli* related hemolytic uremic syndrome (STEC-HUS).

**Important Safety Information**

**Contraindications**

Soliris should not be used in:
- Patients who have meningococcal infection.
- Patients who have not been vaccinated against meningitis infection unless the doctor decides that urgent treatment with Soliris is needed.

**Warnings and precautions**

Soliris increases your risk of getting serious and life-threatening meningococcal infections.

Meningococcal infections may quickly become life-threatening and cause death if not recognized and treated early.

1. You must receive a meningococcal vaccine at least 2 weeks before your first dose of Soliris unless you have already had this vaccine. If your doctor decides that urgent treatment with Soliris is needed, you should receive a meningococcal vaccine as soon as possible.

2. If you had a meningococcal vaccine in the past, you might need a booster dose before starting Soliris. Your doctor will decide if you need another dose of a meningococcal vaccine.

3. A meningococcal vaccine does 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 a fever
   - Headache with a stiff neck or stiff back
   - Fever of 103°F (39.4°C) or higher
   - Fever and a rash
   - Confusion
   - Muscle aches with flu-like symptoms
   - Eyes sensitive to light

Please see accompanying full Prescribing Information and Medication Guide for Soliris, including Boxed WARNING regarding serious meningococcal infection.
Other Infections

Soliris® may also increase the risk of other types of serious infections

If your child is treated with Soliris, make sure that your child receives the following vaccinations:

— Streptococcus pneumoniae
— Haemophilus influenzae type b (Hib)

Monitoring After Soliris Discontinuation

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
— Seizures
— Chest pain (angina)
— Difficulty breathing
— Kidney problems
— Swelling in arms or legs
— A drop in your platelet count

Treatment with Soliris should not stop you from taking your anticoagulant medicine (medicine that keeps your blood from clotting).

Laboratory Monitoring

Early signs of abnormal clotting (thrombotic microangiopathy) include changes in certain laboratory tests such as:

— Decrease in platelet count
— Increase in serum LDH
— Increase in creatinine level

Serious Allergic Reactions

Serious allergic reactions can happen during your Soliris infusion. 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
— Feel faint or pass out

If you have an allergic reaction to Soliris, your doctor may need to infuse Soliris more slowly, or stop Soliris.

Adverse Reactions

Common side effects in people with PNH treated with Soliris include:

— Headaches
— Runny nose and colds
— Sore throat
— Back pain
— Nausea

Common side effects in people with aHUS treated with Soliris include:

— High blood pressure
— Common cold (upper respiratory infection)
— Diarrhea
— Nausea and vomiting
— Low red blood cell count
— Low white blood cell count
— Urinary tract infection

Please see accompanying full Prescribing Information and Medication Guide for Soliris, including Boxed WARNING regarding serious meningococcal infection.
**Allergic reaction:** an overreaction after a substance is introduced into the body.

**atypical Hemolytic Uremic Syndrome (aHUS):** a disease of the blood that causes low red blood cell and platelet counts, kidney failure, and damage to other vital organs, such as the heart and brain.

**Blood clot:** a coagulated mass produced by clotting of blood.

**Complement:** components in the blood that interact as part of the body’s immune system to destroy disease-causing substances.

**Dialysis:** a treatment for kidney failure. Normally, the kidneys work to filter the blood and remove waste, excess salt, and water. Kidney failure, also called “end-stage renal disease,” occurs when the kidneys stop working completely. During hemodialysis, a machine takes over the job of the kidneys by filtering the blood outside of the body and then returning the filtered blood back to the body.

**FDA:** Food and Drug Administration.

**Genetic:** relating to genes, which are units in cells that are passed down through families.

**Immune system:** a complex group of cells, proteins, and other molecules that work together to identify foreign organisms and substances, such as bacteria; the main role of the system is to protect the body against these foreign organisms.

**Inflammation:** an immune system reaction from the body as a result of some type of injury. Signs of inflammation may be redness, swelling, pain, and/or heat.

**Infusion:** a process during which fluid is introduced into the body through a vein.

**Meningococcal infections:** infections caused by a group of bacteria called *Neisseria meningitidis*. The most common forms of meningococcal infections include meningitis (infection of the membranes that surround the brain and spinal cord) and meningococcemia (blood stream infections).

**Natural inhibitors:** the body’s natural protective immune system that regulates the body’s immune response.

**Plasma:** the body’s natural protective immune system that regulates the body’s immune response.

**Plasma exchange/plasma infusion (PE/PI):** a process of removing, treating, and returning, or infusing plasma to the body.

**Platelet:** a small, irregular, disc-shaped element in the blood that assists in blood clotting.

**Thrombotic microangiopathy (TMA):** formation of clots in small blood vessels throughout the body; this is an underlying cause of the clinical signs and symptoms of aHUS.

**Vaccine:** a preparation that is used to increase the body’s natural defense against a disease.
Indications and usage

Soliris® is indicated for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH) to reduce hemolysis. Soliris is indicated for the treatment of patients with atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy. The effectiveness of Soliris in aHUS is based on the effects on thrombotic microangiopathy (TMA) and renal function. Prospective clinical trials in additional patients are ongoing to confirm the benefit of Soliris in patients with aHUS.

Limitation of Use

Soliris is not indicated for the treatment of patients with Shiga toxin
E. coli
related hemolytic uremic syndrome (STEC-HUS).

SOLIRIS AND YOU:

YOUR GUIDE TO living with aHUS

Soliris® is a registered trademark of Alexion Pharmaceuticals, Inc. Copyright © 2013, Alexion Pharmaceuticals, Inc. All rights reserved. SOL XXXX

You are not alone. Call 1.888.SOLIRIS (1.888.765.4747) today!

WARNING: SERIOUS MENINGOCOCCAL INFECTIONS

See full prescribing information for complete boxed warning

Life-threatening and fatal meningococcal infections have occurred in patients treated with Soliris and may become rapidly life-threatening or fatal if not recognized and treated early (5.1).

• Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for meningococcal vaccination in patients with complement deficiencies (5.1).

• Immunize patients with a meningococcal vaccine at least 2 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 Serious Meningococcal Infections (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 (5.2).

CONTRAINDICATIONS

Soliris is contraindicated in:

• Patients with unresolved serious Neisseria meningitidis infection.

• Patients who are not currently vaccinated against Neisseria meningitidis, unless the risks of delaying Soliris treatment outweigh the risk of developing a meningococcal infection.

Please see accompanying full Prescribing Information and Medication Guide for Soliris. Please see Important Safety Information on pages 16-19.

Soliris—helping you manage aHUS

Living with aHUS can seem challenging, but there are steps you can take.

• Always talk to your doctor whenever you have questions or are unsure of what to do

• Get involved in your treatment—learn how to keep track of the results of the laboratory tests that your doctor orders

If your doctor determines that Soliris® is the right treatment for you, you may learn more by visiting the Soliris website or speaking with an Alexion Nurse Case Manager. The Alexion Nurse Case Managers are there to help you with answers to your questions about Soliris and the management of aHUS.

Visit http://www.soliris.net/patients/one-source or call 1.888.SOLIRIS (1.888.765.4747) today!
Indications and usage

Soliris® is indicated for the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH) to reduce hemolysis.

Soliris is indicated for the treatment of patients with atypical hemolytic uremic syndrome (aHUS) to inhibit complement-mediated thrombotic microangiopathy.

The effectiveness of Soliris in aHUS is based on the effects on thrombotic microangiopathy (TMA) and renal function. Prospective clinical trials in additional patients are ongoing to confirm the benefit of Soliris in patients with aHUS.

Limitation of Use

Soliris is not indicated for the treatment of patients with Shiga toxin E. coli related hemolytic uremic syndrome (STEC-HUS).

You are not alone.

Call 1.888.SOLIRIS (1.888.765.4747) today!

WARNING: SERIOUS MENINGOCOCCAL INFECTIONS

See full prescribing information for complete boxed warning

Life-threatening and fatal meningococcal infections have occurred in patients treated with Soliris and may become rapidly life-threatening or fatal if not recognized and treated early (5.1).

- Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for meningococcal vaccination in patients with complement deficiencies (5.1).

- Immunize patients with a meningococcal vaccine at least 2 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 Serious Meningococcal Infections (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 (5.2).

CONTRAINDICATIONS

Soliris is contraindicated in:

- Patients with unresolved serious Neisseria meningitidis infection.

- Patients who are not currently vaccinated against Neisseria meningitidis, unless the risks of delaying Soliris treatment outweigh the risk of developing a meningococcal infection.

Please see accompanying full Prescribing Information and Medication Guide for Soliris. Please see Important Safety Information on pages 16-19.