



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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## Soliris (*eculizumab*)

An overview of Soliris and why it is authorised in the EU

### What is Soliris and what is it used for?

Soliris is a medicine used to treat adults and children with paroxysmal nocturnal haemoglobinuria (PNH) and atypical haemolytic uraemic syndrome (aHUS).

These are life-threatening genetic diseases that cause the breakdown of red blood cells resulting in various medical complications. PNH results in anaemia (low red blood cell counts), thrombosis (blood clots in the blood vessels), pancytopenia (low counts of blood cells) and dark urine, while aHUS results in anaemia, thrombocytopenia (a decrease in the number of platelets, components that help the blood to clot) and kidney failure.

Soliris is used to treat adults and children aged 6 years and above with myasthenia gravis (a disease where the immune system attacks and damages muscle cells causing muscle weakness), in whom other medicines do not work (refractory generalised myasthenia gravis, refractory gMG) and who have a specific antibody in their body called AChR antibody.

Soliris is also used to treat adults with neuromyelitis optica spectrum disorder (NMOSD), a disease where the immune system damages nerve cells causing problems mostly with the optic (eye) nerve and the spinal cord (nerve tissue that runs from the base of the skull down the center of the back). It is used in patients who have an antibody called AQP4 and whose disease is relapsing (where the patient has attacks [relapses] between periods with no symptoms).

Soliris contains the active substance eculizumab.

These diseases are rare, and Soliris was designated an 'orphan medicine' (a medicine used in rare diseases). Further information on the orphan designations can be found on the European Medicines Agency's website ([PNH](#): 17 October 2003; [aHUS](#): 24 July 2009; [myasthenia gravis](#): 29 July 2014; [NMOSD](#): 24 April 2019).

### How is Soliris used?

The medicine can only be obtained with a prescription and must be given under the supervision of a doctor who has experience in the management of patients with kidney disorders and disorders affecting the nervous system or the blood.

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Soliris is given as an infusion (drip) into a vein and the recommended dose depends on what it is used for and, for patients under 18 years of age, on the bodyweight. Soliris is given weekly initially and then every two or three weeks.

Patients are monitored for any reactions during the infusion and for at least one hour afterwards. In case of any infusion-related reactions, the doctor may slow down or stop the infusion.

In some patients who receive plasma exchange (the removal, treatment and return of their own blood plasma, the liquid part of the blood) or infusion of plasma, additional doses of Soliris are required.

Soliris should be given for life unless the patient develops serious side effects. Treatment should also be stopped in patients with refractory gMG who do not respond to Soliris after 12 weeks.

For more information about using Soliris, see the package leaflet or contact your doctor or pharmacist.

## **How does Soliris work?**

The active substance in Soliris, eculizumab, is a monoclonal antibody (a type of protein) that has been designed to attach to the C5 complement protein, which is a part of the body's defence system called the 'complement system'.

In patients with PNH, aHUS, refractory gMG and NMOSD, the complement proteins are over-active and damage the patients' own cells. By blocking the C5 complement protein, eculizumab prevents complement proteins from damaging cells, thereby helping to relieve the symptoms of these diseases.

## **What benefits of Soliris have been shown in studies?**

### **PNH**

For PNH, Soliris was compared with placebo (a dummy treatment) in one main study involving 87 adults with PNH who were treated with at least four blood transfusions for anaemia in the previous year. The main measures of effectiveness were the effect of Soliris on blood levels of haemoglobin and the need for transfusions. Haemoglobin is the protein in red blood cells that carries oxygen around the body. In patients with PNH, the breakdown of red blood cells leads to a reduction in haemoglobin levels. Treatment with Soliris over 26 weeks led to stable haemoglobin levels in 49% of the patients (21 out of 43), without the need for transfusions of red blood cells. In comparison, none of the 44 patients receiving placebo had stable haemoglobin levels, and they needed an average of 10 transfusions.

In a study in 7 children with PNH who were treated with at least one transfusion in the previous two years, all patients received Soliris. Six out of seven patients did not need any transfusion of red blood cells, and haemoglobin levels improved during 12 weeks of treatment with Soliris.

A registry study of patients with PNH who had never had a blood transfusion looked at the blood levels of the enzyme lactate dehydrogenase (LDH). Levels of LDH rise as breakdown of red blood cells increases. The study found that treatment with Soliris for 6 months led to clinically meaningful reductions in levels of LDH, indicating reduced breakdown of red blood cells.

### **aHUS**

For aHUS, Soliris was studied in three main studies involving 67 patients. The first study involved 17 patients with aHUS who did not respond to or could not be treated with plasma exchange or infusion. Treatment with Soliris increased platelet counts in 82% of the patients, and platelet counts rose to

normal levels in 87% (13 out of 15 patients) who had low platelet counts at the start. In addition, 76% achieved haematological normalisation (levels of platelets and LDH within normal levels).

The second study, involving 20 patients with aHUS who were already receiving plasma exchange or infusion, resulted in 80% of the patients no longer requiring plasma exchange, infusion or dialysis and 90% of the patients achieving haematological normalisation after treatment with Soliris.

The third study involved 30 patients with aHUS who had received at least one dose of Soliris. Treatment increased platelet counts to normal levels in 83% of the patients, while the platelet count rose to normal levels in 77% (10 out of 13 patients) who initially had low platelet counts.

### **refractory gMG**

Soliris was compared with placebo in one main study involving 126 adults with myasthenia gravis who had previously received standard treatment which had failed. Treatment with Soliris improved patients' symptoms and their ability to undertake daily activities based on a standard scoring system. Soliris led to a reduction of 4.7 points on the scale whereas placebo led to a 2.8 point reduction after 26 weeks. A reduction in the score by 2 points indicates a clinically significant improvement of the patient's condition.

Similar results were seen in children. A main study in 11 children aged above 12 years of age showed that Soliris improved symptoms and patients' ability to undertake daily activities by 5.2 and 5.8 points after 12 weeks and 26 weeks of treatment respectively. Based on these results, the medicine is expected to also work in a similar way in children aged between 6 and 12 years of age.

### **NMOSD**

For NMOSD, Soliris was compared with placebo in one main study involving 143 adults with NMOSD whose disease was relapsing. The main measure of effectiveness was the time it took until a certain number of patients experienced a relapse. After around 22 months on average, 3% of patients treated with Soliris had experienced a relapse, whereas 43% of patients treated with placebo had already a relapse after around 9 months on average.

## **What are the risks associated with Soliris?**

For the full list of side effects and restrictions with Soliris, see the package leaflet.

The most common side effect with Soliris (which may affect more than 1 in 10 people) include headache. The most serious side effect, which may affect up to 1 in 100 people, is meningococcal sepsis (when bacteria and their toxins circulate in the blood and damage the organs).

Because of the increased risk of developing meningococcal sepsis, Soliris must not be given to people who have an infection caused by *Neisseria meningitides*; it must also not be given to patients who have not been vaccinated against this bacterium, unless they have the vaccination and take appropriate antibiotics to reduce the risk of infection for two weeks after vaccination.

## **Why is Soliris authorised in the EU?**

Soliris was shown to benefit patients with these rare diseases. The safety profile was similar for all the diseases and was considered acceptable. The European Medicines Agency decided that Soliris' benefits are greater than its risks and it can be authorised for use in the EU.

## **What measures are being taken to ensure the safe and effective use of Soliris?**

The company that markets Soliris will ensure that distribution of the medicine occurs only after checking that the patient has been vaccinated appropriately against *Neisseria meningitides*. The company will also provide prescribers and patients with information on the safety of the medicine, and will send reminders to prescribers and pharmacists to check if any further vaccination is needed for patients taking Soliris. Patients will also be given a card that explains the symptoms of certain types of infection, instructing patients to seek medical care immediately if they experience them.

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Soliris have also been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Soliris are continuously monitored. Side effects reported with Soliris are carefully evaluated and any necessary action taken to protect patients.

## **Other information about Soliris**

Soliris received a marketing authorisation valid throughout the EU on 20 June 2007.

Further information on Soliris can be found on the Agency's website:  
[ema.europa.eu/medicines/human/EPAR/Soliris](http://ema.europa.eu/medicines/human/EPAR/Soliris).

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