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Epysqli (*eculizumab*)

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

What is Epysqli and what is it used for?

Epysqli is a medicine used to treat adults and children with paroxysmal nocturnal haemoglobinuria (PNH), a disease in which excessive breakdown of red blood cells results in various medical complications, including anaemia (low levels of red blood cells).

Epysqli is a 'biosimilar medicine'. This means that Epysqli is highly similar to another biological medicine (the 'reference medicine') that is already authorised in the EU. The reference medicine for Epysqli is Soliris. For more information on biosimilar medicines, see here.

Epysqli contains the active substance eculizumab.

How is Epysqli used?

Epysqli can only be obtained with a prescription and must be given by a healthcare professional and under the supervision of a doctor familiar with blood diseases.

Epysqli is given as an infusion (drip) into a vein over 25 to 45 minutes (adults) or 1 to 4 hours (children) every week for the first 2 to 5 weeks and every 2 weeks thereafter. Patients are monitored for any reactions during the infusion and for at least one hour afterwards.

Patients who have no major side effects with the first infusions may be able to have their infusions given at home by a healthcare professional. Epysqli should be given for life unless the patient develops serious side effects.

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

How does Epysqli work?

The complement system is a set of proteins that is part of the immune system (the body's natural defences). In patients with PNH, the complement system is over-active and damages the patients' own blood cells.

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The active substance in Epysqli, eculizumab, is an antibody (a type of protein) designed to attach to the C5 protein of the complement system. By blocking C5, eculizumab prevents the complement system from damaging cells, thereby helping to relieve the symptoms of the disease.

What benefits of Epysqli have been shown in studies?

Laboratory studies comparing Epysqli with Soliris have shown that the active substance in Epysqli is highly similar to that in Soliris in terms of structure, purity and biological activity. Studies have also shown that giving Epysqli produces similar levels of the active substance in the body to giving Soliris.

In addition, a study in 50 patients with PNH compared the blood levels of the enzyme lactate dehydrogenase (LDH) after treatment with Epysqli and treatment with Soliris. Lower levels of LDH mean that the breakdown of red blood cells is reduced. The study found that after 6 months of treatment, average LDH levels with Epysqli were similar to those seen with Soliris (about 284 units per litre with Epysqli compared with 250 units per litre with Soliris).

Because Epysqli is a biosimilar medicine, the studies on effectiveness and safety of eculizumab carried out with Soliris do not all need to be repeated for Epysqli.

What are the risks associated with Epysqli?

The safety of Epysqli has been evaluated, and on the basis of all the studies carried out the side effects of the medicine are considered to be comparable to those of the reference medicine Soliris.

For the complete list of side effects and restrictions of Epysqli, see the package leaflet.

The most common side effect with Epysqli (which may affect more than 1 in 10 people) is headache and the most serious side effect is meningococcal sepsis (when bacteria infect the bloodstream, causing bleeding of the skin and organs).

Because of the increased risk of developing meningococcal sepsis, Epysqli must not be given to people who have an ongoing infection caused by *Neisseria meningitides.* It must also not be given to patients who are not currently vaccinated against this bacterium unless they have the vaccination and take appropriate antibiotics to reduce the risk of infection for 2 weeks after receiving it.

Why is Epysqli authorised in the EU?

The European Medicines Agency decided that, in accordance with EU requirements for biosimilar medicines, Epysqli has a highly similar structure, purity and biological activity to Soliris and is distributed in the body in the same way. In addition, a study in patients with PNH has shown that the safety and effectiveness of Epysqli is equivalent to that of Soliris in PNH.

All these data were considered sufficient to conclude that Epysqli will behave in the same way as Soliris in terms of effectiveness and safety in its authorised use in PNH. Therefore, the Agency's view was that, as for Soliris, the benefits of Epysqli outweigh the identified risks and it can be authorised for use in the EU.

What measures are being taken to ensure the safe and effective use of Epysqli?

The company that markets Epysqli will ensure that distribution of the medicine occurs only after checking that the patient has been vaccinated appropriately. The company will also provide prescribers

and patients with information on the safety of the medicine and will send reminders to prescribers or pharmacists to check the vaccination status of patients.

The company will provide prescribers and patients with a guide on the safety of the medicine. Patients will also be given a 'safety card' that explains the symptoms of certain types of infection, instructing them 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 Epysqli have also been included in the summary of product characteristics and the package leaflet.

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

Other information about Epysqli

Epysqli received a marketing authorisation valid throughout the EU on 26 May 2023.

Further information on Epysqli can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/epysqli.

This overview was last updated in 05-2023.