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Refusal of the marketing authorisation for Yartemlea (narsoplimab)

The European Medicines Agency has recommended the refusal of the marketing authorisation for Yartemlea, a medicine intended for the treatment of adults and children from two years of age with haematopoietic stem cell transplant (HSCT)-associated thrombotic microangiopathy.

The Agency issued its opinion on 25 June 2026. The company that applied for authorisation, Omeros Ireland Limited, may ask for re-examination of the opinion within 15 days of receiving the opinion.

What is Yartemlea and what was it intended to be used for?

Yartemlea was developed as a medicine for treating adults and children from two years of age with high-risk HSCT-associated thrombotic microangiopathy. Thrombotic microangiopathy is a serious and potentially life-threatening complication following HSCT, a procedure where the patient's bone marrow is replaced by stem cells from a donor to form new bone marrow that produces healthy cells. In some patients, treatments given just before and after HSCT can damage small blood vessels, leading to the formation of blood clots and organ damage.

Yartemlea contains the active substance narsoplimab and was to be given by infusion (drip) into a vein.

Yartemlea was designated an orphan medicine (a medicine used in rare diseases) on 22 November 2018 for use in people receiving haematopoietic stem cell transplantation. Further information on the orphan designation can be found on the Agency's website: https://www.ema.europa.eu/en/medicines/human/orphan-designations/eu-3-18-2067.

How does Yartemlea work?

The complement system is part of the immune system (the body's natural defences) and normally helps fight infections. In people who have had a haematopoietic stem cell transplant, the complement system may become activated following damages made to small blood vessels, leading to the formation of blood clots. This condition is called thrombotic microangiopathy.

The active substance in Yartemlea, narsoplimab, is a monoclonal antibody, a type of protein designed to attach to a target in the body called MASP-2. The protein MASP-2 is part of the complement system.

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By attaching to and blocking MASP-2, narsoplimab is expected to reduce damage to small blood vessels and the formation of blood clots in people who have had an HSCT.

What did the company present to support its application?

The company presented results from a main study involving 28 adults with thrombotic microangiopathy following HSCT who received Yartemlea. In this study, the medicine was not compared with a placebo (dummy treatment) or any other treatment. The main measure of effectiveness was the change in levels of platelets (components that help the blood to clot) and lactate dehydrogenase (a substance in the blood that can indicate tissue damage), both of which were used as signs of the disease. The study also looked at whether patients' organs, such as kidneys and lungs, were functioning better, and whether patients required blood transfusions.

The company also presented additional results from 27 adults and 16 children treated with the medicine as part of an expanded access program, which allows patients with serious or life-threatening diseases who have no other available treatment to receive a medicine before it is authorised.

Finally, the company compared how long patients given Yartemlea lived, with how long similar patients who were not given Yartemlea lived.

What were the main reasons for refusing the marketing authorisation?

The Agency considered that the company did not provide sufficient evidence of the effectiveness of Yartemlea.

The main study supporting the application did not compare Yartemlea with placebo or another treatment. Participants were also taking other medications in addition to Yartemlea. Because of this, it was not possible to determine whether the observed benefits were due to Yartemlea, other medicines taken or other reasons. There were also concerns about how the study was conducted, including changes made during the study, how effectiveness was measured and how the dose was chosen. These limitations made the results difficult to interpret and unsuitable for confirming the medicine's effectiveness. Studies published in the medical literature also showed that other treatments for thrombotic microangiopathy gave similar results to Yartemlea. The comparison of how long patients given Yartemlea lived, using data from the main study and expanded access program, with patients outside these studies who were not given Yartemlea could not reliably confirm the medicine's effects.

For use in children, there were not enough data to support the proposed dose and assess the effectiveness or safety. As the benefits of Yartemlea had not been shown in adults, the results could not be reliably extended to children.

The study results also did not confirm how the medicine is expected to work in this condition.

Because of these uncertainties, the Agency's opinion was that the benefits and risks of Yartemlea could not be established. Hence, the Agency recommended refusing marketing authorisation.

Does this refusal affect patients in clinical trials or compassionate use programmes?

The company informed the Agency that there are no consequences for patients in clinical trials or in compassionate use programmes with Yartemlea.

If you are in a clinical trial or compassionate use programme and need more information about your treatment, speak with your clinical trial doctor.