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Refusal of the marketing authorisation for Omblastys (omburtamab (^{131}I))

The European Medicines Agency has recommended the refusal of the marketing authorisation for Omblastys, a medicine intended for the treatment of neuroblastoma, a rare cancer that forms from immature nerve cells.

The Agency issued its opinion on 15 December 2022. Y-mAbs Therapeutics A/S, the company that applied for authorisation, may ask for re-examination of the opinion within 15 days of receiving the opinion.

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

Omblastys was intended for the treatment of neuroblastoma in patients whose disease had spread to the brain, spinal cord or the leptomeninges (thin layers of tissue that cover and protect the brain and spinal cord) and which was previously treated.

Omblastys is a radiopharmaceutical (a medicine with small amounts of a radioactive substance) that contains the active substance omburtamab (^{131}I) and was to be available as a solution for infusion (drip) that is given into existing fluid-filled space in the brain.

Omblastys was designated an 'orphan medicine' (a medicine used in rare diseases) on 27 February 2017 for the treatment of neuroblastoma. Further information on the orphan designation can be found on the Agency's website: ema.europa.eu/medicines/human/orphan-designations/eu3-17-1839.

How does Omblastys work?

The active substance in Omblastsys, omburtamab (^{131}I), is a monoclonal antibody (type of protein) designed to recognise and attach to a protein called CD276, which is present on the surface of neuroblastoma cells, but not normal cells. This monoclonal antibody is linked to radioactive iodine (^{131}I) which produces low-level radiation.

When the medicines attaches to CD276, radiation from ^{131}I is expected to damage the DNA within the cancer cells, resulting in the death of the cancer cells.

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What did the company present to support its application?

The company provided results from one main study conducted in 109 children with neuroblastoma whose disease had spread to either the brain or the spinal cord. Patients in the study were given Omblastys, and the results were compared with those of an external control group (other patients with neuroblastoma who were not in the study and had received other treatments).

The main measure of effectiveness was the proportion of patients who were still alive after 3 years.

What were the main reasons for refusing the marketing authorisation?

The Agency considered that it was not possible to conclude on the effectiveness of Omblastys. As the main study did not have a randomised comparator, it was not possible to determine a treatment effect. Moreover, it could not be ascertained that the patients in the external control group selected for reference by the company, had a similar underlying prognosis as those treated with Omblastys in the study.

Therefore, the Agency's opinion was that the balance of benefits and risks of Omblastys in the treatment of neuroblastoma could not be established. Hence, the Agency recommended refusing marketing authorisation.

Does this refusal affect patients in clinical trials?

The company informed the Agency that there are no consequences for patients in clinical trials with Omblastys.

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