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Outcome of assessment on use of Lutathera in treatment of gastro-entero-pancreatic neuroendocrine tumours in adolescents

The European Medicines Agency has finalised its assessment of an application to extend the use of Lutathera to adolescents aged 12 years and older with unresectable or metastatic, somatostatin receptor-positive gastro-entero-pancreatic neuroendocrine tumours (GEP-NETs). Although EMA did not recommend this use, it agreed that relevant data from the study submitted with the application be included in the medicine's product information so that healthcare professionals have access to up-to-date data on the effects of Lutathera in people with GEP-NETs.

What is Lutathera and what is it used for?

Lutathera is a cancer medicine used to treat adults with tumours in the gut known as GEP-NETs that are unresectable (cannot be removed by surgery) or metastatic (have spread to other parts of the body) and are not responding to treatment. It is used when the cancer cells have receptors (proteins) on their surface that bind to a hormone called somatostatin (somatostatin receptor-positive). Lutathera is a radiopharmaceutical (a medicine that emits a small amount of radioactivity).

Lutathera has been authorised in the EU since September 2017. It contains the active substance lutetium (^{177}Lu) oxodotreotide and is available as a solution to be given by infusion (drip) into a vein.

Further information on Lutathera's current uses can be found on the Agency's website: ema.europa.eu/en/medicines/human/EPAR/lutathera.

What change had the company applied for?

The company applied to extend the use of Lutathera to adolescents aged 12 years and older with unresectable or metastatic, somatostatin receptor-positive GEP-NETs.

Lutathera was designated an 'orphan medicine' (a medicine used in rare diseases) for GEP-NETs on 31 January 2008. Further information on the orphan designation can be found on the Agency's website: ema.europa.eu/medicines/human/orphan-designations/eu-3-07-523.

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How does Lutathera work?

The active substance in Lutathera, lutetium (^{177}Lu) oxodotreotide, is a somatostatin analogue (a man-made version of the hormone somatostatin) combined with lutetium (^{177}Lu), a component that emits a small amount of radioactivity. It works by attaching to somatostatin receptors, which are found in high numbers in some GEP-NETs. The radioactivity emitted by the medicine then kills the tumour cells it is attached to but has little effect on neighbouring cells. In adolescents aged 12 years and older with unresectable or metastatic, somatostatin receptor-positive GEP-NETs, Lutathera is expected to work in the same way as it does in adults with this condition.

What did the company present to support its application?

The company submitted data from a main study involving 4 adolescents aged 12 years and older with unresectable or metastatic, progressive (not responding to treatment) somatostatin receptor-positive GEP-NETs, where the cells are well-differentiated (meaning they resemble normal cells) and grow slowly (grade 1 (G1) or grade 2 (G2) GEP-NETs). The study also involved 7 adolescents aged 12 years and older with pheochromocytoma and paraganglioma, other rare neuroendocrine tumours. In this study, treatment with Lutathera was not compared with another treatment or placebo (a dummy treatment). The study mainly evaluated the amount of radiation absorbed by different organs (e.g. kidneys and bone marrow), as well as the safety and tolerability of Lutathera in adolescents.

What were EMA's conclusions?

EMA considered that, although the data available were very limited, the benefits of Lutathera in adolescents aged 12 years and older with unresectable or metastatic, progressive, well-differentiated (G1 and G2), somatostatin receptor-positive GEP-NETs might outweigh its risks. However, before the extension of use could be given a positive opinion, EMA noted that certain changes needed to be made to the prescribing information, along with an assessment of the possibility of carrying out a study to investigate long-term risks associated with radiation exposure. However, while the application was still under evaluation, the company decided not to further pursue the extension of Lutathera's use to adolescents.

Although Lutathera will therefore not be authorised for adolescents, the prescribing information will be updated to include relevant data, so that healthcare professionals have access to up-to-date data on the effects of Lutathera in adolescents with GEP-NETs.

Does this outcome affect patients in clinical trials?

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

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