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Xospata (*gilteritinib*)

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

What is Xospata and what is it used for?

Xospata is a cancer medicine used to treat adults with acute myeloid leukaemia (AML), a cancer of white blood cells.

Xospata is used when AML has either come back or has not improved following previous treatment and is only given to patients whose cancer cells have a particular change (mutation) in the gene for a protein called FLT3.

The active substance in Xospata is gilteritinib.

AML is rare, and Xospata was designated an 'orphan medicine' (a medicine used in rare diseases) on 17 January 2018. Further information on the orphan designation can be found here: ema.europa.eu/medicines/human/orphan-designations/eu3171961.

How is Xospata used?

Xospata can only be obtained with a prescription and treatment should be started and supervised by a doctor experienced in using cancer medicines.

Xospata is available as tablets (40 mg). The recommended starting dose is three tablets once daily and can be increased to 5 tablets once daily after 4 weeks depending on the patient's response. Treatment should continue for as long as the patient benefits from it. If certain side effects develop, the doctor may decide to reduce the dose or stop the treatment intermittently or entirely.

Before starting treatment, doctors will use a genetic test to confirm that the patient has an FLT3 mutation. The doctor will also carry out regular blood tests and check the patient's heart function before and during treatment.

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

How does Xospata work?

The active substance in Xospata, gilteritinib, blocks the action of enzymes known as tyrosine kinases, in particular a tyrosine kinase called FLT3 which normally controls growth and division of white blood

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cells. In patients who have an FLT3 mutation, FLT3 is overactive and stimulates the growth of too many white blood cells. By blocking FLT3, gilteritinib is expected to stop white blood cell growth and thus slow down the development of the cancer.

What benefits of Xospata have been shown in studies?

In a main study with 371 patients with AML who have an FLT3 mutation, and whose disease had come back or had not improved following previous treatment, Xospata prolonged patients' lives compared with patients receiving chemotherapy. Patients receiving Xospata lived on average 9.3 months compared with 5.6 months for those receiving various types of chemotherapy.

What are the risks associated with Xospata?

The most common side effects with Xospata (which may affect more than 1 in 10 people) are increased levels of some enzymes in the blood (creatine phosphokinase, alanine aminotransferase, aspartate aminotransferase, alkaline phosphatase), diarrhoea, tiredness, nausea (feeling sick), constipation, cough, peripheral oedema (swelling, especially of the ankles and feet), dyspnoea (shortness of breath), dizziness, hypotension (low blood pressure), pain in the extremities, weakness, joint pain and muscle pain.

The most common serious side effects (which may affect up to 1 in 10 people) are diarrhoea, increased levels of liver enzymes in the blood, dyspnoea and hypotension. Other serious side effects include a disorder related to white blood cells known as differentiation syndrome, a brain condition known as posterior reversible encephalopathy syndrome (PRES) and heart rhythm problems.

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

Why is Xospata authorised in the EU?

Patients with AML who have an FLT3 mutation, and whose disease has come back or has not improved following previous treatment, have a poor prognosis and treatment options are limited. Xospata was shown to prolong patients' lives in comparison to other cancer therapies. Side effects with Xospata are those expected of this class of medicines and considered manageable. The European Medicines Agency therefore decided that Xospata's 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 Xospata?

The company that markets Xospata will provide educational materials to healthcare professionals and patients about the signs and symptoms of differentiation syndrome. Patients will also receive a patient card with information on when to seek medical advice if differentiation syndrome is suspected.

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

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

Other information about Xospata

Xospata received a marketing authorisation valid throughout the EU on 24 October 2019.

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

This overview was last updated in 10-2019.