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Scemblix (*asciminib*)

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

What is Scemblix and what is it used for?

Scemblix is a cancer medicine. It is used to treat chronic myeloid leukaemia (CML), a cancer of the white blood cells, in the 'chronic' phase (this is when the condition is developing slowly and the patient has few or no symptoms). The medicine can be used in adult patients whose cancer is 'Philadelphia-chromosome positive' (Ph+). Ph+ means that two of the patient's chromosomes have rearranged themselves and formed a special chromosome called the Philadelphia chromosome. This chromosome produces an enzyme, BCR::ABL1 kinase, that leads to the development of leukaemia.

Scemblix is used in patients who have already been treated with two or more cancer medicines called tyrosine kinase inhibitors.

CML is rare, and Scemblix was designated an 'orphan medicine' (a medicine used in rare diseases) on 24 March 2020. Further information on the orphan designation can be found here: https://www.ema.europa.eu/documents/orphan-designation/eu/3/20/2261-public-summary-opinion-orphan-designation-asciminib-treatment-chronic-myeloid-leukaemia_en.pdf

Scemblix contains the active substance asciminib.

How is Scemblix used?

Scemblix can only be obtained with a prescription and treatment must be started by a doctor who is experienced in the diagnosis and treatment of leukaemia.

The medicine is available as tablets to be taken by mouth twice a day. The doctor may interrupt treatment and reduce the dose if certain side effects occur. Treatment may be stopped if a patient cannot tolerate treatment with the reduced dose.

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

How does Scemblix work?

The active substance in Scemblix, asciminib, is a tyrosine kinase inhibitor (TKI), meaning that it blocks enzymes known as tyrosine kinases. In Ph+ CML, the body produces large numbers of abnormal white

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blood cells. Scemblix specifically blocks the action of the BCR::ABL1 tyrosine kinase that is produced by these cells, and this stops their division and growth.

What benefits of Scemblix have been shown in studies?

The benefits of Scemblix were evaluated in a main study in 233 adults with Ph+ CML in the chronic phase who were previously treated with two or more tyrosine kinase inhibitors. In this study, Scemblix was more effective than bosutinib (another tyrosine kinase inhibitor): after 24 weeks of treatment, 25% (40 out of 157) of patients given Scemblix had a major molecular response (meaning that the number of cells with the *BCR::ABL1* gene had decreased to 1,000 times below the standardised baseline), compared with 13% (10 out of 76) of patients given bosutinib. After 96 weeks of treatment, 38% (59 out of 157) of patients given Scemblix and 16% (12 out of 76) of patients given bosutinib had a major molecular response.

What are the risks associated with Scemblix?

The most common side effects with Scemblix (which may affect more than 2 in 10 people) are pain in the muscles, joints and bones, upper respiratory tract (nose and throat) infections, thrombocytopenia (low levels of blood platelets), tiredness, headache, increased levels of pancreatic enzymes, abdominal pain, diarrhoea and nausea (feeling sick).

The most common serious side effects with Scemblix (which may affect up to 1 in 10 people) are pleural effusion (fluid around the lungs), lower respiratory tract infections (infections of the lungs, such as bronchitis or pneumonia), thrombocytopenia, fever, pancreatitis (inflammation of the pancreas), chest pain (not related to the heart) and vomiting.

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

Why is Scemblix authorised in the EU?

Scemblix has been shown to be more effective than another tyrosine kinase inhibitor at reducing the number of cells with the *BCR::ABL1* gene in patients who had already received at least two previous tyrosine kinase inhibitors. In terms of safety, the side effects with Scemblix are similar to those seen with this class of medicines and are considered manageable. The European Medicines Agency therefore decided that the benefits of Scemblix are greater than its risks and that it can be authorised for use in the EU.

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

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

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

Other information about Scemblix

Scemblix received a marketing authorisation valid throughout the EU on 25 August 2022.

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

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