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Scemblix (*asciminib*)

A plain-language 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 cancer is developing slowly and the patient has few or no symptoms).

It is used in adults 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 (protein) known as BCR::ABL1 tyrosine kinase, that leads to the development of leukaemia.

Scemblix is also for use in adults with Ph+ CML in the chronic phase whose cancer cells have a mutation (change) called *T315I* in the gene that produces the BCR::ABL1 protein and who are not benefiting from or cannot receive treatment with another cancer medicine called ponatinib.

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/en/medicines/human/orphan-designations/eu-3-20-2261

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 every day. The doctor may interrupt treatment or 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.

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How does Scemblix work?

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

Another study involved 405 adults with newly diagnosed Ph+ CML in the chronic phase who had not received prior treatment. In the study, patients were given either Scemblix or another tyrosine kinase inhibitor. After 48 weeks of treatment, around 68% (136 out of 201) of those given Scemblix had a major molecular response compared with around 49% (100 out of 204) of those given another tyrosine kinase inhibitor.

A third study involved 48 adults with Ph+ CML in the chronic phase whose cancer had the *T315I* mutation and who had previously had tyrosine kinase inhibitor treatment. Of these patients, 45 did not have a major molecular response at the start of the study, 26 of whom had previously been treated with ponatinib. After 24 weeks of treatment with Scemblix, around 31% (8 out of 26) of these patients had achieved a major molecular response. After 96 weeks of treatment, this figure was around 35% (9 out of 26). In this study, Scemblix was used at a 5-times higher dose than for people without the *T315I* mutation and was not compared with any other treatment.

Studies carried out with Scemblix are described in more detail in the medicine's assessment reports.

What are the side effects and restrictions with Scemblix?

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

The most common side effects with Scemblix (which may affect more than 2 in 10 people) include musculoskeletal pain (pain in the muscles, joints and bones), thrombocytopenia (low levels of blood platelets), tiredness, upper respiratory tract (nose and throat) infections, headache, neutropenia (low levels of neutrophils, a type of white blood cell), arthralgia (joint pain) and diarrhoea.

Other common side effects with Scemblix when used in people with the *T315I* mutation include increased levels of pancreatic and liver enzymes (which may suggest abnormal function of the pancreas or liver), nausea (feeling sick), vomiting and cough.

Some side effects with Scemblix can be serious. The most frequent (which may affect up to 1 in 10 people) include pleural effusion (fluid around the lungs), lower respiratory tract infections (infections of the lungs, such as bronchitis or pneumonia), thrombocytopenia, pancreatitis (inflammation of the pancreas) and fever.

In people with the *T315I* mutation, the most frequent serious side effects (which may affect up to 1 in 10 people) include abdominal (belly) pain, vomiting, lower respiratory tract infections, constipation, headache, non-cardiac (unrelated to the heart) chest pain and pleural effusion.

Why is Scemblix authorised in the EU?

Scemblix has been shown to be more effective than other tyrosine kinase inhibitors at reducing the number of cells with the *BCR::ABL1* gene in adults with Ph+ CML in the chronic phase. It has also been found effective at a higher dose in people with chronic-phase Ph+ CML, whose cancer cells have a mutation called *T315I*. However, the study involving people with the *T315I* mutation only included a small number of patients and did not compare Scemblix with ponatinib, which was the approved treatment at the time of the study for patients with this mutation. It was therefore not possible to conclude on the effectiveness of Scemblix in patients with the *T315I* mutation who had not been previously treated with ponatinib.

In terms of safety, the side effects with Scemblix in people without the *T315I* mutation are similar to those seen with other medicines of this class of medicines and are considered manageable. For patients with this mutation, who require a much higher dose of Scemblix than those without this mutation, there are limited safety data. However, the uncertainties about a potential increased risk of side effects with this higher dose are described in the product information, along with advice to healthcare professionals to consider close monitoring of these patients.

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?

The company that markets Scemblix will carry out a study to evaluate the long-term effectiveness and safety of Scemblix in patients newly diagnosed with Ph+ CML in the chronic phase.

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, including the package leaflet and assessment report, can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/scemblix.

For information about the availability of this medicine in your country, contact your national competent authority.

This overview was last updated in 04-2026.