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Xromi (*hydroxycarbamide*)

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

What is Xromi and what is it used for?

Xromi is a medicine used in adults, adolescents and children over nine months of age who have sickle cell disease, a genetic disease where the red blood cells become rigid and sticky, and change from being disc-shaped to being crescent-shaped (like a sickle). Xromi is used to prevent so-called vaso-occlusive complications – problems that happen when blood vessels become blocked by the abnormal red blood cells, restricting the flow of blood to parts of the body.

Xromi contains the active substance hydroxycarbamide and is a 'hybrid medicine'. This means that it is similar to a 'reference medicine' containing the same active substance, but there are certain differences between the two: Xromi is given as a liquid to be swallowed rather than as capsules and is authorised for different uses. The reference medicine for Xromi is Hydrea.

How is Xromi used?

Xromi can only be obtained with a prescription and treatment must be supervised by a healthcare professional experienced in the management of sickle cell disease.

Xromi is available as a liquid to be swallowed; the dose is based on the patient's body weight. Patients should drink water after a dose of Xromi to ensure the full dose reaches the stomach.

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

How does Xromi work?

The active substance in Xromi, hydroxycarbamide, blocks the growth and reproduction of some cells, such as blood cells. Although the way that it works in sickle cell disease is not fully understood, hydroxycarbamide can reduce the numbers of cells that are circulating in the blood, as well as prevent red blood cells changing shape in patients with this disease. This reduces the risk of blood vessels becoming blocked.

Hydroxycarbamide, which used to be known as hydroxyurea, has been used for many years to treat sickle cell disease, and has been authorised in EU countries for several decades as Hydrea for use in some types of cancer.

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What benefits of Xromi have been shown in studies?

Because hydroxycarbamide is a well-established substance that has been used for many years in the EU, the company provided information from the published literature on the benefits and risks of hydroxycarbamide in its approved uses. This included information from 4 main studies in sickle cell disease, including 3 studies involving 384 adults and children in whom hydroxycarbamide was shown to substantially reduce the risk of severe, painful blockages of blood supply (called vaso-occlusive crises) compared with a dummy treatment, and a fourth study involving 121 children that showed hydroxycarbamide to be at least as effective as a standard treatment using blood transfusions in reducing blood vessel damage in the brain and the risk of stroke.

Data from an additional study suggest that Xromi is expected to work in the same way in children from 9 months of age as it does in older children; further data from a published study suggest that the benefits and safety of hydroxycarbamide in children from 9 months of age are similar to those in older children.

The company also provided information from a variety of supportive studies. As for every medicine, this included studies showing that the medicine was of acceptable quality. It also carried out a study that showed Xromi is bioequivalent to the reference medicine, Hydrea. Two medicines are bioequivalent when they produce the same levels of the active substance in the body.

What are the risks associated with Xromi?

For the full list of side effects and restriction of Xromi, see the package leaflet.

The most common side effects with Xromi (which may affect more than 1 in 10 people) include bone marrow suppression (reduced ability to produce blood cells) and reduced fertility in men due to oligospermia (low sperm counts) or azoospermia (absence of sperm).

Xromi must not be used in people who have severe problems with their kidneys or liver, or who have dangerously low blood cell counts. It must not be used in pregnancy, and breast-feeding must be stopped while taking the medicine. Xromi must also not be used in patients taking medicines to treat HIV infection.

Why is Xromi authorised in the EU?

The company provided updated information showing the benefits of hydroxcarbamide in preventing complications of sickle cell disease in patients over 9 months of age. The safety issues with hydroxycarbamide are well understood, and Xromi has been shown to be bioequivalent to an authorised hydroxycarbamide medicine. Therefore, the European Medicines Agency decided that Xromi’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 Xromi?

The company that markets Xromi will provide educational materials for doctors and patients about the correct use of the medicine and how to minimise its risks.

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

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

Other information about Xromi

Xromi received a marketing authorisation valid throughout the EU on 1 July 2019.

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

This overview was last updated in 03-2024.