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SCIENCE MEDICINES HEALTH

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EPAR summary for the public

Besponsa

inotuzumab ozogamicin

This is a summary of the European public assessment report (EPAR) for Besponsa. It explains how the Agency assessed the medicine to recommend its authorisation in the EU and its conditions of use. It is not intended to provide practical advice on how to use Besponsa.

For practical information about using Besponsa, patients should read the package leaflet or contact their doctor or pharmacist.

What is Besponsa and what is it used for?

Besponsa is a cancer medicine used to treat a type of blood cancer which affects B cells (a type of white blood cells) called B-cell precursor acute lymphoblastic leukaemia (ALL). Besponsa is used on its own in adults whose cancer has come back or did not respond to previous treatment.

Besponsa is only used in patients with 'CD22-positive B-cell precursor ALL'. This means that patients' have a particular protein (CD22) on the surface of their white blood cells. In patients who have a type of chromosome known as Philadelphia-chromosome, treatment with a cancer medicine called a tyrosine kinase inhibitor should have been tried before starting Besponsa.

Because the number of patients with B-cell precursor ALL is low, the disease is considered 'rare', and Besponsa was designated an 'orphan medicine' (a medicine used in rare diseases) on 7 June 2013.

Besponsa contains the active substance inotuzumab ozogamicin.

How is Besponsa used?

Besponsa is given as an infusion (drip) into a vein lasting for at least one hour. The infusions are given on days 1, 8 and 15 of a 3 or 4 week treatment cycle. The doctor may interrupt treatment or reduce the dose, if the patient develops certain serious side effects.

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Patients in whom Besponsa works well should receive 2 or 3 cycles, after which they can have a stem cell transplant to replace their bone marrow, the only curative treatment. Patients whose treatment works well, but who are not going to receive a stem cell transplant, may receive up to a maximum of 6 cycles of treatment. In patients who do not respond to treatment, Besponsa should be stopped after 3 cycles.

Besponsa can only be obtained with a prescription, and treatment should be given under the supervision of a doctor who has experience in the use of cancer treatments.

For further information, see the package leaflet.

How does Besponsa work?

The active substance in Besponsa, inotuzumab ozogamicin, is a monoclonal antibody (a type of protein) that has been linked to a small molecule, N-acetyl-gamma-calicheamicin dimethylhydrazide. The monoclonal antibody has been designed to recognise and attach to CD22 on the cancerous B cells. Once attached, the medicine is taken up by the cell where calicheamicin becomes active, causing breaks in the cell's DNA and thereby killing the cancer cell.

What benefits of Besponsa have been shown in studies?

Besponsa was shown to be more effective than other chemotherapy (medicines to treat cancer) in one main study involving 326 adults with CD22-positive B-cell precursor ALL, which had come back or had not responded to previous treatment. The main measure of effectiveness was response to treatment. Patients were considered to have responded if they had no remaining cancerous B cells in their blood and bone marrow after treatment.

An analysis of the first 218 patients treated showed that after at least 2 cycles of treatment, 81% (88 out of 109) of patients receiving Besponsa responded to treatment compared with 29% (32 out of 109) of patients receiving other chemotherapy. Patients who responded to treatment could proceed to have a stem cell transplant.

What are the risks associated with Besponsa?

The most common side effects with Besponsa (which may affect more than 1 in 5 people) are thrombocytopenia (low blood platelet counts), neutropenia and leucopenia (low white blood cell counts), infection, anaemia (low red blood cell counts), tiredness, haemorrhage (bleeding), fever, nausea (feeling sick), headache, febrile neutropenia (low white cell count with fever), abdominal pain (stomach ache), increased levels of liver enzymes called transaminases and gamma-glutamyltransferase, and hyperbilirubinaemia (high blood levels of bilirubin, a breakdown product of red blood cells).

The most serious side effects are infection, febrile neutropenia, haemorrhage, abdominal pain, fever, tiredness and veno-occlusive liver disease/sinusoidal obstruction syndrome (VOD/SOS, a serious liver disease).

Besponsa must not be used in patients who have VOD/SOS or have had severe VOD/SOS or have other serious liver diseases.

For the full list of all side effects and restrictions reported with Besponsa, see the package leaflet.

Why is Besponsa approved?

The Agency's Committee for Medicinal Products for Human Use (CHMP) decided that Besponsa's benefits are greater than its risks and recommended that it be approved for use in the EU.

The CHMP considered that although there have been recent advances in the treatment of B-cell precursor ALL, treatment options for patients remain limited. The main study showed that Besponsa was better than other commonly used chemotherapy medicines at inducing a response in patients and allowing them to have a curative stem cell transplant.

With regard to safety, the side effects with Besponsa are similar to those of other chemotherapy medicines and can usually be managed by dose reduction or treatment interruption.

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

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

Other information about Besponsa

The European Commission granted a marketing authorisation valid throughout the European Union for Besponsa on 29 June 2017.

The full EPAR for Besponsa can be found on the Agency's website: [ema.europa.eu/Find/medicine/Human medicines/European public assessment reports](http://ema.europa.eu/Find/medicine/Human%20medicines/European%20public%20assessment%20reports). For more information about treatment with Besponsa, read the package leaflet (also part of the EPAR) or contact your doctor or pharmacist.

The summary of the opinion of the Committee for Orphan Medicinal Products for Besponsa can be found on the Agency's website: [ema.europa.eu/Find medicine/Human medicines/Rare disease designation](http://ema.europa.eu/Find/medicine/Human%20medicines/Rare%20disease%20designation).

This summary was last updated in 06-2017.