EMADOC-1829012207-48119
EMEA/H/C/004119

Besponsa (*inotuzumab ozogamicin*)

A plain-language overview of Besponsa and why it is authorised in the EU

What is Besponsa and what is it used for?

Besponsa is a cancer medicine used to treat a type of blood cancer that 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 and children aged 1 year and older 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 adults 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.

In children aged 1 year and older, Besponsa can be used in any of the following situations:

- the cancer has come back for the first time following an allogeneic haematopoietic stem cell transplantation (a transplantation of stem cells from a donor);
- the cancer has come back for the first time and the disease is considered to be at very high risk;
- the cancer has come back a second time or more;
- the cancer has not responded to previous treatments.

In children who have the Philadelphia chromosome, treatment with medicines that target the BCR-ABL protein must already have been tried and be no longer suitable 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. Further information on the orphan designation can be found on the EMA website.

Besponsa contains the active substance inotuzumab ozogamicin.

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

How is Besponsa used?

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 and in an environment where resuscitation facilities are available, so that patients who develop certain serious side effects can be treated immediately.

Besponsa is given as an infusion (drip) into a vein lasting for at least one hour. In children who weigh less than 35 kg, the infusion time should be increased to 1.5 hours to reduce the risk of certain side effects. 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.

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, which is 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.

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

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 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 that 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.

Another main study involved 28 children aged 1 year and older 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, with or without full recovery of blood counts. About 79% (22 out of 28) of children achieved a response after 1 cycle of treatment with Besponsa. Eighteen (64%) children proceeded to have a stem cell transplant.

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

What are the side effects and restrictions associated with Besponsa?

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

The most common side effects with Besponsa in adults (which may affect more than 1 in 5 people) include 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 (belly) pain, 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 common side effects with Besponsa in children (which may affect more than 3 in 10 people) include thrombocytopenia, fever, anaemia, vomiting, neutropenia, infections, haemorrhage, leucopenia and nausea.

Some side effects can be serious. The most frequent in adults (which may affect more than 2 in 100 people) include infection, febrile neutropenia, haemorrhage, abdominal pain, fever, tiredness and veno-occlusive liver disease/sinusoidal obstruction syndrome (VOD/SOS, a serious liver disease). The most frequent in children (which may affect more than 2 in 100 people) include febrile neutropenia, VOD and infection.

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

Why is Besponsa authorised in the EU?

Besponsa was shown to be more effective than other commonly used chemotherapy medicines at inducing a response in adults with B-cell precursor ALL and allowing them to have a curative stem cell transplant. It was also shown to be effective in children aged 1 year and older with B-cell precursor ALL. With regard to safety, the side effects with Besponsa are similar to those of other chemotherapy medicines and can usually be managed by reducing the dose or suspending treatment.

The European Medicines Agency therefore decided that Besponsa'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 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.

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

Other information about Besponsa

Besponsa received a marketing authorisation valid throughout the EU on 28 June 2017.

Further information on Besponsa, including the package leaflet and assessment reports, can be found on the Agency's website: ema.europa.eu/en/medicines/human/EPAR/besponsa.

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

This overview was last updated in 04-2026.