Decision explained

Medicine: dinutuximab beta (brand name: Qarziba®) for high risk neuroblastoma

EUSA Pharma (UK) Limited

What is dinutuximab beta used for?

Dinutuximab beta is used to treat high risk neuroblastoma in patients aged 12 months and above. Neuroblastoma is a very rare cancer of nerve cells, it mainly develops in infants or young children and the survival rate is low.

Dinutuximab beta is used to treat two different groups of high risk neuroblastoma patients:

- those who have shown some improvement after chemotherapy and have subsequently received treatment to replace their bone marrow with new healthy blood producing cells (myeloablative therapy and stem cell transplantation);
- those whose neuroblastoma has not improved with other treatments or has come back after previous treatment.

If the neuroblastoma has come back and is getting worse, the patient's condition should be stabilised before dinutuximab beta is given.

In patients whose neuroblastoma has come back or who have not had a complete response to initial treatment, dinutuximab beta should be given with another medicine called interleukin-2.

How does dinutuximab beta work?

Dinutuximab beta attaches to a structure called GD2 which is present at high levels on the surface of neuroblastoma cancer cells but not normal cells. By attaching to GD2, dinutuximab beta marks the neuroblastoma cell as a target for the body's immune system. The immune system then attacks and kills the cancer cells.

What has SMC said?

SMC has accepted dinutuximab beta for the treatment of high risk neuroblastoma in patients aged 12 months and older, as described above.



Why has SMC said this?

SMC looks at how well new medicines work compared with current treatments available in Scotland and in relation to how much they will cost to buy and administer (for example, if the medicine has to be given at a clinic or side effects have to be monitored).

SMC carefully considers every new medicine to make sure it benefits patients and is considered to be an acceptable use of the limited resources in NHSScotland. When SMC assesses a medicine it takes account of the needs of all patients in NHSScotland, not just those who may be treated with the medicine under consideration.

To do this SMC consider the following:

- Clinical trial and economic evidence from the company that makes the medicine.
- Advice from healthcare professionals about any benefits of the new medicine compared to current treatment, along with how the new medicine is likely to be used.
- Information from patient groups about the potential impact of the medicine on patients and carers.

After careful consideration, applying extra flexibility because dinutuximab beta is a medicine for a very rare condition, and after the company applied a confidential discount to the cost of the medicine SMC was able to accept it a possible treatment within NHSScotland.

What does SMC's decision mean for me?

If your healthcare professional thinks that dinutuximab beta, for use as described above is the right medicine for you or your child, you or they should be able to have the treatment on the NHS in Scotland. For further information see:



Medicines in Scotland: What's the right treatment for me? www.healthcareimprovementscotland.org/medicinesbooklet.aspx

More information

The organisations below can provide more information and support for the families of children with neuroblastoma. SMC is not responsible for the content of any information provided by external organisations.

Neuroblastoma UK



https://www.neuroblastoma.org.uk 020 8940 4353



Solving Kids' Cancer (Europe)



https://solvingkidscancer.org.uk



(C) 020 7284 0800

You can find out more about dinutuximab beta in the European public assessment report (EPAR) summary for the public by searching for the medicine name on the European Medicines Agency (EMA) website.



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