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Public summary of opinion on orphan designation

Aspacytarabine for the treatment of acute myeloid leukaemia

On 9 December 2020, orphan designation EU/3/20/2371 was granted by the European Commission to Granzer Regulatory Consulting & Services, Germany, for aspacytarabine for the treatment of acute myeloid leukaemia.

What is acute myeloid leukaemia?

Acute myeloid leukaemia (AML) is a cancer of the white blood cells (cells that fight infection). In patients with AML, the bone marrow (the spongy tissue inside the large bones, where blood cells are produced) produces abnormal, immature white blood cells. These abnormal cells quickly build up in large numbers in the bone marrow and are found in the blood.

AML is a long-term debilitating and life-threatening disease because the abnormal immune cells take the place of the normal blood cells, causing bleeding episodes, blood clots and reduced ability to fight infections.

What is the estimated number of patients affected by the condition?

At the time of designation, acute myeloid leukaemia affected approximately 1.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 67,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

Treatment for AML depends on several factors, including the extent of the disease, whether it has been treated before, and the patient's age, symptoms and general state of health. At the time of designation, the main treatments for AML were chemotherapy (medicines to treat cancer) and haematopoietic (blood) stem-cell transplantation (a procedure where the patient's bone marrow is cleared of cells and replaced by stem cells to form new bone marrow that produces healthy blood cells).

*For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with AML. Preliminary results from early studies showed that some patients with acute myeloid leukaemia who had no other treatment options had no sign of the disease after receiving the medicine. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Aspacytarabine is a 'prodrug' (a medicine that is converted to an active form in the body) of cytarabine. After it is given to the patient, it is broken down into cytarabine. Cytarabine interferes with the enzymes involved in the production of new DNA, slowing down the growth and division of cancer cells, and eventually killing them.

What is the stage of development of this medicine?

The effects of aspacytarabine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with aspacytarabine in patients with acute myeloid leukaemia were ongoing.

At the time of submission, aspacytarabine was not authorised anywhere in the EU for the treatment of acute myeloid leukaemia. Orphan designation of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 5 November 2020, recommending the granting of this designation.

Opinions on orphan medicinal product designations are based on the following three criteria:

- the seriousness of the condition;
- the existence of alternative methods of diagnosis, prevention or treatment;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

For more information

Contact details of the current sponsor for this orphan designation can be found on EMA website.

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.