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Public summary of opinion on orphan designation

Zanidatamab for the treatment of gastric cancer

On 13 November 2020, orphan designation EU/3/20/2353 was granted by the European Commission to Voisin Consulting S.A.R.L., France, for zanidatamab (also known as ZW25) for the treatment of gastric cancer.

What is gastric cancer?

Gastric cancer is a cancer that starts in the stomach. It is often detected late because early signs of the disease are the same as those of less serious stomach conditions (such as heartburn, gas and excessive belching). At a later stage, gastric cancer causes unexplained weight loss, loss of appetite and general decline in health. Bleeding can occur, leading to anaemia (low red blood cell counts). Men are about twice as likely to develop the disease as women.

Gastric cancer is a serious and life-threatening illness that is associated with shortened life expectancy.

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

At the time of designation, gastric cancer affected approximately 2.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 125,000 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?

At the time of designation, some patients with gastric cancer were treated with surgery to remove part or the whole of the stomach. Chemotherapy (medicines to treat cancer) was generally used after surgery or on its own if surgery was not possible or the disease had spread to other parts of the body. Several medicines were authorised in the EU for use in gastric cancer, such as capecitabine, cisplatin, docetaxel, doxorubicin, epirubicin, fluorouracil, mitomycin, ramucirumab and trastuzumab. They were often used in combination with one another.

*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 the medicine might be of significant benefit for patients with gastric cancer. Early studies have found that the medicine may be beneficial for patients in whom trastuzumab, chemotherapy and ramucirumab did not work. 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?

In around one fifth of patients with gastric cancer the cancer cells produce on their surface large quantities of a protein called HER2. Zanidatamab is a monoclonal antibody (a type of protein) designed to recognise and attach to HER2. By attaching to HER2, the medicine activates cells of the immune system, which then kill the cancer cells. Zanidatamab also stops HER2 producing signals that cause the tumour cells to grow. These actions are expected to slow down the progress of the disease.

What is the stage of development of this medicine?

The effects of zanidatamab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with gastric cancer were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for the treatment of gastric cancer. 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 8 October 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.