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Public summary of opinion on orphan designation

Anti-(pancreatic adenocarcinoma upregulated factor) IgG1 humanised monoclonal antibody for the treatment of pancreatic cancer

On 13 November 2020, orphan designation EU/3/20/2355 was granted by the European Commission to Prestige Biopharma Belgium, Belgium, for anti-(pancreatic adenocarcinoma upregulated factor) IgG1 humanised monoclonal antibody (also known as PBP1510) for the treatment of pancreatic cancer.

What is pancreatic cancer?

Pancreatic cancer is a cancer of the pancreas, a small organ that lies behind the stomach. The pancreas has two functions: to produce a fluid that helps with the digestion of food, and to produce hormones such as insulin. Due to the absence of symptoms in the early stages of pancreatic cancer, most patients are diagnosed when the cancer has spread nearby or to other parts of the body.

Pancreatic cancer is a severe and life-threatening disease that can shorten life expectancy.

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

At the time of designation, pancreatic cancer affected approximately 2.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 135,000 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, several medicines were authorised in the EU for treating pancreatic cancer. The choice of treatment depended on several factors, including how far the disease had advanced. Treatments included surgery, radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with pancreatic cancer because laboratory studies suggest it may improve results

* 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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when added to standard treatment. 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?

The medicine is a monoclonal antibody (a type of protein) that attaches to a specific target called pancreatic adenocarcinoma up-regulated factor (PAUF). PAUF is a substance produced in large amounts by pancreatic cancer cells, but not by ordinary cells in the pancreas, and is thought to play a role in the growth and spread of the cancer. By attaching to PAUF, the medicine is expected to block its action, slowing the progression of the cancer.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with this medicine in patients with pancreatic cancer had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for the treatment of pancreatic cancer. Orphan designation had been granted in the United States for treatment of 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.