European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

Document Date: London, 18 August 2009
Doc.Ref.: EMEA/COMP/1194/03 Rev.2

Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of cytochrome P450 isoform 2B1 gene transfected human embryonic kidney 293 cells encapsulated in polymeric cellulose sulphate for the treatment of pancreatic cancer in combination with ifosfamide

On 30 June 2003, orphan designation (EU/3/03/149) was granted by the European Commission to FSG-Biotechnologie GmbH, Austrianova, Austria, for cytochrome P450 isoform 2B1 gene transfected human embryonic kidney 293 cells encapsulated in polymeric cellulose sulphate for the treatment of pancreatic cancer in combination with ifosfamide.
FSG Biotechnologie Austrianova GmbH changed its name to Austrianova Biotechnology GmbH in 2005.
The sponsorship was transferred to Ziel Biopharma Limited, Ireland, in December 2008.

What is pancreatic cancer?
Cancer that begins in the pancreas is called pancreatic cancer. The pancreas is a small organ that lies behind the stomach and in front of the spine. The pancreas has two main functions in the body. It makes juices that help digest (break down) food. It produces hormones, such as insulin, that help to control blood sugar levels. About 95% of pancreatic cancers begin in the cells that make digestive juices. These cancers of the pancreas are called adenocarcinomas. Pancreatic cancer is life-threatening.

What is the estimated number of patients affected by the condition?
At the time of designation, pancreatic cancer affected approximately 1 people in 10,000 per year in the European Union (EU)*. This is equivalent to a total of around 38,000 people per year, which was considered to be below the threshold for orphan designation. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?
The choice of the treatment of pancreatic cancer depends on several factors, including the stage of the disease. Treatments may include surgery, radiation therapy (using high-dose x-rays or other high-energy rays to kill cancer cells), and chemotherapy (using drugs to kill cancer cells). Medicinal products have been authorised for use in pancreatic cancer in the European Union. This orphan medicinal product might be of potential significant benefit for patients affected by the condition as it might offer a new way to kill the cancer cells. This assumption remains to be proven. This will be necessary to maintain the orphan status.

*Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition.

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How is this medicine expected to work?
This orphan medicinal product is made of cells whose genetic material has been modified by adding a gene. This gene allows the cells to be very efficient in activating an anti-cancer agent called ifosfamide. The active ifosfamide is then able to kill tumour cells. After having been genetically modified, the cells are packed into capsules, and the product is administered in a vessel near to the cancer. Polymeric cellulose sulphate is a material that is used so that the capsules get trapped into the small vessels of the tumour. Once the cells are in place, the agent ifosfamide is given. In this way, it is expected that the cells will be able to produce high levels of active ifosfamide near the tumour where it is most needed for killing the cancer cells.

What is the stage of development of this medicine?
At the time of submission of the application for orphan designation, clinical trials in patients with pancreatic cancer were ongoing.

Cytochrome P450 isoform 2B1 gene transfected human embryonic kidney 293 cells encapsulated in polymeric cellulose sulphate was not marketed anywhere worldwide for pancreatic cancer or designated as orphan medicinal product elsewhere for this condition, at the time of submission of the application to the EMEA.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 8 May 2003 a positive opinion recommending the grant of the above-mentioned 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 Community) 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.

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