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EMA/COMP/166535/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Diaspirin cross-linked haemoglobin for the treatment of oesophageal cancer

On 21 March 2016, orphan designation (EU/3/16/1628) was granted by the European Commission to New B Innovation (UK) Limited, United Kingdom, for diaspirin cross-linked haemoglobin (also called YQ23) for the treatment of oesophageal cancer.

What is oesophageal cancer?

Oesophageal cancer is cancer of the oesophagus (the tube that connects the mouth to the stomach). It usually starts in the cells lining the oesophagus, and can spread to other parts of the body.

Oesophageal cancer is often detected late because in the early stage of the disease patients do not have noticeable symptoms. The cancer is usually diagnosed in people aged over 60 years, and men are more likely to develop the disease than women.

Oesophageal cancer is a life-threatening disease because of its serious complications such as dysphagia (difficulty swallowing), bleeding and weight loss that lead to poor overall survival.

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

At the time of designation, oesophageal cancer affected approximately 0.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 41,000 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?

The choice of treatment for oesophageal cancer depends on where in the oesophagus the cancer is located, and how advanced it is. At the time of orphan designation, the main treatments were surgery, radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with oesophageal cancer because early laboratory studies showed that when used

*Disclaimer: 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 (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 513,700,000 (Eurostat 2016).

with cisplatin and fluorouracil it further reduced tumour growth. 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?

Because of the irregular growth of blood vessels in cancers, some parts of the cancer have low levels of oxygen (hypoxia). Lack of oxygen makes cancer cells become resistant to cancer treatments.

This medicine contains haemoglobin, the protein in red blood cells that carries oxygen around the body. It is expected to reach hypoxic cancer cells and to deliver oxygen to them. Since hypoxic cells are unable to cope with large amounts of oxygen, the increased oxygen levels are expected to reduce resistance to cancer treatment and lead to death of the cancer cells, thus slowing down the growth of the cancer.

What is the stage of development of this medicine?

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

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

At the time of submission, the medicine was not authorised anywhere in the EU for oesophageal cancer or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 February 2016 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

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

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.

Translations of the active ingredient and indication in all official EU languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Diaspirin cross-linked haemoglobin	Treatment of oesophageal cancer
Bulgarian	Хемоглобин свързан с диаспирин	Лечение на рак на хранопровода
Croatian	Hemoglobin unakrsno povezan s diaspirinom	Liječenje carcinoma jednjaka
Czech	Diaspirin vázaný na hemoglobin	Léčba karcinomu jícnu
Danish	Diaspirin krydsbundet hæmoglobin	Behandling af øsofagus cancer
Dutch	Diaspirine kruis-gebonden hemoglobine	Behandeling van slokdarm kanker
Estonian	Diaspiriiniga ristühendatud hemoglobiin	Söögitorukasvaja ravi
Finnish	Diaspiriini ristiyhdistetty hemoglobiini	Ruokatorvensyövän hoito
French	Hémoglobine réticulée par diaspirine	Traitement du cancer de l'œsophage
German	Diaspirin vernetztes Hämoglobin	Behandlung des Speiseröhrenkarzinoms
Greek	Αιμοσφαιρίνη διασυνδεδεμένη με διασπιρίνη	Θεραπεία του καρκίνου του οισοφάγου
Hungarian	Diaszpirin-térhálós hemoglobin	Nyelőcsőrák kezelése
Italian	Emoglobina cross-legata alla disaspirina	Trattamento del cancro dell'esofago
Latvian	Diaspirīna šķērseniski-saistīts hemoglobīns	Barības vada vēža ārstēšana
Lithuanian	Su hemoglobinu sujungtas diaspirinas	Stemplės vėžio gydymas
Maltese	Emoglobina b'rabta inkroċjata ma' diaspirin	Kura tal-kanċer tal-esofagu
Polish	Hemoglobina usieciowana kwasem dwusalicylowym	Leczenie raka przełyku
Portuguese	Hemoglobina reticulada por diaspirina	Tratamento de cancro do esófago
Romanian	Hemoglobină cuplată cu diaspirină	Tratamentul cancerului esofagian
Slovak	Hemoglobín viazaný diaspirínom	Liečba karcinómu ezofágu
Slovenian	Diaspirin povezan s hemoglobinom	Zdravljenje raka požiralnika
Spanish	Hemoglobina reticulado por diaspirina	Tratamiento de cáncer del esófago
Swedish	Diaspirin krosslänkad hemoglobin	Behandling av cancer i matstrupen
Norwegian	Diaspirin kryssbundet til haemoglobin	Behandling av kreft i spiserøret
Icelandic	Díaspírín kross-tengdur blóðrauði	Meðferð á vélinda krabbameini

¹ At the time of designation