27 April 2016
EMA/COMP/172769/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Fosbretabulin tromethamine for the treatment of gastro-entero-pancreatic neuroendocrine tumours

Withdrawn

On 21 March 2016, orphan designation (EU/3/16/1633) was granted by the European Commission to Diamond BioPharm Limited, United Kingdom for fosbretabulin tromethamine for the treatment of gastro-entero-pancreatic neuroendocrine tumours.

What are gastro-entero-pancreatic neuroendocrine tumours?

Gastro-entero-pancreatic neuroendocrine tumours (GEP-NETs) are tumours that arise from neuroendocrine cells in the gut. These cells release hormones that control various functions of the digestive system. The symptoms of GEP-NETs depend on where in the gut the tumour is growing and on whether it produces excess hormones. Often by the time of diagnosis the tumours have spread to other organs such as the liver.

GEP-NETs are debilitating as they often produce excess hormones that may cause severe symptoms. They are life-threatening if they spread to other organs in the body.

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

At the time of designation, GEP-NETs affected approximately 3.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 178,000 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 products were authorised in the EU for treating GEP-NETs, including lanreotide and octreotide for the management of symptoms, everolimus and sunitinib.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with GEP-NETs because early data showed positive responses in previously treated

*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).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

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patients. 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?

Fosbretabulin tromethamine is a medicine that disrupts the cells of blood vessels supplying the tumour, disrupting small structures called microtubules within the cells which allow these cells to divide. By disrupting these blood vessels, it is expected to cause the death of tumour cells by cutting off their blood supply, and thereby slow down the progression of the disease.

What is the stage of development of this medicine?

The effects of fosbretabulin tromethamine 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 GEP-NETs were ongoing.

At the time of submission, Fosbretabulin tromethamine was not authorised anywhere in the EU for GEP-NETs. Orphan designation had been granted in the EU for ovarian cancer.

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.

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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.

Withdrawn

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.

Withdrawn

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Fosbretabulin tromethamine	Treatment of gastro-entero-pancreatic neuroendocrine tumours
Bulgarian	Фосбретабулин трометамин	Лечение на гастро-ентеро-панкреатични невроендокринни тумори
Croatian	Fosbretabulinov trometamin	Liječenje gastroenteropankreatičnih neuroendokrinih tumora
Czech	Fosbretabulin trometamin	Léčba gastro-entero- pankreatických neuroendokrinních tumorů
Danish	Fosbretabulin trometamin	Behandling af gastro-entero-pancreatiske neuroendocrine tumorer
Dutch	Fosbretabuline tromethamine	Behandeling van gastro-entero-pancreatische neuroendocriene tumoren
Estonian	Fosbretabuliin trometamiin	Gastroenteropankreaatiliste neuroendokriintuumorite ravi
Finnish	Fosbretabuliini-trometamiini	Maha-suolikanavan ja haiman neuroendokriinisten kasvainten hoito
French	Tromethamine fosbretabuline	Traitement des tumeurs neuroendocrines gastroentéropancréatiques
German	Fosbretabulin-Tromethamin	Behandlung von gastro-entero-pankreatischen neuroendokrinen Tumoren
Greek	Φοσβρεταβουλίνη τρομεθαμίνη	Θεραπεία των γαστρεντεροπαγκρεατικών νευροενδοκρινικών όγκων
Hungarian	Foszbretabulin trometamin	Gasztro-entero-pankreatikus neuroendokrin tumorok kezelése
Italian	Fosbretabulina trometamina	Trattamento dei tumori gastro-entero-pancreatici neuroendocrini
Latvian	Fosbretabulīna trometamīns	Kuņģa-zarnu -aizkuņģa dziedzera neiroendokrīnu audzēju ārstēšana
Lithuanian	Fosbretabulino trometaminas	Skrandžio-žarnyno-kasos neuroendokrininių navikų gydymas
Maltese	Fosbretabulin tromethamine	Kura ta' tumuri newroendokrini gastro-entero-pankrejatiċi
Polish	Fosbretabuliny trometamina	Leczenie guzów neuroendokrynnych przewodu pokarmowego i trzustki
Portuguese	Trometamina de fosbretabulina	Tratamento dos tumores neuroendócrinos gastro-entero-pancreáticos
Romanian	Fosbretabulin trometamină	Tratamentul tumorilor neuroendocrine gastro-entero-pancreatice
Slovak	Fosbretabulín trometamín	Liečba gastroenteropankreatických neuroendokrinných nádorov
Slovenian	Fosbretabulinov trometamin	Zdravljenje gastro-entero-pancreatičnih neuroendokrinih tumorjev

[1] At the time of designation

Language	Active ingredient	Indication
Spanish	Fosbretabulina trometamol	Tratamiento de los tumores neuroendocrinos gastroenteropancreáticos
Swedish	Fosbretabulin trometamin	Behandling av gastroenteropankreatiska neuroendokrina tumörer
Norwegian	Fosbretabulintrometamin	Behandling av gastroenteropankreatiske nevroendokrine tumorer
Icelandic	Fosbretabúlín trómetamín	Meðferð á maga-þarma- bris æxlum af taugainnkirtla¬toga

Withdrawn