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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Copper meso-5,15-bis[3-[(1,2-dicarba-closo-dodecaboranyl)methoxy]phenyl]-meso-10,20-dinitroporphyrin for the treatment of squamous cell carcinoma of the head and neck in patients undergoing radiotherapy

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Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 27 June 2013, orphan designation (EU/3/13/1138) was granted by the European Commission to MorEx Development Partners LLP, United Kingdom, for copper meso-5,15-bis[3-[(1,2-dicarba-closo-dodecaboranyl)methoxy]phenyl]-meso-10,20-dinitroporphyrin for the treatment of squamous cell carcinoma of the head and neck in patients undergoing radiotherapy.

What is squamous cell carcinoma of the head and neck?

Squamous cell carcinoma of the head and neck is a type of cancer that starts in the 'squamous cells' lining the mouth, nose, throat or larynx (voice box). This type of cancer is often associated with alcohol and tobacco use, and may also be associated with local viral infection. Radiotherapy, the use of radiation to kill cancer cells, is used in about two thirds of patients with this cancer.

Squamous cell carcinoma of the head and neck is a long-term debilitating and life-threatening disease that is associated with poor overall survival.

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

At the time of designation, squamous cell carcinoma of the head and neck in patients undergoing radiotherapy affected approximately 3.7 in 10,000 people in the European Union (EU). This was

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equivalent to a total of around 190,000 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, treatment of patients with squamous cell carcinoma of the head and neck depended on the site and stage of development of their cancer. Surgery was widely used where possible to remove the tumour, followed by radiotherapy and chemotherapy (medicines to treat cancer), while a number of other medicines were authorised for use in this disease.

The sponsor has provided sufficient information to show that copper meso-5,15-bis[3-[(1,2-dicarba-closo-dodecaboranyl)methoxy]phenyl]-meso-10,20-dinitroporphyrin might be of significant benefit for patients with squamous cell carcinoma of the head and neck because early studies in experimental models suggest that, when used together with radiotherapy, it might offer better control over the growth of the tumour than radiotherapy alone. 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?

Most of the damage to cells caused by treatment with radiation is not due to the direct effect of the radiation on the cells, but to the formation of 'free radicals' that react with the oxygen circulating between cells, producing very toxic molecules called 'reactive oxygen species' (ROS). These ROS are the cause of cell death. As the blood supply to cancer cells is generally poor they tend to have low levels of intracellular oxygen, which makes them less responsive to treatment with radiation.

This medicine is expected to work by diffusing into tumour tissue and acting as oxygen molecules, thereby increasing the effect of radiation on cancer cell. In addition, the copper in the medicine when exposed to the radiation is expected to emit electrons, which are highly damaging to surrounding cancer cells.

What is the stage of development of this medicine?

The effects of copper meso-5,15-bis[3-[(1,2-dicarba-closo-dodecaboranyl)methoxy]phenyl]-meso-10,20-dinitroporphyrin 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 squamous cell carcinoma of the head and neck had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for treatment of squamous cell carcinoma of the head and neck 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 15 May 2013 recommending the granting of this designation.

*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.
At the time of designation, this represented a population of 512,200,000 (Eurostat 2013).

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:

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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Copper meso-5,15-bis[3-[(1,2-dicarba-closo-dodecaboranyl)methoxy]phenyl]-meso-10,20-dinitroporphyrin	Treatment of squamous cell carcinoma of the head and neck in patients undergoing radiotherapy
Bulgarian	Мед мезо-5,15-Бис[3-[(1,2-Дикарба-клозо-додекаборанил)метокси]фенил]-мезо-10,20-динитропорфирин	лечение на плоскоклетъчен карцином на главата и врата при пациенти, които са подложени на радиотерапия
Czech	komplexy mědi meso-5,15-bis[3-[(1,2-dikarba-kloso-dodekaboranyl)metoxy]fenyl]-meso-10,20-dinitroporfyrin	Léčba spinocelulárního karcinomu hlavy a krku u pacientů podstupujících radioterapii
Danish	Kobber-meso-5,15-bis[3-[(1,2-dicarba-closo-dodecaboranyl)methoxy]fenyl]-meso-10,20-dinitroporphyrin	Behandling af pladeepitelcarcinom i hoved- og halsregion i patienter som får strålebehandling
Dutch	Koper meso-5,15-bis[3-[(1,2-dicarba-closo-dodecaboranyl)methoxy]fenyl]-meso-10,20-dinitroporfyrine	Behandeling van plaveiselcelcarcinoom in hoofd en nek bij patiënten die radiotherapie ondergaan
Estonian	Vask *meso*-5,15-bis[3-[(1,2-dikarba-*closo*-dodekaboranüül)metoksü]fenüül]-meso-10,20-dinitroporfüriin	Pea ja kaela lamerakulise vähiga patsientide ravi radioteraapia korral
Finnish	Kupari-meso-5,15-bis[3-[(1,2-dikarba-kloso-dodekaboranyyli)metoksi]fenyyli]-meso-10,20-dinitroporfyriini	Sädehoitoa saavien potilaiden pään ja kaulan levyepiteelikarsinooman hoito
French	Méso-5,15-Bis[3-[(1,2-Dicarba-closo-dodécaboranyl)méthoxy]phényl]-méso-10,20-dinitroporphyrine de cuivre	Traitement du carcinome épidermoïde de la tête et du cou chez les patients subissant une radiothérapie
German	Kupfer-Meso-5,15-bis[3-[(1,2-Dicarba-closo-dodecaboranyl)methoxy]phenyl]-meso-10,20-dinitroporphyrin	Behandlung von Plattenepithelkarzinomen im Kopf- und Halsbereich bei Patienten, die sich einer Strahlentherapie unterziehen
Greek	Μεσο-5,15-δι-[3-[(1,2-δικαρβο-κλοσο-δωδεκαβορανυλο)μεθοξυ]φαινυλο]-μεσο-10,20-δινιτροπορφυρίνη χαλκού	θεραπεία του πλακώδους καρκινώματος της κεφαλής και του αυχένα σε ασθενείς που υποβάλλονται σε ακτινοθεραπεία
Hungarian	Réz-mezo-5,15-bisz[3-[(1,2-dikarba-klozo-dodekarboranil)metoxi]fenil]-mezo-10,20-dinitroporfirin	Feji és nyaki pikkelyes rákban szenvedő, sugárkezelésben részesülő betegek kezeléséhez
Italian	Rame meso-5,15-Bis[3-[(1,2-Dicarba-closo-dodecaboranil)metossi]fenil]-meso-10,20-dinitroporfirina	Trattamento del carcinoma spinocellulare della testa e del collo in pazienti sottoposti a radioterapia
Latvian	Vara mezo-5,15-bis [3 - [(1,2-dikarba-klozo-dodekaboranil) metoksi] fenil]-mezo-10,20-dinitroporfirīns	Galvas un kakla plakanšūnu vēža slimnieku ārstēšanai, kuri saņem staru terapiju.

[1] At the time of designation

Language	Active ingredient	Indication
Lithuanian	Vario mezo-5,15-bis[3-[(1,2-dikarba-klozo-dodekaboranil)metoksi]fenil]-mezo-10,20-dinitroporfirinas	Plokščialąstelinio galvos ir kaklo vėžio gydymas, radioterapija gydomiems pacientams
Maltese	Copper meso-5,15-bis[3-[(1,2-dicarba-closo-dodecaboranyl)methoxy]phenyl]-meso-10,20-dinitroporphyrin	Kura ta' kanċer taċ-ċelluli skwamużi tar-ras u tal-għonq f'pazjenti għaddejjin bir-radjuterapija
Polish	Mezo-5,15-bis[3-[(1,2-dikarba-kloso-dodekaboranyl)metoksy]fenyl]-mezo-10,20-dwunitroporfiryna miedzi	Leczenie nowotworu kolczystokomórkowego głowy i szyi u pacjentów przechodzących radioterapię
Portuguese	Meso-5,15-Bis[3-[(1,2-Dicarba-closo-dodecaboranil)metoxi]fenil]-meso-10,20-dinitroporfirina de cobre	Tratamento do carcinoma de células esquamosas da cabeça e do pescoço, em doentes submetidos a radioterapia
Romanian	meso-5,15-bis[3-[(1,2-dicarba-closo-dodecaboranil)metoxi]fenil]-meso-10,20-dinitroporfirină complex de cupru	Tratamentul carcinomului scuamos al capului şi gâtului la pacienţii supuşi radioterapiei
Slovak	komplexy medi meso-5,15-bis[3-[(1,2-dikarba-kloso-dodekaboranyl)metoxy]fenyl]-mezo-10,20-dinitroporfyrín	Liečba karcinómudlaždicových buniek na hlave a krku u pacientov podstupujúcich rádioterapiu.
Slovenian	Bakrov mezo-5,15-bis[3-[(1,2-dikarba-klozo-dodekaboranil)metoksi]fenil]-mezo-10,20-dinitroporfirin	Zdravljene raka skvamoznih celic glave in vratu, ob potekajoči radioterapiji bolnika
Spanish	Meso-5,15-bis[3-[(1,2-dicarba-closo-dodecaboranil)metoxi]fenil]-meso-10,20-dinitroporfirina de cobre	Tratamiento del carcinoma de células escamosas de cabeza y cuello en pacientes recibiendo radioterapia
Swedish	Kopparmeso-5,15-bis-[3-[(1,2-dikarba-kloso-dodekaboranyl)-metoxi]fenyl]-meso-10,20-dinitroporfyrin	Behandling av skivepitelkarcinom i huvud- och halsområdet hos patienter som får strålbehandling
Norwegian	Kobber-meso-5,15-bis[3-[(1,2-dikarba-kloso-dodekaboranyl)metoksy]fenyl]-meso-10,20-dinitroporfyrin	Behandling av plateepitelkarsinom i hode- og halsregionen hos pasienter som får strålebehandling
Icelandic	Kopar mesó-5,15-bis[3-[(1,2-díkarba-klósó-dódekabóranýl)metoxý]fenýl]-mesó-10,20-dínítróporfýrín	Meðferð á flöguþekjukrabbameini á höfuð- og hálssvæði hjá sjúklingum sem eru í geislameðferð.