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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Methylthioninium for the treatment of frontotemporal dementia with parkinsonism-17

First publication	14 December 2010
Rev.1: transfer of sponsorship	20 March 2012
Rev.2: administrative update	9 December 2013

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 26 November 2010, orphan designation (EU/3/10/807) was granted by the European Commission to Dr Hans Moebius, United Kingdom, for methylthioninium for the treatment of frontotemporal dementia with parkinsonism-17.

The sponsorship was transferred to Prof. Claude Wischik, United Kingdom, in February 2012.

What is frontotemporal dementia with parkinsonism-17?

Frontotemporal dementia with parkinsonism-17 is a brain disorder with a wide range of symptoms that worsen over time. Patients may lose the ability to control or adjust their behaviour in different situations, leading to inappropriate behaviour. Language skills may also be affected, with the patient losing the ability to speak correctly, to pronounce words properly or to remember the right words. Patients may also show signs of Parkinson's disease such as shaking, slow movement and muscular stiffness.

The exact cause of the disease is unclear, but is thought to be related to the abnormal clumping together of proteins in the brain called 'tau', damaging different areas of the brain. The parts of the brain that are affected are the frontal and temporal (side) lobes.

Frontotemporal dementia with parkinsonism-17 is a debilitating disease that is life threatening because of its damaging effects on the brain.

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What is the estimated number of patients affected by frontotemporal dementia with parkinsonism-17?

At the time of designation, frontotemporal dementia with parkinsonism-17 affected approximately 0.03 in 10,000 people in the European Union (EU). This was equivalent to a total of around 2,000 people[*], and is below the threshold 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?

No satisfactory methods of treatment were authorised at the time of application. Patients were supported in their day-to-day activities by caregivers with help from experts such as speech therapists, psychologists, and physiotherapists.

How is this medicine expected to work?

Methylthioninium is expected to work by dissolving the abnormal tangles of tau proteins in the brain of patients who have frontotemporal dementia with parkinsonism-17, thereby slowing down or reversing the symptoms of the disease.

What is the stage of development of this medicine?

The effects of methylthioninium have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with methylthioninium in patients with frontotemporal dementia with parkinsonism-17 had been started.

At the time of submission, methylthioninium was used or authorised in several countries for the treatment of other diseases including urinary-tract infection, drug-induced methaemoglobinaemia, ifosfamide encephalopathy and refractory shock syndromes.

At the time of submission, methylthioninium was not authorised anywhere in the EU for frontotemporal dementia with parkinsonism-17 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 9 September 2010 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 European Union) or insufficient returns on investment.

[*]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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 506,300,000 (Eurostat 2010).

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

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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	Methylthioninium	Treatment of frontotemporal dementia with parkinsonism-17
Bulgarian	Метилтионин	Лечение на фронто-темпорална деменция с паркинсонизъм-17
Czech	Methylthioninium	Léčba frontotemporální demencie u parkinsonismu -17
Danish	Methylthioninium	Behandling af frontotemporal demens med parkinsonisme-17
Dutch	Methylthioninium	Behandeling van frontotemporale dementie met parkinsonisme-17
Estonian	Metüültioniinium	Parkinsonism-17 frontotemporaalse dementsuse ravi
Finnish	Metyylitioniini	Frontotemporaalisen dementian, johon liittyy Parkinsonin tauti 17, hoito
French	Méthylthioninium	Traitement de la démence frontotemporale avec parkinsonisme lié au chromosome17
German	Methylthioninium	Behandlung der mit Chromosom 17 verbundenen Fronto-temporalen Demenz mit Parkinsonismus
Greek	Μεθυλοθειονίνιο	Θεραπεία της μετωποκροταφικής ανοίας με παρκινσονισμό-17
Hungarian	Metiltionin	17- Parkinzonizmushoz társuló frontotemporális demencia kezelése
Italian	Blu di metilene	Trattamento della demenza frontotemporale e parkinsonismo legati al cromosoma 17
Latvian	Metiltionīns	Frontotemporālās demences ar parkinsonismu-17 ārstēšana
Lithuanian	Metiltioninis	Frontotemporalinės demencijos su parkinsonizmu – 17 gydymas
Maltese	Methylthioninium	Kura tad-dimenzja frontotemporali mal-marda ta' Parkinson-17
Polish	Metylotionina	Lecznie otępienia czołowo-skroniowego z zespołem parkinsonowskim sprzężonym z chromosomem 17
Portuguese	Metiltionina	Tratamento da demência frontotemporal associada ao parkinsonismo-17
Romanian	Metiltioniniu	Tratamentul demenţei fronto-temporale cu parkinsonism-17
Slovak	Metyltionín	Liečba frontotemporálnej demencie s parkinsonizmom-17
Slovenian	Metiltioninij	Zdravljenje frontotemporalne demence s parkinsonizmom-17
Spanish	Metiltionina	Tratamiento de la demencia frontotemporal associada al Parkinsonismo ligado al cromosoma 17
Swedish	Metyltionin	Behandling av frontotemporal demens med parkinsonism (FTDP-17)
Norwegian	Metyltionin	Behandling av frontotemporal demens med parkinsonisme-17
Icelandic	Metýltíónín	Meðferð frontotemporal vitglapa með parkinsonismus-17

[1] At the time of designation