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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Methylthioninium for the treatment of behavioural variant frontotemporal dementia

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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 26 November 2010, orphan designation (EU/3/10/805) was granted by the European Commission to Dr Hans Moebius, United Kingdom, for methylthioninium for the treatment of behavioural variant frontotemporal dementia.

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

What is behavioural variant frontotemporal dementia?

Behavioural variant frontotemporal dementia is a brain disorder in which patients gradually lose their ability to control or adjust their behaviour in different situations, leading to inappropriate behaviour. Language skills may also be affected, with the patient not being able to speak correctly, to pronounce words properly or to remember the right words.

The exact cause of the disease is unclear, but it 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.

Behavioural variant frontotemporal dementia 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 behavioural variant frontotemporal dementia?

At the time of designation, behavioural variant frontotemporal dementia affected approximately 1.5 in 10,000 people in the European Union (EU). This is equivalent to a total of around 76,000 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 in the EU at the time of designation. Patients were supported in their day-to-day activities by caregivers with help from experts such as psychologists, physiotherapists and speech therapists.

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 behavioural variant frontotemporal dementia, 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 behavioural variant frontotemporal dementia 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 behavioural variant frontotemporal dementia 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.

[*]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).

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.

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	Methylthioninium	Treatment of behavioural variant frontotemporal dementia
Bulgarian	Метилтионин	Лечение на повиденчески вариант на фронто-темпорална деменция
Czech	Methylthioninium	Léčba behaviorální varianty fronto-temporální demencie
Danish	Methylthioninium	Behandling af frontotemporal demens varianten karakteriseret af adfærdsforstyrrelser
Dutch	Methylthioninium	Behandeling van gedragsvariant frontotemporale dementie.
Estonian	Metüültioniinium	Frontotemporaalse dementsuse käitumusliku variandi ravi
Finnish	Metyylitioniini	Frontotemporaalisen, käytöshäiriöpainotteisen dementian hoito
French	Méthylthioninium	Traitement du variant de la démence fronto-temporale comportementale
German	Methylthioninium	Behandlung der Fronto-temporalen Demenz vom Verhaltenstyp
Greek	Μεθυλοθειονίνιο	Θεραπεία της μετωποκροταφικής ανοίας με συμπεριφορικές διαταραχές
Hungarian	Metiltionin	Frontotemporális demencia viselkedészavaros változatának kezelése
Italian	Blu di metilene	Trattamento della variante comportamentale della demenza frontotemporale
Latvian	Metiltionīns	Frontotemporālās demencijas uzvedības traucējumu varianta ārstēšanai
Lithuanian	Metiltioninis	Frontotemporalinės demencijos elgsenos tipo gydymas
Maltese	Methylthioninium	Kura tad-dimenzja varjanti frontotemporali tal-imġieba
Polish	Metylotionina	Lecznie wariantu behawioralnego otępienia czołowo-skroniowego
Portuguese	Metiltionina	Tratamento da variante comportamental da demência frontotemporal
Romanian	Metiltioninіu	Tratamentul variantei comportamentale a demenţei frontotemporale
Slovak	Metyltionín	Liečba behaviorálneho variantu frontotemporálnej demencie
Slovenian	Metiltioninij	Zdravljenje vedenjske variante frontotemporalne demence
Spanish	Metiltionina	Tratamiento de la variante frontal de la demencia frontotemporal
Swedish	Metyltionin	Behandling av frontotemporal demens beteendevariant
Norwegian	Metyltionin	Behandling av frontotemporal demens med atferdsvariant
Icelandic	Metýltíónín	Meðferð hegðunar frávika frontotemporal vitglapa

[1] At the time of designation