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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Methylthioninium for the treatment of progressive supranuclear palsy

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Rev.1: transfer of sponsorship	20 March 2012
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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/3/10/804) was granted by the European Commission to Dr Hans Moebius, United Kingdom, for methylthioninium for the treatment of progressive supranuclear palsy.

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

What is progressive supranuclear palsy?

Progressive supranuclear palsy (PSP), which is also known as Steele-Richardson-Olszewski syndrome, is a rare disease that involves the gradual deterioration of parts of the brain. Symptoms include loss of balance with unexplained falls, stiffness, difficulty moving the eyes, particularly up and down, personality changes and dementia (loss of intellectual function). The disease usually starts in people aged over 40 years and gradually gets worse over a number of years.

Patients with PSP have abnormal tangles of a protein called 'tau' in their brain, which are thought to cause the gradual deterioration of brain tissue seen in these patients.

PSP is a debilitating and life-threatening disease that leads to a progressive inability to move and poor prospects of long-term survival.

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What is the estimated number of patients affected by progressive supranuclear palsy?

At the time of designation, PSP affected approximately 0.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 30,000 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?

At the time of designation, no satisfactory methods were authorised in the EU for the treatment of PSP. Because of their tendency to fall, patients were often offered walking aids, as well as special glasses to help them to look down. Physiotherapy was used to keep the joints flexible. Medicines developed to treat Parkinson's disease were used in some PSP patients, but their effect was usually temporary.

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 progressive supranuclear palsy, 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 PSP 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 PSP 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 progressive supranuclear palsy
Bulgarian	Метилтионин	Лечение на прогресивна супрануклеарна парализа
Czech	Methylthioninium	Léčba progresivní supranukleární paralýzy
Danish	Methylthioninium	Behandling af progressiv, supranukleær parese
Dutch	Methylthioninium	Behandeling van progressieve supranucleaire paralyse
Estonian	Metüültioniinium	Progressiivse supranukleaarse halvatuse ravi
Finnish	Metyylitioniini	Progressiivisen supranukleaarisen halvauksen hoito
French	Méthylthioninium	Traitement de la paralysie supranucléaire progressive
German	Methylthioninium	Behandlung der progressiven supranukleären Lähmung
Greek	Μεθυλοθειονίνιο	Θεραπεία προϊούσας υπερπυρηνικής παράλυσης
Hungarian	Metiltionin	Progresszív supranuclearis bénulás kezelése
Italian	Blu di metilene	Trattamento della paralisi sopranucleare progressiva
Latvian	Metiltionīns	Progresējošās supranukleārās triekas ārstēšana
Lithuanian	Metiltioninis	Progresuojančio supranuklearinio paralyžiaus gydymas
Maltese	Methylthioninium	Kura ta' paraliżi supranukleari progressiva
Polish	Metylotionina	Leczenie postępującego porażenia nadjądrowego
Portuguese	Metiltionina	Tratamento da paralisia supranuclear progressiva
Romanian	Metiltioniniu	Tratamentul paraliziei supra-nucleare progresive
Slovak	Metyltionín	Liečba progresívnej supranukleárnej paralýzy
Slovenian	Metiltioninij	Zdravljenje progresivne supranuklearne paralize
Spanish	Metiltionina	Tratamiento de parálisis supranuclear progresiva
Swedish	Metyltionin	Behandling av progressiv supranukleär pares
Norwegian	Metyltionin	Behandling av progressiv supranukleær parese
Icelandic	Metýltíónín	Meðferð við ágengri ofankjarnalömun

[1] At the time of designation