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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

4-[3-(methylsulfonyl)phenyl]-1-propylpiperidine x HC1 for the treatment of Huntington's disease

First publication	8 July 2005
Rev.1: sponsor's change of address	12 November 2009
Rev.2: transfer of sponsorship	5 June 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 20 June 2005, orphan designation (EU/3/05/288) was granted by the European Commission to A Carlsson Research AB, Sweden, for 4-[3-(methylsulfonyl)phenyl]-1-propylpiperidine x HC1 for the treatment of Huntington's disease.

In June 2007, A Carlsson Research AB changed name to NeuroSearch Sweden AB.

The sponsorship was transferred to NSAB, Filial af NeuroSearch Sweden AB, Sverige, Denmark in July 2009 and subsequently to Teva Pharma GmbH, Germany, in April 2013.

What is Huntington's disease?

Huntington's disease is a hereditary disease where the cells (neurons) of specific areas of the brain (the so-called basal ganglia and cerebral cortex) degenerate. When activated the neurons release a group of substances, the so-called neurotransmitters. Once released, the neurotransmitters activate or inhibit the target cells in the different body organs. In Huntington's disease, due to the extensive degeneration of neurons, the nervous system cannot regulate properly the target organs anymore. This leads to the typical symptoms of the disease, namely involuntary movements, behavioural disturbances and mental deterioration. The disease progresses over time and is chronically debilitating with potentially life-threatening complications.

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

At the time of designation, Huntington's disease affected between 0.4 to 0.8 in 10,000 people in the European Union (EU). This was equivalent to a total of between 19,000 and 37,000 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?

No satisfactory methods exist that were authorised at the time of application.

How is this medicine expected to work?

4-[3-(methylsulfonyl)phenyl]-1-propylpiperidine x HC1 acts by stabilising the effects of a neurotransmitter called dopamine, which might result in some improvement in the clinical symptoms associated with Huntington's disease.

What is the stage of development of this medicine?

The evaluation of the effects of 4-[3-(methylsulfonyl)phenyl]-1-propylpiperidine x HC1 in experimental models is ongoing.

At the time of submission of the application for orphan designation, clinical trials in patients with Huntington's disease were ongoing.

The medicinal product was not marketed anywhere worldwide for Huntington's disease or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 May 2005 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.

*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 25), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 466,600,000 (Eurostat 2005).

For more information

Sponsor's contact details:

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89079 Ulm
Germany
Telephone: +49 6105 97 676 17
Telefax: +49 6105 97 767 60
http://www.teva-deutschland.de/kontakt.html

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	4-[3-(methylsulfonyl)phenyl]-1-propylpiperidine x HCl	Treatment of Huntington's disease
Bulgarian	4-[3-(метилсулфонил)фенил]-1-пропилпиперидин x HC1	Лечение на болест на Хънтингтон
Czech	4-[3-(methylsulfonyl)phenyl]-1-propylpiperidine x HCl	Léčba Huntingtonovy nemoci
Danish	4-[3-(methylsulfonyl)fenyl]-1-propylpiperidin x HCl	Behandling af Huntington's sygdom
Dutch	4-[3-(methylsulfonyl)fenyl]-1-propylpiperidine x HCl	Behandeling van de ziekte van Huntington
Estonian	4-[3-(metüülsulfonüül)fenüül]-1-propüülpiperidiin x HCl	Huntington'i tõve ravi
Finnish	4-[3-(metyylisulfonyyli)fenyyli]-1-propyylipiperidiini x HCl	Huntingtonin taudin hoito
French	4-[3-(methylsulfonyl)phenyl]-1-propylpiperidine x HCl	Traitement de la maladie d'Huntington
German	4-[3-(methylsulfonyl)phenyl]-1-propylpiperidin x HCl	Behandlung der Huntington Erkrankung
Greek	4-[3-(methylsulfonyl)phenyl]-1-propylpiperidine x HCl	Θεραπεία της νοσου Huntington
Hungarian	4-[3-(metilszulfonil)fenil]-1-propilpiperidin HCl	Huntington kór kezelése
Italian	4-[3-(metilsulfonil)fenil]-1-propilpiperidina x HCl	Trattamento della malattia di Huntington
Latvian	4-[3-(metilsulfonil)fenil]-1-propilpiperidīns x HCl	Hantingtona slimības ārstēšanai
Lithuanian	4-[3-(metilsulfonil)fenil]-1-propilpiperidinas x HCl	Huntington'o ligos gydymas
Maltese	4-[3-(methylsulfonyl)phenyl]-1-propylpiperidine x HC1	Kura tal-marda ta' Huntington
Polish	Chlorowodorek 4-[3-(metylosulfonylo)-fenylo]-1-propylopiperydyny	Leczenie pląsawicy Huntingtona
Portuguese	4-[3-(metilsulfonil)fenil]-1-propilpiperidina x HCl	Tratamento da doença de Huntington
Romanian	4-[3-(metilsulfonil)fenil]-1-propilpiperidină x HC1	Tratamentul bolii Huntington
Slovak	4-[3-(metylsulfonyl)fenyl]-1-propylpiperidín x HCl	Liečba Huntingtonovej choroby
Slovenian	4-[3(metilsulfonil)fenil]-1-propilpiperidin hidroklorid	Zdravljenje Huntingtonove bolezni

[1] At the time of transfer of sponsorship

Language	Active Ingredient	Indication
Spanish	4-[3-(metilsulfonil)fenil]-1-propilpiperidina x HCl	Tratamiento de la enfermedad de Huntington
Swedish	4-[3-(metylsulfonyl)fenyl]-1-propylpiperidin x HCl	Behandling av Huntingtons sjukdom
Norwegian	4-[3-(metylsulfonyl)fenyl]-1-propylpiperidin x HCl	Behandling av Huntingtons sykdom.
Icelandic	4-[3-(methýlsúlfónýl)phenýl]-1-propýlpiperidín x HCl	Meðferð við Huntingtons sjúkdómi