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EMA/COMP/275974/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

AASSGVSTPGSAGHDIITEQPRS for the treatment of Huntington's disease

On 12 May 2015, orphan designation (EU/3/15/1492) was granted by the European Commission to Centre National de la Recherche Scientifique (CNRS), France, for AASSGVSTPGSAGHDIITEQPRS for the treatment of Huntington's disease.

What is Huntington's disease?

Huntington's disease is a hereditary disease that causes brain cells to die, leading to symptoms such as involuntary jerky movements, behavioural problems and dementia (loss of intellectual function). The disease is usually first noticed between 35 and 45 years of age and gets worse over time.

Huntington's disease is caused by defects in the gene responsible for the production of a protein called huntingtin. The gene abnormalities result in an abnormal form of the protein being produced, which causes damage to the cells in specific areas of the brain.

Huntington's disease is a debilitating and life-threatening condition because it causes severe behavioural and mental problems, a progressive loss of the ability to move and potentially life-threatening complications.

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

At the time of designation, Huntington's disease affected approximately 1.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 67,000 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, the treatments authorised in the EU for Huntington's disease were aimed at relieving the symptoms of the disease. In some Member States, haloperidol, pimozide, tetrabenazine and tiapride were authorised for the abnormal involuntary movements that occur in Huntington's

*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. This represents a population of 512,900,000 (Eurostat 2015).

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disease. In addition, benzodiazepines were used for anxiety, and antidepressants and lithium to treat depression and mood swings.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with Huntington's disease because early studies in experimental models show that it may slow down the progression of symptoms of the disease affecting movement. 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?

In Huntington's disease, the abnormal huntingtin proteins stick together (aggregate) and form deposits that damage brain cells. This medicine is made of a shorter version of the huntingtin protein. It is expected to work by preventing the abnormal huntingtin proteins sticking together, thereby reducing damage to brain cells and improving the symptoms of the disease.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicine in experimental models was ongoing.

At the time of submission, no clinical trials with the medicine in patients with Huntington's disease had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for Huntington's disease 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 16 April 2015 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.

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	AASSGVSTPGSAGHDIITEQPRS	Treatment of Huntington's disease
Bulgarian	AASSGVSTPGSAGHDIITEQPRS	Лечение на болест на Хънтингтон
Croatian	AASSGVSTPGSAGHDIITEQPRS	Liječenje Huntingtonove bolesti
Czech	AASSGVSTPGSAGHDIITEQPRS	Léčba Huntingtonovy nemoci
Danish	AASSGVSTPGSAGHDIITEQPRS	Behandling af Huntington's sygdom
Dutch	AASSGVSTPGSAGHDIITEQPRS	Behandeling van de ziekte van Huntington
Estonian	AASSGVSTPGSAGHDIITEQPRS	Huntington'i tõve ravi
Finnish	AASSGVSTPGSAGHDIITEQPRS	Huntingtonin taudin hoito
French	AASSGVSTPGSAGHDIITEQPRS	Traitement de la maladie d'Huntington
German	AASSGVSTPGSAGHDIITEQPRS	Behandlung der Huntington Erkrankung
Greek	AASSGVSTPGSAGHDIITEQPRS	Θεραπεία της νόσου Huntington
Hungarian	AASSGVSTPGSAGHDIITEQPRS	Huntington kór kezelése
Italian	AASSGVSTPGSAGHDIITEQPRS	Trattamento della malattia di Huntington
Latvian	AASSGVSTPGSAGHDIITEQPRS	Hantingtona slimības ārstēšanai
Lithuanian	AASSGVSTPGSAGHDIITEQPRS	Huntington'o ligos gydymas
Maltese	AASSGVSTPGSAGHDIITEQPRS	Kura tal-marda ta' Huntington
Polish	AASSGVSTPGSAGHDIITEQPRS	Leczenie pląsawicy Huntingtona
Portuguese	AASSGVSTPGSAGHDIITEQPRS	Tratamento da doença de Huntington
Romanian	AASSGVSTPGSAGHDIITEQPRS	Tratamentul bolii Huntington
Slovak	AASSGVSTPGSAGHDIITEQPRS	Liečba Huntingtonovej choroby
Slovenian	AASSGVSTPGSAGHDIITEQPRS	Zdravljenje Huntingtonove bolezni
Spanish	AASSGVSTPGSAGHDIITEQPRS	Tratamiento de la enfermedad de Huntington
Swedish	AASSGVSTPGSAGHDIITEQPRS	Behandling av Huntingtons sjukdom
Norwegian	AASSGVSTPGSAGHDIITEQPRS	Behandling av Huntingtons sykdom
Icelandic	AASSGVSTPGSAGHDIITEQPRS	Meðferð við Huntingtons sjúkdómi

[1] At the time of designation