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Public summary of opinion on orphan designation

2-(3,7-Dimethyl-octa-2, 6-dienyl)-6-ethylamino-3-hydroxy-5-pentyl-[1,4]benzoquinone for the treatment of Huntington's disease

On 9 January 2020, orphan designation EU/3/19/2237 was granted by the European Commission to Emerald Health Pharmaceuticals Espana S.L, Spain, for 2-(3,7-dimethyl-octa-2, 6-dienyl)-6-ethylamino-3-hydroxy-5-pentyl-[1,4]benzoquinone (also known as VCE-003.2) 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. This leads to symptoms such as jerky movement, behavioural problems and dementia (loss of intellectual ability). 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 producing a protein called huntingtin. The defects result in an abnormal form of the protein being produced, which damages nerve cells in certain 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 in 10,000 people in the European Union (EU). This was equivalent to a total of 52,000 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, other treatments authorised for

*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 518,400,000 (Eurostat 2019).

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managing movement disorders as well psychiatric symptoms included antipsychotics, benzodiazepines, antidepressants and medicines for treating mood swings.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with Huntington's disease. Laboratory data suggest that the medicine treats the underlying process of nerve damage and may slow down the loss of ability to move.

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?

The medicine activates a receptor (target) on cells called PPARgamma, which plays a key role in the generation and growth of nerve cells.

By activating PPARgamma it is thought that this medicine will help the body to produce new brain cells to replace the damaged ones in people with Huntington' disease. This is expected to slow down progression of the disease.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, 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 the treatment of Huntington's disease. Orphan designation of the medicine had been granted in the United States for Huntington's disease.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 5 December 2019, recommending the granting of this designation.

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:

Contact details of the current sponsor for this orphan designation can be found on EMA website.

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	2-(3,7-dimethyl-octa-2, 6-dienyl)-6-ethylamino-3-hydroxy-5-pentyl-[1,4]benzoquinone	Treatment of Huntington's disease
Bulgarian	2-(3,7-Диметил-окта-2, 6-диенил)-6-етиламино-3-хидрокси-5-пентил-[1,4]бензоквинон	Лечение на болест на Хънтингтон
Croatian	2-(3,7-dimetilokta-2, 6-dienil)-6-etilamino-3-hidroksi-5-pentil-[1,4]benzokinon	Liječenje Huntingtonove bolesti
Czech	2-(3,7-dimethyl-okta-2, 6-dienyl)-6-ethylamino-3-hydroxy-5-pentyl-[1,4]benzochinon	Léčba Huntingtonovy nemoci
Danish	2-(3,7-Dimethyl-octa-2, 6 dienyl)-6-ethylamin-3-hydroxy-5-pentyl-[1,4]benzoquinon	Behandling af Huntington’s sygdom
Dutch	2-(3,7-dimethyl-octa-2, 6-di-enyl)-6-ethylamino-3-hydroxy-5-pentyl-[1,4]benzochinon	Behandeling van de ziekte van Huntington
Estonian	2-(3,7-dimetüül-okta-2, 6-dienüül)-6-etüülamino-3-hüdroksü-5-pentüül-[1,4]bensokinoon	Huntington’i tõve ravi
Finnish	2-(3,7-dimetyyli-okta-2, 6-dienyyli)-6-etyyliamino-3-hydroksi-5-pentyyli-[1,4]bentsokiniini	Huntingtonin taudin hoito
French	2-(3,7-diméthyl-octa-2, 6-diényl)-6-éthylamino-3-hydroxy-5-pentyl-[1,4]benzoquinone	Traitement de la maladie d’Huntington
German	2-(3,7-Dimethyl-octa-2, 6 dienyl)-6-ethylamino-3-hydroxy-5-pentyl-[1,4]Benzochinon	Behandlung der Huntington Erkrankung
Greek	2-(3,7-διμεθυλο-οκτα-2, 6-διενυλο)-6-αιθυλαμινο-3-υδροξυ-5-πεντυλο-[1,4]βενζοκινόνη	Θεραπεία της νόσου Huntington
Hungarian	2-(3,7-Dimetil-okta-2, 6-dienil)-6-etilamino-3-hidroxi-5-pentil-[1,4]benzokinon	Huntington kór kezelése

[1] At the time of designation

Language	Active ingredient	Indication
Italian	2-(3,7-dimetil–otta-2, 6-dienile)-6-etilammino-3-idrossi-5-pentile-[1,4]benzochinone	Trattamento della malattia di Huntington
Latvian	2-(3,7-dimetil-okta-2,6-dienil)-6-etilamino-3-hidroksi-5-pentil-[1,4]benzohinons	Hantingtona slimības ārstēšanai
Lithuanian	2-(3,7-dimetil-okta-2, 6-dienil)-6-etilamino-3-hidroksi-5-pentil-[1,4]benzokvinonas	Huntington’o ligos gydymas
Maltese	2-(3,7-dimethyl-octa-2, 6-dienyl)-6-ethylamino-3-hydroxy-5-pentyl-[1,4]benzoquinone	Kura tal-marda ta’ Huntington
Polish	2-(3,7-dimetylo-okta-2, 6-dienylo)-6-etyloamino-3-hydroksy-5-pentylo-[1,4]benzochinon	Leczenie pląsawicy Huntingtona
Portuguese	2-(3,7-Dimetil-octa-2, 6-dienil)-6-etilamino-3-hidroxi-5-pentil-[1,4]benzoquinona	Tratamento da doença de Huntington
Romanian	2-(3,7-dimetil-octa-2, 6-dienil)-6-etilamino-3-hidroxi-5-pentil-[1,4]benzochinonă	Tratamentul bolii Huntington
Slovak	2-(3,7-dimetyl-okta-2,6-dienyl)-6-etylamino-3-hydroxy-5-pentyl-[1,4]benzochinón	Liečba Huntingtonovej choroby
Slovenian	2-(3,7-dimetil-okta-2, 6-dienil)-6-etilamino-3-hidroksi-5-pentil-[1,4]benzokvinon	Zdravljenje Huntingtonove bolezni
Spanish	2-(3,7-dimetilocta-2, 6-dienilo)-6-etilamino-3-hidroxi-5-pentil-[1,4]benzoquinona	Tratamiento de la enfermedad de Huntington
Swedish	2-(3,7-dimetylokta-2, 6-dienyl)-6-etylamino-3-hydroxi-5-pentyl-[1,4]bensokinon	Behandling av Huntingtons sjukdom
Norwegian	2-(3,7-dimetylokta-2,6-dienyl)-6-etylamino-3-hydroksyl-5-pentyl-[1,4]benzokinon	Behandling av Huntingtons sykdom
Icelandic	2-(3,7-dímetýl-okta-2, 6-dienýl)-6-etýlamínó-3-hýdroxý-5-pentýl-[1,4]bensókínónón	Meðferð við Huntingtons sjúkdómi