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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Trientine tetrahydrochloride for the treatment of Wilson's disease

On 19 March 2015, orphan designation (EU/3/15/1471) was granted by the European Commission to GMP-Orphan SAS, France, for trientine tetrahydrochloride for the treatment of Wilson's disease.

What is Wilson's disease?

Wilson's disease is a genetic disorder that causes copper absorbed from food to accumulate in the body. In healthy people, liver cells remove most of the excess copper and release it into the bile, which flows into the intestines. In people with Wilson's disease, due to a genetic defect, the liver cannot remove copper and the copper builds up in the liver and in other organs such as the kidneys, brain and eyes, and damages them.

Wilson's disease is chronically debilitating and can be life threatening if not treated due to the toxicity of copper in the liver and brain.

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

At the time of designation, Wilson's disease affected approximately 0.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 31,000 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, several medicines were authorised in the EU for the treatment of Wilson's disease, including penicillamine and trientine dihydrochloride, which is used in patients who cannot tolerate penicillamine but is only authorised in one EU country. These medicines were used to remove excess copper from the body and to limit the absorption of further copper from food. Zinc acetate was also authorised for Wilson's disease, although it was not recommended for use at the beginning of treatment.

*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).

The sponsor has provided sufficient information to show that trientine tetrahydrochloride might be of significant benefit for patients with Wilson's disease because it is assumed to be similar to trientine dihydrochloride and an orphan designation may encourage it to be made more widely available. 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?

Trientine tetrahydrochloride is expected to work in the same way as trientine dihydrochloride, by attaching to the free copper in the body and forming a compound that can be excreted in the urine by the kidneys.

What is the stage of development of this medicine?

The effects of trientine tetrahydrochloride have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with trientine tetrahydrochloride had been started.

At the time of submission, trientine tetrahydrochloride was not authorised anywhere in the EU for Wilson'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 12 February 2015 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:

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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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Trientine tetrahydrochloride	Treatment of Wilson's disease
Bulgarian	Триентин тетраhydroхлорид	Лечение на болест на Уилсън
Croatian	Trientintetraklorid	Liječenje Wilsonove bolesti
Czech	Trientine tetrahydrochloridu	Léčba Wilsonovy choroby
Danish	Trientintetrahydrochlorid	Behandling af Wilsons sygdom
Dutch	Trientine tetrahydrochloride	Behandeling van de ziekte van Wilson
Estonian	Trientiintetrahydrokloriid	Wilsoni haiguse ravi
Finnish	Trientiini tetrahydrokloridi	Wilsonin taudin hoito
French	Tétrachlorydrate de trientine	Traitement de la maladie de Wilson
German	Trientine Tetrahydrochlorid	Behandlung des Morbus Wilson
Greek	Τετραϋδροχλωρική τριεντίνη	Θεραπεία της νόσου του Wilson
Hungarian	Trientin tetrahydroklorid	Wilson-betegség kezelése
Italian	Tetracloridrato di trientina	Trattamento del morbo di Wilson
Latvian	Trientīna tetrahidrohlorīds	Vilsona slimības ārstēšana
Lithuanian	Trientino tetrahydrochloridas	Vilsono ligos gydymas
Maltese	Trientine tetrahydrochloride	Kura tal-marda ta' Wilson
Polish	Trientyny chlorowodorek	Leczenie choroby Wilsona
Portuguese	Tetracloridrato de trientina	Tratamento da doença de Wilson
Romanian	Tetraclorhidrat de trientină	Tratamentul bolii Wilson
Slovak	Trientine tetrahydrochloride	Liečba Wilsonovej choroby
Slovenian	Trientinijev tetrahidroklorid	Zdravljenje Wilsonove bolezni
Spanish	Tetrahydrochloride de trientina	Tratamiento de la enfermedad de Wilson
Swedish	Trientin tetrahydroklorid	Behandling av Wilsons sjukdom
Norwegian	Trientintetrahydroklorid	Behandling av Wilsons sykdom
Icelandic	Tríentín tetrahyðróklóríð	Meðferð við Wilsons sjúkdómi

¹ At the time of designation