



3 March 2015
EMA/COMP/795268/2012 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Choline tetrathiomolybdate for the treatment of Wilson's disease

First publication	18 February 2013
Rev.1: transfer of sponsorship	3 March 2015
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 24 January 2013, orphan designation (EU/3/12/1089) was granted by the European Commission to Medical Need Europe AB, Sweden, for choline tetrathiomolybdate for the treatment of Wilson's disease.

The sponsorship was transferred to Wilson Therapeutics AB, Sweden, in July 2014.

What is Wilson's disease?

Wilson's disease is a genetic disorder that causes excessive copper accumulation in the body. In healthy people, copper absorbed from food is cleared from the body primarily in the bile, a liquid produced from the liver and secreted into the intestines. In people with Wilson's disease, the liver cannot get rid of the copper, so it 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).

*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. At the time of designation, this represented a population of 512,200,000 (Eurostat 2013).



What treatments are available?

At the time of designation, several medicines were authorised in the EU for the treatment of Wilson's disease. These medicines were used to remove excess copper from the body and to limit the absorption of further copper from food.

The sponsor has provided sufficient information to show that choline tetrathiomolybdate might be of significant benefit for patients with Wilson's disease because it is expected to rapidly decrease copper levels in the body. In particular, it is expected to be beneficial for patients with Wilson's disease who suffer from neurological symptoms (affecting the brain) and who sometimes get worse when starting treatment with available therapies. 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?

Choline tetrathiomolybdate is expected to rapidly attach to copper and to a protein called albumin in the bloodstream. Once attached to choline tetrathiomolybdate and albumin, copper cannot be taken up by the organs and is eliminated from the body. Therefore, the damaging effects of copper are expected to decrease.

What is the stage of development of this medicine?

The effects of choline tetrathiomolybdate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with choline tetrathiomolybdate in patients with Wilson's disease had been started.

At the time of submission, choline tetrathiomolybdate was not authorised anywhere in the EU for Wilson's disease. Orphan designation of choline tetrathiomolybdate had been granted in the United States of America for Wilson's disease.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 6 December 2012 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:

Wilson Therapeutics AB
Västra Trädgårdsgatan 15
111 53 Stockholm
Sweden
Tel. +46 7 09 44 18 77
Fax +46 8 50 90 95 52
E-mail: info@wilsontherapeutics.com

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	Choline tetrathiomolybdate	Treatment of Wilson's disease
Bulgarian	Холин тетратиомолибдат	Лечениена болест на Уилсън
Croatian	Kolinijev tetratiomolibdat	Liječenje Wilsonove bolesti
Czech	Cholin tetrathiomolybdat	Léčba Wilsonovy choroby
Danish	Cholin tetrathiomolybdat	Behandling af Wilsons sygdom
Dutch	Choline tetrathiomolybdate	Behandeling van de ziekte van Wilson
Estonian	Koliini tetrathiomolübdäatybdate	Wilsoni haiguse ravi
Finnish	Koliinitetratiomolybdaatti	Wilsonin taudin hoito
French	Choline tétrathiomolybdate	Traitement de la maladie de Wilson
German	Cholin tetrathiomolybdat	Behandlung des Morbus Wilson
Greek	Τετραθειομολυβδαινική χολίνη	Θεραπεία της νόσου του Wilson
Hungarian	Kolin tetratiomolibdát	Wilson-betegség kezelése
Italian	Colina tetrathiomolybdate	Trattamento del morbo di Wilson
Latvian	Holīna tetratiomolibdāts	Vilsons slimības ārstēšana
Lithuanian	Cholino tetratiomolibdatas	Vilsono ligos gydymas
Maltese	Choline tetrathiomolybdate	Kura tal-marda ta' Wilson
Polish	Tetratiomolibdenian choliny	Leczenie choroby Wilsona
Portuguese	Colina tetratiomolibdato	Tratamento da doença de Wilson
Romanian	Colină tetratiomolibdat	Tratamentul bolii Wilson
Slovak	Cholín tetratiomolybdát	Liečba Wilsonovej choroby
Slovenian	Holin tetratiomolibdat	Zdravljenje Wilsonove bolezni
Spanish	Tetratiomolibdato de Colina	Tratamiento de la enfermedad de Wilson
Swedish	Kolin tetratiomolybdat	Behandling av Wilsons sjukdom
Norwegian	Kolintetratiomolybdat	Behandling av Wilsons sykdom
Icelandic	Kólín tetrathíómólýbdat	Meðferð við Wilsons sjúkdómi

¹ At the time of transfer of sponsorship