



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

26 February 2015
EMA/COMP/1293/2003 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Zinc acetate dihydrate for the treatment of Wilson's disease

First publication	3 March 2009
Rev.1: administrative update	3 May 2011
Rev.2: withdrawal from the Community Register	26 February 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.	

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in October 2014 at the end of the period of market exclusivity.

On 30 July 2001, orphan designation (EU/3/01/050) was granted by the European Commission to Orphan Europe s.a.r.l., France, for zinc acetate dihydrate for the treatment of Wilson's disease.

What is Wilson's disease?

Wilson's disease is a genetic disorder that causes excessive copper accumulation in the body, typically in the liver and brain. The liver of a person who has Wilson's disease does not release copper into the bile, as it should. Bile is a liquid produced by the liver that helps with digestion. In Wilson's disease, the copper that is normally absorbed from the food by the intestines builds up in the liver and injures liver tissue. Eventually, the damage causes the liver to release the copper directly into the bloodstream, which carries the copper throughout the body. The copper accumulated and transported by the bloodstream can then cause damage in other organs like the kidneys, brain, and eyes. If not treated, Wilson's disease can be chronically debilitating and life threatening.



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 23,000 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?

Treatment of Wilson's disease generally consisted of anti-copper agents to remove excess copper from the body and to prevent it from re-accumulating. Several medicinal products were authorised for Wilson's disease in the Community at the time of submission of the application for orphan drug designation.

Satisfactory argumentation has been provided by the sponsor to justify that zinc acetate dihydrate could be of potential significant benefit for the treatment of Wilson's disease because it could contribute to the existing patient care. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Zinc acetate dihydrate blocks the absorption of copper and increases copper excretion in the stool.

What is the stage of development of this medicine?

The effects of zinc acetate have been evaluated in experimental models. At the time of submission, clinical trials in patients with Wilson's disease were ongoing.

At the time of submission, zinc acetate was not authorised anywhere in the Community for Wilson's disease. Orphan designation of zinc acetate had been granted in the United States for Wilson's disease.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 June 2001 recommending the granting of this designation.

Update: Zinc acetate dihydrate (Wilzin) has been authorised in the EU since 13 October 2004 for treatment of Wilson's disease.

More information on Wilzin can be found in the European public assessment report (EPAR) on the Agency's website: [ema.europa.eu/Find_medicine/Human_medicines/European Public Assessment Reports](http://ema.europa.eu/Find_medicine/Human_medicines/European_Public_Assessment_Reports)

*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.
At the time of designation, this represented a population of 378,800,000 (Eurostat 2001).

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	Zinc acetate dihydrate	Treatment of Wilson's disease
Danish	Zinkacetatdihydrat	Behandling af Wilsons sygdom
Dutch	zinkacetaatdihydraat	Behandeling van de ziekte van Wilson
Finnish	Sinkkiasetaatin dihydraatti	Wilsonin taudin hoito
French	Acétate de zinc dihydraté	Traitement de la maladie de Wilson
German	Zinkacetat Dihydrat	Behandlung des Morbus Wilson
Greek	Διένυδρος οξικού ψευδαργύρου	Θεραπεία της νόσου του Wilson
Italian	Zinco acetato diidrato	Trattamento del morbo di Wilson
Portuguese	Acetato de zinco dihidrato	Tratamento da doença de Wilson
Spanish	Acetato de zinc dihidrato	Tratamiento de la enfermedad de Wilson
Swedish	Zinkacetat dihydrat	Behandling av Wilsons sjukdom

¹ At the time of designation