



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

11 December 2012
EMA/COMP/682684/2012
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Erdosteine for the treatment of mercury toxicity

On 8 November 2012, orphan designation (EU/3/12/1067) was granted by the European Commission to Rafifarm SRL, Romania, for erdosteine for the treatment of mercury toxicity.

What is mercury toxicity?

Mercury toxicity can occur when a patient has been exposed to mercury. The level of toxicity depends on the chemical form of the mercury and how the patient was exposed to it. Exposure may occur through certain foods (such as fish), from mercury-containing dental amalgams (a substance used in some dental fillings) or through contact with materials in factories where mercury-containing products are used or manufactured.

Mercury toxicity can lead to symptoms affecting many parts of the body, including the nervous system, gastrointestinal tract (gut including the liver), lungs, and kidneys. Acute toxicity can result in shortness of breath, chest pain, chills, nausea and vomiting, joint swelling and rash, while long-term exposure to mercury commonly results in mouth lesions, tremor, poor coordination and psychiatric problems among others.

Mercury toxicity is a long-term debilitating condition that may be life threatening due to the damage it can cause to the nervous, renal (kidney), endocrine (hormonal) and gastrointestinal systems.

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

At the time of designation, mercury toxicity affected less than 1 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 51,000 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 27), Norway, Iceland and Liechtenstein. This represents a population of 506,300,000 (Eurostat 2011).



What treatments are available?

At the time of designation, medicines authorised in EU countries for mercury toxicity included dimercaprol, D-penicillamine, 2,3-dimercaptosuccinic acid (DMSA), and 2,3-dimercapto-1-propane sulfonic acid (DMPS). These medicines are 'chelating' agents: they work by attaching to mercury to form a compound that can be more easily excreted from the body.

The sponsor has provided sufficient information to show that erdosteine might be of significant benefit for patients with mercury toxicity because early studies in experimental models show that it might improve survival. 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?

Erdosteine is a mucolytic (a substance that makes mucus thinner). It is not known how erdosteine works in neutralising mercury toxicity, but studies in experimental models show that during intoxication it increases the excretion of mercury via the urine.

What is the stage of development of this medicine?

The effects of erdosteine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with erdosteine in patients with mercury toxicity had been started.

At the time of submission, erdosteine was not authorised anywhere in the EU for mercury toxicity 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 5 October 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:

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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 substance	Indication
English	Erdosteine	Treatment of mercury toxicity
Bulgarian	Ердостеин	Лечение на живачна токсичност
Czech	Erdostein	Léčba toxicity rtuti
Danish	Erdosteine	Behandling af kviksølv toksicitet
Dutch	Erdosteïne	Behandeling van kwikvergiftiging
Estonian	Erdosteine	Elavhõbeda toksilisuse ravi
Finnish	Erdosteiniini	Elohopeamyrkytyksen hoito
French	Erdostéine	Traitement de la toxicité au mercure
German	Erdostein	Behandlung einer Quecksilbervergiftung
Greek	Ερδοστεϊνή	Θεραπεία της τοξικότητας υδραργύρου
Hungarian	Erdostein	Higanymérgezés kezelése
Italian	Erdosteina	Trattamento di tossicità del mercurio
Latvian	Erdosteīns	Dzīvsudraba toksitātes ārstēšana
Lithuanian	Erdosteinas	Gyvsidabrio toksiškumo gydymas
Maltese	Erdosteine	Kura ta' tossicità bil-merkurju
Polish	Erdosteina	Leczenie zatruc rtęcią
Portuguese	Erdosteína	Tratamento da intoxicação pelo mercúrio
Romanian	Erdosteina	Tratamentul intoxicației cu mercur
Slovak	Erdosteín	Liečba otravy ortuťou
Slovenian	Erdostein	Zdravljenje zastrupitev z živim srebrom
Spanish	Erdosteine	El tratamiento de la toxicidad por mercurio
Swedish	Erdosteine	Behandling av kvicksilverförgiftning
Norwegian	Erdostein	Behandling av kvikksølvforgiftning
Icelandic	Erdosteine	Meðferð kvikasilfurs eitrunar

¹ At the time of designation