10 May 2017
EMA/208446/2017
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Emeramide for the prevention of mercury toxicity

On 20 April 2017, orphan designation (EU/3/17/1864) was granted by the European Commission to NBMI Science Limited, Ireland, for emeramide for the prevention of mercury toxicity.

What is mercury toxicity?

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

Mercury toxicity can affect many parts of the body, including the nervous and digestive systems, lungs, and kidneys. Short-term exposure 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 debilitating condition due to the damage it can cause to various parts of the body. It can be life-threatening following exposure to high doses.

What is the estimated number of patients at risk of developing the condition?

At the time of designation, the number of people at risk of mercury toxicity was estimated to be less than 0.1 people in 10,000 per year in the European Union (EU). This was equivalent to a total of fewer than 5,000 people per year[*], and is below the ceiling for orphan designation. 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 at risk of developing 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 515,700,000 (Eurostat 2017).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

What methods of prevention are available?

At the time of designation, no methods were authorised in the EU for the prevention of mercury toxicity. Prevention of mercury toxicity was limited to avoiding mercury exposure. Some medicines (known as chelating agents) were authorised in the EU for treating mercury toxicity but it was not clear if they could be used for prevention.

How is this medicine expected to work?

Emeramide is a chelating agent: it works by attaching to mercury to form a safer compound that can be more easily eliminated from the body. While other chelating agents are mostly 'hydrophilic' (water soluble) and cannot pass through cell membranes and attach to the mercury inside cells, emeramide is 'lipophilic' (fat soluble). Therefore it is expected to be able to reach the mercury inside cells in the body including the brain, forming a compound which the body is then able to eliminate. Each dose of emeramide is expected to prevent mercury toxicity for about 24 hours.

What is the stage of development of this medicine?

The effects of emeramide have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with emeramide in people at risk of mercury toxicity were ongoing.

At the time of submission, emeramide was not authorised anywhere in the EU for prevention of mercury toxicity. Orphan designation of emeramide (under the name N,N'-bis(2-mercaptoethyl)isophthalamide) had been granted in the EU and in the United States for treatment of mercury toxicity.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 March 2017 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, on the medicine's rare disease designations page.

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	Emeramide	Prevention of mercury toxicity
Bulgarian	Емерамид	Профилактика на живачна токсичност
Croatian	Emeramid	Prevencija otrovanja živom
Czech	Emeramid	Prevence toxicity rtuti
Danish	Emeramid	Forebyggelse af kviksølv toksicitet
Dutch	Emeramide	Preventie van kwikvergiftiging
Estonian	Emeramiid	Elavhõbeda toksilisuse preventsioon
Finnish	Emeramidi	Elohopeamyrkytyksen ennaltaehkäisy
French	Emeramide	Prévention de la toxicité au mercure
German	Emeramide	Prävention einer Quecksilbervergiftung
Greek	Εμεραμίδη	Πρόληψη της τοξικότητας υδραργύρου
Hungarian	Emeramid	Higanymérgezés megelőzése
Italian	Emeramide	Prevenzione della tossicità da mercurio
Latvian	Emeramīds	Dzīvsudraba toksicitātes novēršana
Lithuanian	Emeramidas	Gyvsidabrio toksiškumo prevencija
Maltese	Emeramid	Prevenzjoni ta' tossiċità bil-merkurju
Polish	Emeramid	Zapobieganie zatruć rtęcią
Portuguese	Emeramida	Prevenção da intoxicação pelo mercúrio
Romanian	Emeramidă	Prevenirea intoxicaţiei cu mercur
Slovak	Emeramide	Prevencia otravy ortuťou
Slovenian	Emeramide	Preprečevanje zastrupitev z živim srebrom
Spanish	Emeramide	Prevención de la toxicidad por mercurio
Swedish	Emeramide	Förebyggande av kvicksilverförgiftning
Norwegian	Emeramid	Forebygging av kvikksølvforgiftning
Icelandic	Emeramíð	Forvörn kvikasilfurs eitrunar

[1] At the time of designation