European Medicines Agency
*Pre-authorisation Evaluation of Medicines for Human Use*

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***Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in December 2008 on request of the Sponsor.***

## Committee for Orphan Medicinal Products

# Public summary of positive opinion for orphan designation of sodium dichloroactetate for the treatment of systemic monochloroacetate poisoning

On 2 September 2004, orphan designation (EU/3/04/225) was granted by the European Commission to Dr Anthony W. Fox, Germany, for sodium dichloroacetate for the treatment of systemic monochloroacetate poisoning.

**What is systemic monochloroacetate poisoning?**
Industrial activities are a potential source of accidental exposure to a substance called monochloracetate. Monochloracetate might enter the body through the skin (most importantly) but also by breathing or eating.
Systemic monochloroacetate poisoning is characterised by accumulation of an organic acid (lactate) in the blood, which leads to nausea, vomiting, difficulties in breathing, low blood pressure, seizures and confusion. The condition is life-threatening.

**What is the estimated number of patients affected by the condition*?**
At the time of designation, systemic monochloroacetate poisoning affected not more than 0.01 in 10,000 people in the European Union (EU)*. This is equivalent to a total of not more than 500 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

**What treatments are available?**
Methods that were used at the time of application consisted of decontamination of skin through chemical neutralisation of the poison and general supportive measures. No satisfactory methods exist that were authorised at the time of application.

**How is this medicine expected to work?**
Sodium dichloroacetate would act as an antidote (counteracting the effects of the poison) and directly oppose the harmful systemic action of monochloroacteate by inducing breakdown of the accumulated lactate.

**What is the stage of development of this medicine?**
The effects of sodium dichloroacetate were evaluated in experimental models.

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004).

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At the time of submission of the application for orphan designation, no clinical trials in patients with systemic monochloroacetate poisoning were initiated.

The medicinal product was not marketed anywhere worldwide for systemic monochloroacetate poisoning, at the time of submission. Orphan designation of sodium dichloroacetate was granted in the United States as an antidote in the management of systemic monochloroacetic acid poisoning.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 22 July 2004 a positive opinion recommending the grant of the above-mentioned designation.

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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 Community) 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:**

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Patients' associations contact points: Not available

**Translations of the active ingredient and indication in all EU languages and Norwegian and Icelandic**

| Language | Active Ingredient | Indication |
|---|---|---|
| English | Sodium dichloroacetate | Treatment of systemic monochloroacetate poisoning |
| Czech | Natriumdichlóracetát | Léčba otravy monochlóracetátem |
| Danish | Natrium dichloroacetat | Behandling af systemisk monochloracetat forgiftning |
| Dutch | Natrium dichlooroacetaat | Behandeling van systemische monochloroacetaat vergiftiging |
| Estonian | Naatrium diklooratsetaat.e | Süsteemse monoklooratsetaadi mürgistuse ravi |
| Finnish | Natriumdiklooriasetaatti | Monoklooriasetaattimyrkytyksen hoito |
| French | Dichloroacetate de sodium | Traitement de l'intoxication systémique au monochloroacetate |
| German | Natrium dikloroacetate | Behandlung von systemischer Monochloracetatvergiftung |
| Greek | Διχλωροξεικό νάτριο | Θεραπεία κατά της συστηματικής μονοχλωροξεικής δηλητηρίασης |
| Hungarian | Diklóracetát nátrium | Szisztémás monoklóracetát mérgezés kezelése |
| Italian | Dicloroacetato di sodio | Trattamento dell'avvelenamento sistemico da monocloroacetato |
| Latvian | Nātrija dihloracetāts | Sistēmiskas saindēšanās ar monohloracetātu ārstēšana |
| Lithuanian | Natrio dichloroacetatas | Sisteminio apsinuodijimo monochloroacetatu gydymas |
| Maltese | Sodium dichloroacetate | Treatment of systemic monochloroacetate poisoning |
| Polish | Dichlorooctan sodu | Leczenie ogólnoustrojowego zatrucia monocholooctanem |
| Portuguese | Dicloroacetato de sódio | Tratamento do envenenamento sistémico por monocloroacetato |
| Slovak | Dichlóracetát sodný | Liečba systémovej intoxikácie monochloracetátom |
| Slovenian | Natrij ev dikloracetate | Zdravljenje sistemske zastrupitve z monokloracetatom |
| Spanish | Dicloroacetato de sodio | Tratamiento del envenenamiento por monocloroacetato por vía sistémica |
| Swedish | Natriumdicklooracetat | Behandling av systemisk monokloracetatfärgiftning |
| Norwegian | Natriumdikloracetat | Behandling av systemisk monokloracetat-forgiftning |
| Icelandic | Natrium dichloroacetate | Meðhöndlun af kerfabundinn monochloroacetate eitrun |