



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Mifamurtide for the treatment of echinococcosis

On 14 July 2016, orphan designation (EU/3/16/1699) was granted by the European Commission to Delta Proteomics SAS, France, for mifamurtide for the treatment of echinococcosis.

#### What is echinococcosis?

Echinococcosis is a tapeworm disease caused by eating or drinking food or water contaminated with a type of tapeworm larva (tapeworm that are newly hatched from eggs) or by being in close contact with an infected animal. Two important types of the disease are cystic echinococcosis and alveolar echinococcosis, where a large number of tapeworms form a mass in the liver (called a hydatid cyst) or in the lungs. Other less common types of the disease affect organs such as kidneys, spleen, muscles, bones, eyes, brain and spinal cord.

Echinococcosis in the liver can cause pain, nausea and vomiting, and block the biliary duct (the tube that carries bile and digestive enzymes to the gut). In the lung, the disease causes cough, chest pain and shortness of breath. The cyst can burst and cause fever, skin reactions, severe allergic reaction, and the disease can spread to other parts of the body.

Echinococcosis is long-term debilitating and life-threatening because it can cause liver failure and severe allergic reactions.

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

At the time of designation, echinococcosis affected approximately 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 5,000 people<sup>\*</sup>, 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).

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<sup>\*</sup>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. This represents a population of 513,700,000 (Eurostat 2016).



## **What treatments are available?**

At the time of designation, echinococcosis was generally treated with surgery or procedures to remove the cyst. The medicine albendazole was authorised in some EU countries for treating echinococcosis when the cyst could not be removed.

The sponsor has provided sufficient information to show that mifamurtide might be of significant benefit for patients with echinococcosis because laboratory studies showed that when used in combination with albendazole it decreased cyst growth. 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?**

Mifamurtide is expected to work by activating macrophages (a type of white blood cells) and other components of the body's immune (defence) system that can destroy foreign organisms such as tapeworms. The mifamurtide in this medicine is contained between layers of fat-based substances. Mifamurtide is released slowly as these fatty layers break down in the cells. Therefore, mifamurtide, given in combination with a tapeworm medicine, might have a long-lasting effect against tapeworm larvae.

## **What is the stage of development of this medicine?**

The effects of mifamurtide have been evaluated in experimental models.

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

At the time of submission, mifamurtide was authorised in the EU for severe osteosarcoma (a type of bone cancer).

At the time of submission, it was not authorised anywhere in the EU for echinococcosis 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 16 June 2016 recommending the granting of this 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 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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Mifamurtide	Treatment of echinococcosis
Bulgarian	Мифамуртид	Лечение на ехинококоза
Croatian	Mifamurtid	Liječenje ehinokokoze
Czech	Mifamurtid	Léčba echinokokózy
Danish	Mifamurtid	Behandling af ekinokokkose
Dutch	Mifamurtid	Behandeling van echinokokkose
Estonian	Mifamurtiid	Ehhinokokoosi ravi
Finnish	Mifamurtidi	Ekinokokkoosin hoito
French	Mifamurtide	Traitement de l'échinococcose
German	Mifamurtid	Behandlung von Echinokokkose
Greek	Μιφαμουρτιδης	Θεραπεία της εχινokokκίωσης
Hungarian	Mifamurtid	Echinococcosis kezelése
Italian	Mifamurtide	Trattamento dell' echinococcosi
Latvian	Mifamurtīds	Ehinokokoze ārstēšana
Lithuanian	Mifamurtidas	Echinokokozės gydymas
Maltese	Mifamurtide	Kura tal-ekinokokkosi
Polish	Mifamurtyd	Leczenie echinokokozy
Portuguese	Mifamurtido	Tratamento da equinococose
Romanian	Mifamurtid	Tratamentul echinococozei
Slovak	Mifamurtidu	Liečba echinokokózu
Slovenian	Mifamurtid	Zdravljenje ehinokokoze
Spanish	Mifamurtida	Tratamiento de la equinococosis
Swedish	Mifamurtid	Behandling av echinococcosis
Norwegian	Mifamurtide	Behandling av echinococcosis
Icelandic	Mífamúrtíð	Meðferð á sullaveiki

<sup>1</sup> At the time of designation