2 March 2015
EMA/COMP/786341/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sodium thiosulfate for the treatment for calciphylaxis

On 15 January 2015, orphan designation (EU/3/14/1414) was granted by the European Commission to Hope Pharmaceuticals, Ltd, United Kingdom, for sodium thiosulfate for the treatment of calciphylaxis.

What is calciphylaxis?

Calciphylaxis, also known as calcific uraemic arteriolopathy, is a severe and progressive disease mainly seen in patients with end-stage kidney disease (when the kidneys have stopped working). It involves the build-up of calcium in very small arteries resulting in a restricted blood supply and small clots. The skin develops ulcers (sores) that do not heal and usually cause severe pain.

Calciphylaxis is a long-term debilitating and life-threatening condition, particularly due to the deep, painful, non-healing ulcers and the risk of infection.

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

At the time of designation, calciphylaxis affected less than 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 26,000 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?

At the time of designation, no satisfactory methods were authorised in the EU for the treatment of calciphylaxis. Treatments included medicines to reduce the build-up of calcium in the arteries, skin wound management and surgery.

How is this medicine expected to work?

Sodium thiosulfate is a medicine that has been used for many years in the EU for the treatment of poisoning. In patients with calciphylaxis it is expected to act as a 'calcium chelator'. This means that it

*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 511,100,000 (Eurostat 2014).

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is expected to attach to calcium to form a compound which is easily eliminated in the urine, thereby reducing the build-up of calcium in the arteries seen in patients with calciphylaxis. It is also thought to act as an antioxidant (a molecule that may prevent damage to cells caused by other molecules known as 'free radicals'), which may help restore the healthy functioning of cells lining the interior walls of the arteries.

What is the stage of development of this medicine?

The effects of sodium thiosulfate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with sodium thiosulfate in patients with calciphylaxis were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for calciphylaxis. Orphan designation of the medicine had been granted in the United States of America for the treatment of uremic and non-uremic calciphylaxis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 December 2014 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:

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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Sodium thiosulfate	Treatment for calciphylaxis
Bulgarian	Натриев тиосулфат	Лечение калцифилаксия
Croatian	Natrijev tiosulfat	Liječenje kalcifilaksije
Czech	Thiosulfát sodný	Terapie kalcifylaxe
Danish	Natriumthiosulfat	Behandling af kalcifylaksi
Dutch	Natriumthiosulfaat	Behandeling van calciphylaxis
Estonian	Naatriumtiosulfaat	Kaltsifülaksi ravi
Finnish	Natriumtiosulfaatti	Kalsifylaksian hoito
French	Thiosulfate de sodium	Traitement de la calciphylaxie
German	Natriumthiosulfat	Behandlung der Kalziphylaxie
Greek	Θειοθειικὸ νάτρο	Θεραπεία καλσιφύλαξης
Hungarian	Nátrium-tioszulfát	Calciphylaxis kezelése
Italian	Tiosolfato di sodio	Trattamento della calciphylaxis
Latvian	Nātrija tiosulfāts	Kalcifilakses ārstēšana
Lithuanian	Natrio tiosulfatas	Kalcifilaksijos gydymas
Maltese	Sodium thiosulphate	Kura tal-kalċifilassi
Polish	Tiosiarczan sodu	Leczenie kalcyfilaksji
Portuguese	Tiossulfato de sódio	Tratamento da calcifilaxia
Romanian	Tiosulfat de sodiu	Tratamentul calcifilaxiei
Slovak	Tiosíran sodný	Liečba kalcifylaxie
Slovenian	Natrijev tiosulfat	Zdravljenje kalcifilaksije
Spanish	Tiosulfato de sodio	Tratamiento de calcifilaxia
Swedish	Natriumtiosulfat	Behandling av kalcifylaxis
Norwegian	Natriumtiosulfat	Behandling af kalsifylaksi
Icelandic	Natríum thíosúlfat	Meðhöndlun calciphylaxis

[1] At the time of designation