

24 April 2012
EMA/COMP/142664/2012
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sodium thiosulfate for the treatment of calciphylaxis

On 2 April 2012, orphan designation (EU/3/12/979) was granted by the European Commission to Aptiv Solutions (UK) Limited, 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 the very small arteries causing a restricted blood supply and small clots. The skin is affected with the appearance of skin 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 is equivalent to a total of fewer than 25,000 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 application, no satisfactory methods were authorised in the EU to treat calciphylaxis. Treatments included medicines to reduce the build up of calcium in the arteries, skin wound management and surgery.

*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).

How is this medicine expected to work?

Sodium thiosulfate is expected to act as a 'calcium chelator'. This means that it 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?

At the time of submission of the application for orphan designation, no preclinical studies in experimental models had been performed. However, the sponsor provided results of studies from the published literature.

At the time of submission, no clinical trials with sodium thiosulfate in patients with calciphylaxis had been started.

At the time of submission, sodium thiosulfate was not authorised anywhere in the EU for calciphylaxis 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 8 February 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 ingredient	Indication
English	Sodium thiosulfate	Treatment of calciphylaxis
Bulgarian	Натриев тиосулфат	Лечение калцифилаксия
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	Θειοθειικό νάτριο	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-kalcifilassi
Polish	Tiosiarczan sodu	Leczenie kalcyfilaksji
Portuguese	Tiosulfato 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