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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Hexasodium phytate for the treatment of calciphylaxis

On 17 July 2012, orphan designation (EU/3/12/1026) was granted by the European Commission to Sanifit Laboratoris, S.L., Spain, for hexasodium phytate 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 resulting in 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 approximately 0.5 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 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).

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How is this medicine expected to work?

Hexasodium phytate is expected to interfere with the formation of the calcium crystals seen in the blood vessels of patients with calciphylaxis. It does this by attaching to the calcium salt 'calcium phosphate' in the crystals present in the blood vessels. This is expected to reduce further calcium salt attaching to the existing crystals, thereby reducing the build-up of calcium in the blood vessels.

What is the stage of development of this medicine?

The effects of hexasodium phytate have been evaluated in experimental models.

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

At the time of submission, hexasodium phytate 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 13 June 2012 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	Hexasodium phytate	Treatment of calciphylaxis
Bulgarian	Хексанатриев фитат	Лечение калцифилаксия
Czech	Fytát sodný	Terapie kalcifylaxe
Danish	Hexanatrium phytat	Behandling af kalcifylaksi
Dutch	Hexanatriumfytaat	Behandeling van calciphylaxis
Estonian	Heksanaatrium fütaat	Kaltsifülaksi ravi
Finnish	Heksanatriumfytaatti	Kalsifylaksian hoito
French	Phytate d'hexasodium	Traitement de la calciphylaxie
German	Hexanatriumphytat	Behandlung der Kalziphylaxie
Greek	Φυτικό εξανάτριο	Θεραπεία καλσιφύλαξης
Hungarian	Hexa-nátrium-fitát	Calciphylaxis kezelése
Italian	Fitato di esasodio	Trattamento della calcifilassi
Latvian	Heksanātrija fitāts	Kalcifilakses ārstēsana
Lithuanian	Heksanatrio fitatas	Kalcifilaksijos gydymas
Maltese	Hexasodium phytate	Kura tal-kalċifilassi
Polish	fitynian sodowy	Leczenie kalcyfilaksji
Portuguese	Fitato de hexasódio	Tratamento da calcifilaxia
Romanian	Fitat de hexasodiu	Tratamentul calcifilaxiei
Slovak	Fytát hexasodný	Liečba kalcifylaxie
Slovenian	6-natrijev fitat	Zdravljenje kalcifilaksije
Spanish	Fitato hexasódico	Tratamiento de calcifilaxia
Swedish	Hexanatriumfytat	Behandling av kalcifylaxis
Norwegian	Heksanatriumfytat	Behandling af kalsifylaksi
Icelandic	Hexanatríumfýtat	Meðhöndlun calciphylaxis

[1] At the time of designation