



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sodium nitrite for the treatment of aneurysmal subarachnoid haemorrhage

On 16 January 2014, orphan designation (EU/3/13/1224) was granted by the European Commission to Hope Pharmaceuticals, Ltd., United Kingdom, for sodium nitrite for the treatment of aneurysmal subarachnoid haemorrhage.

What is aneurysmal subarachnoid haemorrhage?

Aneurysmal subarachnoid haemorrhage is a form of stroke that occurs when part of the wall of a blood vessel in the brain that has weakened and expanded (a cerebral aneurysm) subsequently bursts, so that bleeding occurs into the subarachnoid space (the space between the 'arachnoid membrane' and the 'pia mater', two membranes that surround the brain) damaging the brain tissue. In addition, blood vessels near the site of the aneurysm go into spasm (vasospasm) by reflex reducing blood supply to the brain and therefore the supply of oxygen and essential nutrients to brain cells.

Aneurysms in the brain are considered to be acquired (they are not present at birth but develop over a lifetime). However, evidence indicates that genetic factors make some people more likely to develop them.

Aneurysmal subarachnoid haemorrhage is life threatening and can be debilitating because it could lead to oxygen shortage in the brain cells and thus to impairment of the brain functions.

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

At the time of designation, aneurysmal subarachnoid haemorrhage affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 51,000 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).

*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 512,200,000 (Eurostat 2013).



What treatments are available?

At the time of designation, nimodipine was authorised in the European Union for the prevention and treatment of complications due to vasospasm following subarachnoid haemorrhage.

The sponsor has provided sufficient information to show that sodium nitrite might be of significant benefit for patients with aneurysmal subarachnoid haemorrhage because early results in patients suggest it may be beneficial in treating vasospasm when used in combination with nimodipine. 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?

Sodium nitrite is converted in the body into nitric oxide, which relaxes and widens blood vessels, thus helping to overcome the effects of vasospasm. By preventing or reversing vasospasm, sodium nitrite could help to avoid oxygen shortage to the brain cells and thus the impairment of brain functions.

What is the stage of development of this medicine?

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

At the time of submission of the application for orphan designation, clinical trials with sodium nitrite in patients with aneurysmal subarachnoid haemorrhage had started.

At the time of submission, sodium nitrite was not authorised anywhere in the EU for aneurysmal subarachnoid haemorrhage. Orphan designation of sodium nitrite had been granted in the United States for the prevention of vasospasm associated with subarachnoid haemorrhage.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 December 2013 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 nitrite	Treatment of aneurysmal subarachnoid haemorrhage
Bulgarian	натриев нитрит	Лечение на субарахноидален кръвоизлив, причинен от аневризма
Czech	Dusitan sodný	Léčba subarachnoidálního krvácení z aneurysmatu
Croatian	Natrijev nitrit	Liječenje aneurizmatskog subarahnoidalnog krvarenja
Danish	Natriumnitrit	Behandling af aneurysmatisk subaraknoidalblødning
Dutch	Natriumnitriet	Behandeling van aneurysmatische subarachnoïdale bloeding
Estonian	Naatriumnitrit	Aneurüsmaatilise subarahnoidse e hemorraagia ravi
Finnish	Natriumnitriitti	Lukinkalvoon liittyvän valtimoverenvuodon hoito
French	Nitrate de sodium	Traitement de l'hémorragie subarachnoïdienne par rupture d'anévrisme
German	Natriumnitrit	Behandlung von aneurysmalen Subarachnoidalblutungen
Greek	νιτρῶδες νάτριο	Θεραπεία της ανευρυσματικής υπαραχνοειδούς αιμορραγίας
Hungarian	Nátrium-nitrit	Aneurysmális eredetű subarachnoidealis vérzés kezelése
Italian	Nitrito di sodio	Trattamento dell'emorragia da aneurisma subaracnoideo
Latvian	Nātrija nitrīts	Subarahnoidāla asinsizplūduma no aneirismas ārstēšana
Lithuanian	Natrio nitritas	Aneurizminės subarachnoidinės hemoragijos gydymas
Maltese	Sodium nitrite	Kura ta' emorraġija minn aneurizma subaraknojde
Polish	Azotyn sodu	Leczenie krwotoku podpajęczynówkowego tętniaka
Portuguese	Nitrito de sódio	Tratamento da hemorragia sub-aracnoideia aneurismática
Romanian	Nitrit de sodiu	Tratamentul hemoragiei subarahnoidiane aneurismală
Slovak	Dusitan sodný	Liečba subarachnoidálneho krvácania z aneurizmy
Slovenian	Natrijev nitrit	Zdravljenje anevrizemske subarahnoidalne krvavitve
Spanish	Nitrito de sodio	Tratamiento de la hemorragia subaracnoidea por rotura de aneurisma
Swedish	Natriumnitrit	Behandling av aneurysmal subaraknoidalblødning
Norwegian	Natriumnitritt	Behandling av aneurysmal subaraknoidalblødning
Icelandic	Natríum nítrít	Meðferð æðagúlsinnanskúms blæðingar

¹ At the time of designation