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Public summary of opinion on orphan designation

Fasudil hydrochloride for the treatment of non-traumatic subarachnoid haemorrhage

On 27 July 2020, orphan designation EU/3/20/2300 was granted by the European Commission to Aneuryst (Ireland) Limited, Ireland, for fasudil hydrochloride for the treatment of non-traumatic subarachnoid haemorrhage.

What is non-traumatic subarachnoid haemorrhage?

Non-traumatic subarachnoid haemorrhage is bleeding into the subarachnoid space (the space between two membranes that surround the brain). The bleeding is not due to an injury to the head but is usually caused by the bursting of a weakened blood-vessel wall in the brain (a cerebral aneurysm). It is a form of stroke, and the bleeding increases pressure on the brain and damages surrounding brain tissue. In addition, blood vessels near the site of the aneurysm go into spasm (vasospasm), reducing the supply of oxygen and essential nutrients to brain cells from the blood. Patients may develop seizures (fits) and other complications including heart problems.

Non-traumatic subarachnoid haemorrhage is life threatening and debilitating because of brain damage, impairment of brain functions and heart problems.

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

At the time of designation, non-traumatic subarachnoid haemorrhage affected approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 52,000 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?

Treatment of subarachnoid haemorrhage involves surgery or a procedure using a catheter to close or block off the bleeding blood vessel. At the time of designation, nimodipine, a medicine that relaxes

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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blood vessels and lowers blood pressure, was authorised in the EU for the prevention and treatment of complications due to vasospasm following subarachnoid haemorrhage.

The sponsor has provided sufficient information to show that the fasudil hydrochloride might be of significant benefit for patients with non-traumatic subarachnoid haemorrhage. Early data suggest that patients treated with the medicine would have reduced occurrence of vasospasms and improved symptoms compared 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?

Fasudil hydrochloride blocks an enzyme called Rho kinase which is involved in the contraction of smooth muscles, such as those in brain arteries. By blocking Rho kinase, the medicine relaxes brain arteries which are abnormally constricted in patients who have had a subarachnoid haemorrhage. This is expected to ease the supply of oxygen and essential nutrients to brain cells from the blood and reduce the symptoms of the disease.

What is the stage of development of this medicine?

The effects of fasudil hydrochloride have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with non-traumatic subarachnoid haemorrhage had finished.

Fasudil hydrochloride is authorised in Japan and China for the treatment of subarachnoid haemorrhage.

At the time of submission, fasudil hydrochloride was not authorised anywhere in the EU for the treatment of non-traumatic subarachnoid haemorrhage. Orphan designation of fasudil hydrochloride had been granted in the United States for the condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 18 June 2020, 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

Contact details of the current sponsor for this orphan designation can be found on EMA website.

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	Fasudil hydrochloride	Treatment of non-traumatic subarachnoid haemorrhage
Bulgarian	Фасудил хидрохлорид	Лечение на нетравматичен субархноидален кръвоизлив
Croatian	Fasudil hidroklorid	Liječenje netraumatskog subarahnoidalnog krvarenja
Czech	Fasudil hydrochlorid	Léčba netraumatického subarachnoidálního krvácení
Danish	Fasudilhydrochlorid	Behandling af non-traumatisk subarachnoidalblødning
Dutch	Fasudilhydrochloride	Behandeling van niet-traumatische subarachnoïdale bloeding
Estonian	Fasudiilvesinikkloriid	Mittetraumaatilise subarahnoidaalse hemorraagia ravi
Finnish	Fasudiilihydrokloridi	Ei-traumaattisen subaraknoidaalisen verenvuodon (SAV) hoito
French	Chlorhydrate de fasudil	Traitement d'hémorragie sous-arachnoïdienne non traumatique
German	Fasudilhydrochlorid	Behandlung der nichttraumatischen Subarachnoidalblutung
Greek	Φασουδίλη υδροχλωρική	Θεραπεία της μη τραυματικής υπαραχνοειδούς αιμορραγίας
Hungarian	Fasudil-hidroklorid	Nem traumás szubarachnoidális vérzés kezelése
Italian	Fasudil cloridrato	Trattamento dell'emorragia subaracnoidea non traumatica
Latvian	Fasudila hidrohlorīds	Netraumatiska subarahnoidāla asinsizplūduma ārstēšana
Lithuanian	Fasudilio hidrochloridas	Netrauminės subarachnoidinės hemoragijos gydymas
Maltese	Kloridrat tal-fasudil	Trattament ta' emorraġija subaraknojde mhux trawmatika
Polish	Chlorowodorek fasudilu	Leczenie nietraumatycznego krwotoku podpajęczynówkowego
Portuguese	Cloridrato de fasudil	Tratamento da hemorragia subaracnoideia não traumática
Romanian	Clorhidrat de fasudil	Tratamentul hemoragiei subarahnoidiană netraumatică
Slovak	Fasudil hydrochlorid	Liečba netraumatickej subarachnoidálnej hemorágie

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	Fasudil hidroklorid	Zdravljenje netravmatske subarahnoidne krvavitve
Spanish	Clorhidrato de fasudil	Tratamiento de la hemorragia subaracnoidea no traumatica
Swedish	Fasudilhydroklorid	Behandling av icke-traumatisk subarachnoid blödning
Norwegian	Fasudilhydroklorid	Behandling av ikke-traumatisk subaraknoidalblødning
Icelandic	Fasudil hýdróklóríð	Meðferð við innanskúmsblæðingu sem ekki er vegna áverka