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Public summary of opinion on orphan designation

Lumacaftor for the treatment of non-traumatic subarachnoid haemorrhage

On 4 June 2020, orphan designation EU/3/20/2279 was granted by the European Commission to Qanatpharma GmbH, Germany, for lumacaftor for the treatment of non-traumatic subarachnoid haemorrhage.

What is non-traumatic subarachnoid haemorrhage?

Non-traumatic subarachnoid haemorrhage is a form of stroke that occurs due to bleeding into the subarachnoid space (the space between two membranes that surround the brain) without previous trauma. Most times it occurs when the wall of a blood vessel in the brain that has weakened and expanded over time (a cerebral aneurysm) subsequently bursts, causing bleeding into the subarachnoid space and damaging the brain tissue. In addition, blood vessels near the site of the aneurysm go into spasm (vasospasm), 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.

Non-traumatic subarachnoid haemorrhage is life threatening and can be debilitating because it can lead to lack of oxygen in the brain and thus impairment of brain functions.

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

At the time of designation, non-traumatic subarachnoid haemorrhage affected approximately 1.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 62,000 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).

*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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What treatments are available?

At the time of designation, nimodipine 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 lumacaftor might be of significant benefit for patients with non-traumatic subarachnoid haemorrhage because early studies in experimental models indicate that the medicine could give better results than 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?

Lumacaftor, in combination with ivacaftor, is already authorised for the treatment of cystic fibrosis, a disease caused when a protein called CFTR is not working properly. It is thought that CFTR may also be involved in the functioning of blood vessels and that, following subarachnoid haemorrhage, reduction of the amount of CFTR on the surface of cells could cause narrowing of small blood vessels and decrease blood supply to the brain. Lumacaftor increases the amount of CFTR on the surface of cells and is thereby expected to restore normal function of small blood vessels and increased blood flow to the brain, thereby improving symptoms of subarachnoid haemorrhage.

What is the stage of development of this medicine?

The effects of lumacaftor have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with lumacaftor in patients with non-traumatic subarachnoid haemorrhage had been started.

At the time of submission, lumacaftor was not authorised anywhere in the EU for the treatment of non-traumatic subarachnoid haemorrhage or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 23 April 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

Sponsor's contact details:

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	Lumacaftor	Treatment of non-traumatic subarachnoid haemorrhage
Bulgarian	Лумакафтор	Лечение на нетравматичен субарахноидален кръвоизлив
Croatian	Lumakaftor	Liječenje netraumatskog subarahnoidalnog krvarenja
Czech	Lumakaftor	Léčba netraumatického subarachnoidálního krvácení
Danish	Lumacaftor	Behandling af non-traumatisk subarachnoidalblødning
Dutch	Lumacaftor	Behandeling van niet-traumatische subarachnoïdale bloeding
Estonian	Lumakaftoor	Mittetraumaatilise subarahnoidaalse hemorraagia ravi
Finnish	Lumakaftori	Ei-traumaattisen SAV:n hoito
French	Lumacaftor	Traitement d'hémorragie sous-arachnoïdienne non traumatique
German	Lumacaftor	Behandlung der nichttraumatischen Subarachnoidalblutung
Greek	Lumacaftor	Θεραπεία της μη τραυματικής υπαραχνοειδούς αιμορραγίας
Hungarian	Lumakaftor	Nem traumás szubarachnoidális vérzés kezelése
Italian	Lumacaftor	Trattamento dell'emorragia subaracnoidea non traumatica
Latvian	Lumakaftors	Netraumatiska subarahnoidāla asinsizplūduma ārstēšana
Lithuanian	Lumakaftoras	Netrauminės subarachnoidinės hemoragijos gydymas
Maltese	Lumacaftor	Trattament ta' emorraġija subaraknojde mhux trawmatika
Polish	Lumakaftor	Leczenie nietraumatycznego krwotoku podpajęczynówkowego
Portuguese	Lumacaftor	Tratamento da hemorragia subaracnoideia não traumática
Romanian	Lumacaftor	Tratamentul hemoragiei subarahnoidiană netraumatică
Slovak	Lumakaftor	Liečba netraumatickej subarachnoidálnej hemorágie
Slovenian	Lumakaftor	Zdravljenje netravmatske subarahnoidne krvavitve
Spanish	Lumacaftor	Tratamiento de la hemorragia subaracnoidea no traumatica
Swedish	Lumakaftor	Behandling av icke-traumatisk subarachnoid blödning
Norwegian	Lumakaftor	Behandling av ikke-traumatisk subaraknoidalblødning
Icelandic	Lumacaftor	Meðferð við innanskúmsblæðingu sem ekki er vegna áverka

[1] At the time of designation