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Public summary of opinion on orphan designation

Concizumab for the treatment of haemophilia B

On 12 October 2017, orphan designation (EU/3/17/1940) was granted by the European Commission to Novo Nordisk A/S, Denmark, for concizumab for the treatment of haemophilia B.

What is haemophilia B?

Haemophilia B is an inherited bleeding disorder that is caused by the lack of factor IX, which is one of the proteins involved in the blood coagulation (clotting) process. Patients with haemophilia B are more prone to bleeding than normal and have poor wound healing after injury or surgery. Bleeding can also happen within muscles or the spaces in the joints, such as the elbows, knees and ankles. This can lead to permanent injury if it happens repeatedly.

Haemophilia B is a debilitating disease that is life long and may be life threatening because bleeding can also happen in the brain and spinal cord, the throat or the gut.

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

At the time of designation, haemophilia B affected approximately 0.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 10,000 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 designation, medicines containing factor IX were authorised in the EU for the treatment of haemophilia B, to replace the missing protein. However, factor IX medicines did not work in some patients with haemophilia B because the immune system (the body's natural defences) can produce 'inhibitors' (antibodies) against factor IX which stop the factor IX medicine from working. In these cases, other treatments needed to be used, such as factor VIIa (the activated form of factor VII, another protein involved in blood clotting), either alone or as part of a combination treatment.

*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 515,700,000 (Eurostat 2017).

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The sponsor has provided sufficient information to show that concizumab might be of significant benefit for patients with haemophilia B because the medicine will be given by injection under the skin, which is more convenient for patients than an injection into a vein, which is the way currently authorised products are given. 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?

This medicine is a monoclonal antibody (a type of protein) that has been designed to recognise, attach to and block a molecule in the body called 'tissue factor pathway inhibitor' (TFPI). TFPI controls another pathway for blood clotting that does not involve factor IX. By blocking TFPI, this medicine is expected to increase the ability of the blood to clot and help control the bleeding disorder, bypassing the need for factor IX.

What is the stage of development of this medicine?

The effects of concizumab 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 haemophilia B were ongoing.

At the time of submission, concizumab was not authorised anywhere in the EU for haemophilia B 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 5 October 2017 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, on the medicine's rare disease designations page.

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	Concizumab	Treatment of haemophilia B
Bulgarian	Концизумаб	Лечение на хемофилия B
Croatian	Koncizumab	Liječenje hemofilije B
Czech	Concizumab	Léčba hemofilie B
Danish	Concizumab	Behandling af hæmofili B
Dutch	Concizumab	Behandeling van hemofilie B
Estonian	Kontsizumaab	Hemofiilia B ravi
Finnish	Konsitsumabi	Hemofilia B:n hoito
French	Concizumab	Traitement de l'hémophilie B
German	Concizumab	Behandlung der Hämophilie B
Greek	Κονσιζουμάμπη	Θεραπεία της αιμορροφιλίας B
Hungarian	Koncizumab	B típusú hemofília kezelése
Italian	Concizumab	Trattamento dell'emofilia B
Latvian	Koncizumabs	B tipa hemofilijas ārstēšana
Lithuanian	Koncizumabas	Hemofilijos B gydymas
Maltese	Konċiżumab	Kura ta' l-emofilja B
Polish	Koncizumab	Leczenie hemofilii B
Portuguese	Concizumab	Tratamento da hemofilia B
Romanian	Concizumab	Tratamentul hemofiliei B
Slovak	Concizumab	Liečba hemofílie B
Slovenian	Koncizumab	Zdravljenje hemofilije B
Spanish	Concizumab	Tratamiento de la hemofilia B
Swedish	Koncizumab	Behandling av hemofili B
Norwegian	Koncizumab	Behandling av hemofili B
Icelandic	Concizúmab	Meðferð við dreyrasýki B

[1] At the time of designation