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Public summary of opinion on orphan designation

Marzeptacog alfa (activated) for the treatment of haemophilia B

On 1 April 2019, orphan designation (EU/3/19/2151) was granted by the European Commission to Voisin Consulting S.A.R.L., France, for marzeptacog alfa (activated) (also known as MarzAA) 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 life-long debilitating disease that may be life threatening because bleeding can 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 0.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 16,000 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 518,400,000 (Eurostat 2019).

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The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with haemophilia B. This is because studies in patients indicated that it might be possible for patients who have developed inhibitors to use the medicine routinely to prevent bleeding. 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?

Marzeptacog alfa (activated) is a version of factor VIIa which is expected to work in the same way as human factor VIIa in the body. Factor VIIa activates another factor called factor X, which starts the clotting process. By activating factor X, this medicine is expected to work in patients who have developed inhibitors to factor IX because it acts directly on factor X, independently of factor IX.

What is the stage of development of this medicine?

The effects of marzeptacog alfa (activated) 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, marzeptacog alfa (activated) was not authorised anywhere in the EU for haemophilia B. Orphan designation of the medicine had been granted in the United States for haemophilia A and B.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 21 February 2019 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 the 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	Marzeptacog alfa (activated)	Treatment of haemophilia B
Bulgarian	Марцептаког алфа (активиран)	Лечение на хемофилия B
Croatian	Marzeptakog alfa (aktivirani)	Liječenje hemofilije B
Czech	Marzeptacog alfa (aktivovaný)	Léčba hemofilie B
Danish	Marzeptacog alfa (activeret)	Behandling af hæmofili B
Dutch	Marzeptacog alfa (geactiveerd)	Behandeling van hemofilie B
Estonian	Marzeptacog alfa (aktiviseeritud)	Hemofiilia B ravi
Finnish	Martseptakogi alfa (aktivoitu)	Hemofilia B:n hoito
French	Marzeptacog alfa (activé)	Traitement de l'hémophilie B
German	Marzeptacog alfa (aktiviert)	Behandlung der Hämophilie B
Greek	Marzeptacog alfa (ενεργοποιημένο)	Θεραπεία της αιμορροφιλίας B
Hungarian	Marzeptacog alfa (aktivált)	B típusú hemofília kezelése
Italian	Marzeptacog alfa (attivato)	Trattamento dell'emofilia B
Latvian	Marzeptacog alfa (aktivēts)	B tipa hemofilijas ārstēšana
Lithuanian	Marzeptakogas alfa (aktyvintas)	Hemofilijos B gydymas
Maltese	Marzeptacog alfa (attivat)	Kura ta' l-emofilja B
Polish	Marzeptakog alfa (aktywowany)	Leczenie hemofilii B
Portuguese	Marzeptacog alfa (ativado)	Tratamento da hemofilia B
Romanian	Marzeptacog alfa (activat)	Tratamentul hemofiliei B
Slovak	Marzeptacog alfa (aktivovaný)	Liečba hemofílie B
Slovenian	Marzeptakog alfa (aktiviran)	Zdravljenje hemofilije B
Spanish	Marzeptacog alfa (activado)	Tratamiento de la hemofilia B
Swedish	Marzeptakog alfa (aktiverad)	Behandling av hemofili B
Norwegian	Marzeptakog alfa (aktivert)	Behandling av hemofili B
Icelandic	Marzeptacog alfa (virkjað)	Meðferð við dreyrasýki B

[1] At the time of designation