15 January 2019
EMA/755561/2018

Public summary of opinion on orphan designation

Fidanacogene elaparvovec for the treatment of haemophilia B

On 19 November 2018, orphan designation (EU/3/18/2090) was granted by the European Commission to Pfizer Europe MA EEIG, Belgium, for fidanacogene elaparvovec for the treatment of haemophilia B.

What is haemophilia B?

Haemophilia B is an inherited bleeding disorder caused by the lack of factor IX, a protein 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. Internal bleeding can also happen within muscles or in the joints, such as the elbows, knees and ankles. This can lead to permanent damage 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 0.3 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 517,400,000 (Eurostat 2018).

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The sponsor has provided sufficient information to show that fidanacogene elaparvovec might be of significant benefit for patients with haemophilia B. Preliminary results in patients show that a single dose can increase the amount of factor IX circulating in the blood in the long term, thus reducing bleeding and the need for treatment with factor IX medicines.

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?

Patients with haemophilia B cannot make enough working factor IX because the gene for producing the clotting factor is damaged.

This medicine is made of a virus that contains copies of the gene responsible for producing factor IX. When injected into the patient, it is expected that the virus will be carried into the liver cells and start producing factor IX. It is expected that a single dose of the medicine will maintain raised levels of factor IX for a long time, thereby reducing bleeding.

The type of virus used in this medicine ('adeno-associated virus') does not cause disease in humans.

What is the stage of development of this medicine?

The effects of fidanacogene elaparvovec 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, fidanacogene elaparvovec was not authorised anywhere in the EU for haemophilia B. Orphan designation of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 October 2018 recommending the granting of this designation.

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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	Fidanacogene elaparvovec	Treatment of haemophilia B
Bulgarian	Фиданакоген елапарвовец	Лечение на хемофилия B
Croatian	Fidanacogen elaparvovec	Liječenje hemofilije B
Czech	Fidanacogen elaparvovek	Léčba hemofilie B
Danish	Fidanacogen elaparvovec	Behandling af hæmofili B
Dutch	Fidanacogen elaparvovec	Behandeling van hemofilie B
Estonian	Fidanakogeenelaparvovek	Hemofiilia B ravi
Finnish	Fidanacogene elaparvovec	Hemofilia B:n hoito
French	Fidanacogène élaparvovec	Traitement de l'hémophilie B
German	Fidanacogen elaparvovec	Behandlung der Hämophilie B
Greek	Fidanacogene elaparvovec	Θεραπεία της αιμορροφιλίας B
Hungarian	Fidanakogén elaparvovek	B típusú hemofília kezelése
Italian	Fidanacogene elaparvovec	Trattamento dell'emofilia B
Latvian	Fidanakogēna elaparvoveks	B tipa hemofilijas ārstēšana
Lithuanian	Fidanakogeno *elaparvovec*	Hemofilijos B gydymas
Maltese	Fidanakoġen elaparvovek	Kura ta' l-emofilja B
Polish	Fidanakogen elaparwowek	Leczenie hemofilii B
Portuguese	Fidanacogene elaparvovec	Tratamento da hemofilia B
Romanian	Fidanacogen elaparvovec	Tratamentul hemofiliei B
Slovak	Fidanacogene elaparvovec	Liečba hemofílie B
Slovenian	Fidanakogen elaparvovek	Zdravljenje hemofilije B
Spanish	Fidanacogene elaparvovec	Tratamiento de la hemofilia B
Swedish	Fidanakogen-elaparvovek	Behandling av hemofili B
Norwegian	Fidanakogen elaparvovek	Behandling av hemofili B
Icelandic	Fidanacogen elaparvovec	Meðferð við dreyrasýki B

[1] At the time of designation