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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Adeno-associated viral vector containing the human factor IX gene for the treatment of haemophilia B

On 11 January 2012, orphan designation (EU/3/11/938) was granted by the European Commission to Amsterdam Molecular Therapeutics BV, the Netherlands, for adeno-associated viral vector containing the human factor IX gene for the treatment of haemophilia B.

In July 2012, Amsterdam Molecular Therapeutics BV changed name to uniQure biopharma B.V.

What is haemophilia B?

Haemophilia B is an inherited bleeding disorder caused by the lack of a substance called factor IX, one of the proteins involved in blood coagulation (clotting). Patients with haemophilia B are more prone to bleeding than normal and after injury or surgery their wounds do not heal normally. Bleeding can also happen within muscles or the spaces in the joints such as the elbows, knees and ankles, which can lead to permanent injury if it happens repeatedly.

Haemophilia B is a debilitating lifelong disease which may be life threatening because patients can suffer bleeding 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 between 0.1 and 0.3 in 10,000 people in the European Union (EU). This was equivalent to a total of between 5,100 and 15,000 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. These medicines replace the missing protein. In some patients, other treatments

*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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 509,000,000 (Eurostat 2012).

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needed to be used, including medicines containing other substances involved in blood clotting such as factor VIIa.

The sponsor has provided sufficient information to show that adeno-associated viral vector containing the human factor IX gene might be of significant benefit for patients with haemophilia B because it may reduce the need for regular administrations of the missing protein, thereby improving the treatment of patients. 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?

The medicine is made of a virus that contains the gene for factor IX, which is lacking in patients with haemophilia B. After being given to the patient as a single injection into a vein, the virus is expected to carry the factor IX gene into the liver cells, enabling them to produce the missing factor IX. This is expected to control the bleeding disorder.

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?

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicine in experimental models was ongoing.

At the time of submission, no clinical trials with the medicine in patients with haemophilia B had been started.

At the time of submission, the medicine 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 9 November 2011 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:

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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	Adeno-associated viral vector containing the human factor IX gene	Treatment of haemophilia B
Bulgarian	Адено-свързан вирусен вектор, съдържащ ген на човешки фактор IX	Лечение на хемофилия B
Czech	Adeno-asociovaný virový vektor obsahující gen pro humánní faktor IX	Léčba hemofilie B
Danish	Adenoassocieret viral vektor indeholdende det humane faktor IX-gen	Behandling af hæmofili B
Dutch	Adeno-geassocieerde virale vector welke het humaan factor IX gen bevat	Behandeling van hemofilie B
Estonian	Inimese IX faktori geeni sisaldav adenoviirusega assotsieerunud viirusvektor	Hemofiilia B ravi
Finnish	AAV-vektori, joka sisältää ihmisen hyytymistekijä IX -geenin	Hemofilia B:n hoito
French	Vecteur viral adéno-associé contenant le gène du facteur IX humain	Traitement de l'hémophilie B
German	Adeno-assoziierter viraler Vektor, der das humane Faktor-IX-Gen enthält	Behandlung der Hämophilie B
Greek	Ιικός φορέας σχετιζόμενος με αδενοϊό που περιέχει το ανθρώπινο γονίδιο για τον παράγοντα IX	Θεραπεία της αιμορροφιλίας B
Hungarian	Humán IX. faktor gént tartalmazó adeno-asszociált vírusvektor	B típusú hemofília kezelése
Italian	Vettore virale adeno-associato contenente il gene del fattore IX umano	Trattamento dell'emofilia B
Latvian	Adeno-asociētā vīrusa vektors, kas satur cilvēka IX asinsreces faktora gēnu	B tipa hemofīlijas ārstēšana
Lithuanian	Adenoasocijuoto viruso vektorius, turintis žmogaus IX faktoriaus geną	Hemofilijos B gydymas
Maltese	Vettur imnissel mill-adenovirus li fih il-ġene uman tal-fattur IX	Kura ta' l-emofilja B
Polish	Wektor adenowirusowy zawierający gen ludzkiego czynnika IX	Leczenie hemofilii B
Portuguese	Vector viral adeno-associado contendo o gene do factor IX humano	Tratamento da hemofilia B
Romanian	Vector viral adeno-asociat care conţine gena factorului IX uman	Tratamentul hemofiliei B
Slovak	Adeno-asociovaný vírusový vektor obsahujúci gén ľudského faktora IX	Liečba hemofílie B
Slovenian	Adenovirusni vektor, ki vsebuje gen humani faktorja IX	Zdravljenje hemofilije B

[1] At the time of designation

Language	Active ingredient	Indication
Spanish	Vector vírico adenoasociado que contiene el gen del factor IX humano	Tratamiento de la hemofilia B
Swedish	Adenoassocierad virusvektor innehållande genen för human faktor IX	Behandling av hemofili B
Norwegian	Adenoassosiert virusvektor som inneholder genet for human faktor IX	Behandling av hemofili B
Icelandic	Adenó-tengd veiruferja sem inniheldur manna tákna-bættan erfðavísi fyrir þátt IX	Meðferð við dreyrasýki B