23 July 2015
EMA/COMP/360279/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

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

withdrawn

On 19 June 2015, orphan designation (EU/3/15/1501) was granted by the European Commission to Baxalta Innovations GmbH, Austria, for adeno-associated viral vector containing the human factor IX gene 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, not all patients with haemophilia B could benefit from these medicines because the immune system (the body's natural defences) can react against them by producing 'inhibitors' (antibodies) against factor IX. In these cases, 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 28), Norway, Iceland and Liechtenstein. This represents a population of 512,900,000 (Eurostat 2015).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

An agency of the European Union

© European Medicines Agency, 2017. Reproduction is authorised provided the source is acknowledged.

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.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with haemophilia B. Data from early studies in experimental models showed that the medicine reduces bleeding without causing the body to produce the inhibitors that limit the use of some current treatments. 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 has been modified to contain the gene for factor IX, which is lacking in patients with haemophilia B. After being given once to the patient as an 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 for an extended period of time. 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?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with the medicine in patients with haemophilia B was ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for haemophilia B. Orphan designation of the medicine had been granted in the United States for haemophilia B.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 May 2015 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.

withdrawn

For more information

Sponsor's contact details:

Baxalta Innovations GmbH
Industriestrasse 67
A-1221 Vienna
Austria
Tel. +43 1 2010 0247 2542
Fax +43 1 2010 0247 5990
E-mail: Europe_BioSci_GlobalRa@baxter.com

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.

withdrawn

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
Croatian	Adeno-povezani virusni vektor koji sadrži ljudski gen za faktor IX	Liječenje hemofilije 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	Αδενο-σχετιζόμενος ιικός φορέας 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 hemofilijas 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

withdrawn