26 February 2015
EMA/COMP/527614/2012 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Vatreptacog alfa (activated) for the treatment of haemophilia A

First publication	25 September 2012
Rev.1: withdrawal from the Community Register	26 February 2015
Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in September 2014 on request of the Sponsor.

On 9 August 2012, orphan designation (EU/3/12/1030) was granted by the European Commission to Novo Nordisk A/S, Denmark, for vatreptacog alfa (activated) for the treatment of haemophilia A.

What is haemophilia A?

Haemophilia A is an inherited bleeding disorder that is caused by the lack of factor VIII, which is one of the proteins involved in the blood coagulation (clotting) process. Patients with haemophilia A 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 A is a debilitating disease that is life-long and may be life threatening because bleeding can also happen in the brain, the spinal cord, the joints or the gut.

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

At the time of designation, haemophilia A affected approximately 0.73 in 10,000 people in the European Union (EU). This was equivalent to a total of around 37,000 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 VIII were authorised in the EU for the treatment of haemophilia A, to replace the missing factor VIII protein. However, not all patients with haemophilia A could benefit from these medicines because the immune system (the body's natural defences) can react against them by producing 'inhibitors' (antibodies) against factor VIII. In these cases, other treatments were used, such as medicines containing other coagulation factors such as factor VIIa, either alone or as part of combination treatment.

The sponsor has provided sufficient information to show that vatreptacog alfa (activated) might be of significant benefit for patients with haemophilia A because early studies indicate that it could be used in haemophilia A patients who have developed inhibitors against factor VIII, to better control the bleeding episodes than existing medicines. The medicine is also expected to be given for a shorter period of time than existing treatments, which is expected to improve compliance with treatment. These assumptions 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?

Vatreptacog alfa (activated) is expected to work in the same way as human factor VIIa. In the body, factor VIIa is involved in blood clotting. It activates another factor called factor X, which starts the clotting process. By activating factor X, this medicine is expected to control the bleeding disorder in patients who have developed inhibitors to factor VIII because it acts directly on factor X, independently of factor VIII.

Vatreptacog alfa (activated) is made by a method known as 'recombinant DNA technology': it is made by a cell that has received the human gene (DNA) that makes it able to produce factor VIIa. The protein has also been modified: its sequence of amino acids (the building blocks of proteins) is slightly different from that of naturally occurring factor VIIa, which may result in it activating more factor X than existing medicines.

What is the stage of development of this medicine?

The effects of vatreptacog alfa (activated) have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with vatreptacog alfa (activated) in patients with haemophilia A were ongoing.

At the time of submission, vatreptacog alfa (activated) was not authorised anywhere in the EU for haemophilia A or designated as an orphan medicinal product elsewhere for this condition.

*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).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 July 2012 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	Vatreptacog alfa (activated)	Treatment of haemophilia A
Bulgarian	Ватрептаког алфа (активиран)	Лечение на хемофилия А
Czech	Vatreptakog alfa (aktivovaný)	Léčba hemofilie A
Danish	Vatreptacog alfa (aktiveret)	Behandling af hæmofili A
Dutch	Vatreptacog alfa (geactiveerd)	Behandeling van hemofilie A
Estonian	Vatreptakog alfa (aktiveeritud)	Hemofiilia A ravi
Finnish	Vatreptakogi alfa (aktivoitu)	Hemofilia A:n hoito
French	Vatreptacog alfa (activé)	Traitement de l'hémophilie A
German	Vatreptacog alfa (aktiviert)	Behandlung der Hämophilie A
Greek	Vatreptacog alfa (ενεργοποιημένος)	Θεραπεία της αιμορροφιλίας Α
Hungarian	Alfa-vatreptakog(aktivált)	A típusú hemofília kezelése
Italian	Vatreptacog alfa (attivato)	Trattamento dell'emofilia A
Latvian	Alfa vatreptakogs (aktivēts)	A tipa hemofīlijas ārstēšana
Lithuanian	Vatreptakogas alfa (aktyvintas)	Hemofilijos A gydymas
Maltese	Vatreptacog alfa (attivat)	Kura ta' l-emofilja A
Polish	Watreptakog alfa (aktywowany)	Leczenie hemofilii A
Portuguese	Vatreptacog alfa (ativado)	Tratamento da hemofilia A
Romanian	Vatreptacog alfa (activat)	Tratamentul hemofiliei A
Slovak	Vatreptakog alfa (aktivovaný)	Liečba hemofílie A
Slovenian	Vatreptakog alfa (aktivirani)	Zdravljenje hemofilije A
Spanish	Vatreptacog alfa (activado)	Tratamiento de la hemofilia A
Swedish	Vatreptacog alfa (aktiverad)	Behandling av hemofili A
Norwegian	Vatreptacog alfa (aktivert)	Behandling av hemofili A
Icelandic	Vatreptacog alfa (virkjað)	Meðferð við dreyrasýki A

[1] At the time of designation