29 October 2013
EMA/COMP/556625/2013
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant fusion protein linking coagulation factor VIIa with albumin for the treatment of congenital factor VII deficiency

On 7 October 2013, orphan designation (EU/3/13/1188) was granted by the European Commission to CSL Behring GmbH, Germany, for recombinant fusion protein linking coagulation factor VIIa with albumin for the treatment of congenital factor VII deficiency.

What is congenital factor VII deficiency?

Congenital factor VII deficiency is an inherited bleeding disorder that is characterised by the lack of factor VII, which is one of the proteins involved in the blood coagulation (clotting) process. Patients with factor VII deficiency 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.

Congenital factor VII deficiency is a debilitating disease that is life long and may be life threatening because bleeding can also happen in the brain.

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

At the time of designation, congenital factor VII deficiency affected not more than 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 5,000 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 VII were authorised in the EU for the treatment of congenital factor VII deficiency. These medicines were used to replace the missing factor VII protein. However, some of these medicines had to be given frequently during bleeding episodes.

*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 512,200,000 (Eurostat 2013).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8416
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

The sponsor has provided sufficient information to show that the medicine 'recombinant fusion protein linking coagulation factor VIIa with albumin' might be of significant benefit for patients with congenital factor VII deficiency because early studies have shown that it lasts longer inside the body and thus may be given less often than existing 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 copy of human factor VIIa (factor VII in its activated form), which is attached to albumin, a protein that acts as a carrier. In the body, the medicine is expected to replace the missing factor VII, making the patient less prone to bleeding.

Attaching albumin to factor VIIa is expected to decrease the rate at which the factor VIIa is cleared from the body, allowing injections to be given less frequently than medicines that contain only factor VIIa.

The medicine is made by a method known as 'recombinant DNA technology': it is made by cells into which a gene (DNA) has been introduced that makes them able to produce factor VIIa linked to albumin.

What is the stage of development of this medicine?

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

At the time of submission of the application for orphan designation, clinical trials with this medicine in patients with congenital factor VII deficiency were planned.

At the time of submission, this medicine was not authorised anywhere in the EU for the treatment of congenital factor VII deficiency. Orphan designation of this medicine has been granted in the United States of America for the treatment of congenital factor VII deficiency.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 September 2013 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:

CSL Behring GmbH
Emil-von-Behring-Str. 76
35041 Marburg
Germany
Tel. +49 642 139 12
Fax +49 642 139 85
E-mail: Marburg.rVIIa-FP@cslbehring.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.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Recombinant fusion protein linking coagulation factor VIIa with albumin	Treatment of congenital factor VII deficiency
Bulgarian	Рекомбинантен фузионен протеин, свързващ човешки коагулационен фактор VIIa албумин	Лечение на вроден дефицит на Фактор VII
Czech	Rekombinantní fúzní protein skládající se z lidského koagulačního faktoru VIIa navázaného na lidský albumin	Léčba vrozeného nedostatku faktoru VII
Croatian	Rekombinantni fuzijski protein kojeg čini faktor koagulacije VIIa vezan na albumin	Liječenje prirođenog nedostatka faktora VII
Danish	Rekombinant fusionsprotein, som binder koagulationsfaktor VIIa til albumin	Behandling af medfødt faktor VII-mangel
Dutch	Recombinant fusieproteïne van humane stollingsfactor VIIa met humane albumine	Behandeling van aangeboren factor VII tekort
Estonian	Rekombinantne sulandvalk, mis seob VIIa hüübimisfaktori albumiiniga.	Kaasasündinud VII hüübimisfaktori puudulikkuse ravi
Finnish	Rekombinantti fuusioproteiini, joka yhdistää ihmisen albumiinin hyytymistekijään VIIa	Synnynnäisen hyytymistekijä VII:n puutoksen hoito
French	Protéine de fusion recombinante liée au Facteur VIIa activé de coagulation et à l'albumine	Traitement des déficits congénitaux en facteur VII
German	Rekombinantes Fusionsprotein bestehend aus menschlichem Blutgerinnungsfaktor VIIa gebunden an humanes Albumin	Behandlung von angeborenem Faktor VII Mangel
Greek	Ανασυνδυασμένη πρωτεΐνη σύντηξης αποτελούμενη από ανθρώπινο παράγοντα πήξης VIIa συνδεδεμένο με την ανθρώπινη λευκωματίνη	Θεραπεία συγγενούς έλλειψης παράγοντα VII
Hungarian	Rekombináns VIIa-es human véralvadási faktor és human albumin fúziós fehérje	Veleszületett VII-es véralvadási faktor hiány kezelése
Italian	Proteina di fusione ricombinante costituita dal fattore VIIa di coagulazione del sangue umano collegato con albumina umana	Trattamento del deficit congenito del fattore VII
Latvian	Rekombinantais konjugētais proteīns, kurš savieno VIIa koagulācijas faktoru ar albumīnu	Iedzimta VII faktora deficīta ārstēšana
Lithuanian	Rekombinantinis sulietas baltymas, susidedantis iš žmogaus VIIa krešėjimo faktoriaus ir albuminoalbumin	Įgimtos VII faktoriaus stokos gydymas
Maltese	Proteina tal-fużjoni rikombinanti li tgħaqqad il-fattur VIIa tal-koagulazzjoni mal-albumina	Kura ta' nuqqas konġenitali ta' fattur VII

[1] At the time of designation

Language	Active ingredient	Indication
Polish	Rekombinowane białko fuzyjne składające się z ludzkiego czynnika krzepnięcia VIIa połączonego z albuminą	Leczenie wrodzonego niedoboru czynnika VII
Portuguese	Proteína de fusão recombinante composta pelo fator VIIa da coagulação humana ligado à albumina humana	Tratamento da deficiência congénita de Fator VII
Romanian	Proteină recombinantă de fuziune, constituită dinfactorul de coagulare VIIa uman legat de albumina umană	Tratamentul deficienței congenitale de Factor VII
Slovak	Rekombinantný fúzny proteín skladajúci sa z ľudského koagulačného faktora VIIa naviazaného na ľudský albumin	Liečba vrodeného nedostatku faktora VII
Slovenian	Rekombinantni fuzijski protein, sestavljen iz humanega koagulacijskega faktorja VIIa, povezanega z albuminom	Zdravljenje prirojenega pomanjkanja faktorja VII
Spanish	Proteina de fusion recombinante compuesta del factor VIIa de coagulacion humano unido a la albumina humana	Tratamiento de déficit del factor VII
Swedish	Rekombinant fusionsprotein bestående av human koagulationsfaktor VIIa bunden till humant albumin	Behandling av medfödd faktor VII-brist
Norwegian	Rekombinant fusjonsprotein bestående av human koagulasjionsfaktor VIIa bundet til humant albumin	Behandling av medfødt faktor VII-mangel
Icelandic	Raðbrigða samrunaprótein sem samanstendur af manna storkuþætti VIIa tengt mannaalbúmíni	Meðferð við meðfæddum skorti á storkuþætti VII