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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human plasmin for the treatment of acute peripheral arterial occlusion

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Rev.1: sponsor's name change	18 April 2012
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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.	

On 23 February 2011, orphan designation (EU/3/10/834) was granted by the European Commission to Talecris Biotherapeutics GmbH, Germany, for human plasmin for the treatment of acute peripheral arterial occlusion.

In January 2012, Talecris Biotherapeutics GmbH changed name to Grifols Deutschland GmbH.

What is acute peripheral arterial occlusion?

Acute peripheral arterial occlusion is the sudden blockage of an artery supplying blood to the limbs by a blood clot. The blood clot can form locally because the artery is already narrowed, or it can come from another part of the body, for example after surgery to the heart. Typically, the patient experiences severe pain in the affected limb, which also becomes cold and pale. The local nerves may become severely damaged, leading to loss of sensation and the inability to move the limb. The lack of blood flow in the limb could also lead to tissue damage.

Acute peripheral arterial occlusion is a debilitating disease that may be life threatening, especially because of the irreversible tissue damage. It could also lead to amputation of the affected limb.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

What is the estimated number of patients affected by acute peripheral arterial occlusion?

At the time of designation, acute peripheral arterial occlusion affected less than 1.7 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 86,000 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, satisfactory methods had been authorised in the EU for the treatment of acute peripheral arterial occlusion. These included medicines that activate a protein called plasminogen into its active form, plasmin, which then breaks down the blood clots. In addition, surgery was used to restore the blood flow and prevent limb loss and disability.

The sponsor has provided sufficient information to show that human plasmin might be of significant benefit for patients with this condition because of its direct action on the blood clot causing the blockage of the arteries compared with the indirect action of 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?

Plasmin is a protein found naturally in the blood that breaks down blood clots. This medicine is a purified form of human plasmin, prepared from human plasma (the liquid part of the blood), which is expected to be delivered directly into the affected arteries of patients with acute peripheral arterial occlusion using a catheter (a thin sterile tube). When delivered, the medicine is expected to break down the clot, thereby removing the obstruction and relieving the patient's symptoms.

What is the stage of development of this medicine?

The effects of human plasmin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with human plasmin in patients with acute peripheral arterial occlusion were ongoing.

At the time of submission, human plasmin was not authorised anywhere in the EU for acute peripheral arterial occlusion.

Orphan designation of human plasmin had been granted in the United States of America for acute peripheral arterial occlusion.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 November 2010 recommending the granting of this designation.

*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 507,700,000 (Eurostat 2011).

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:

Grifols Deutschland GmbH
Lyoner Strasse 15
D-60528 Frankfurt am Main
Germany
Tel.: +49 69 66 05 93 401
Fax: +49 69 66 05 93 110
E-mail: wolfgang.schulten@grifols.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	Human plasmin	Treatment of acute peripheral arterial occlusion
Bulgarian	Човешки плазмин	Лечение на остра периферна артериална оклузия
Czech	Humánní plazmin	Léčba akutní arteriální okluze
Danish	Humant plasmin	Behandling af akut perifer arterieokklusion
Dutch	Humaan plasmine	Behandeling van acute perifere arteriële occlusie
Estonian	Inimese plasmin	Ägeda perifeerse arterialse oklusiooni ravi
Finnish	Ihmisen plasmiini	Akuutin perifeerisen valtimotukoksen hoito
French	Plasmine humaine	Traitement de l'occlusion artérielle périphérique aiguë
German	Humanes Plasmin	Behandlung akuter peripherer arterieller Verschlüsse
Greek	Ανθρώπινη πλασμίνη	Θεραπεία της οξείας περιφερικής αρτηριακής απόφραξης
Hungarian	Humán plazmin	Heveny perifériás verőér elzáródás kezelése
Italian	Plasmina umana	Trattamento dell'occlusione arteriosa periferica acuta
Latvian	Cilvēka plazmīns	Akūtas perifēriskas arteriālās oklūzijas ārstēšana
Lithuanian	Žmogaus plazminas	Ūmios periferinių arterijų okliuzijos gydymas
Maltese	Plażmina umana	Kura ta' sadd arterjali periferali akut
Polish	Plazmina ludzka	Leczenie ostrej niedrożności tętnic obwodowych
Portuguese	Plasmina humana	Tratamento da oclusão arterial periférica aguda
Romanian	Plasmină umană	Tratamentul ocluziei arteriale periferice acute
Slovak	Ľudský plazmín	Liečba akútnej periférnej arteriálnej oklúzie
Slovenian	Humani plazmin	Zdravljenje akutne periferne arterijske zapore
Spanish	Plasmina humana	Tratamiento de la oclusión arterial periférica aguda
Swedish	Humant plasmin	Behandling av akut perifer arteriell ocklusion
Norwegian	Humant plasmin	Behandling av akutt, perifer, arteriell okklusjon
Icelandic	Mannaplasmín	Meðferð á bráðri útlægri slagæðalokun

[1] At the time of designation