

12 August 2015
EMA/COMP/426689/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human plasminogen for the treatment of plasminogen deficiency

On 28 July 2015, orphan designation (EU/3/15/1511) was granted by the European Commission to ProMetic BioTherapeutics Ltd, United Kingdom, for human plasminogen for the treatment of plasminogen deficiency.

What is plasminogen deficiency?

Plasminogen deficiency is an inherited disorder in which patients cannot produce normal amounts of plasminogen or in which plasminogen does not work properly. Plasminogen is a protein important in dissolving blood clots and helping wounds to heal. The first type, which is the more common form in Europe, is usually more severe and is associated with the formation of pseudomembranes, fibrous layers of protein, on moist body surfaces such as the conjunctiva (the lining of the inner eyelids and outer layer of the white of the eye), the lining of the mouth and the linings of the windpipe, gut, and vagina. This can lead to inflammation and organ damage. In addition, some children with the condition are born with fluid in the brain which can lead to brain damage if untreated.

Plasminogen deficiency is a long-term debilitating condition due to damage to the eyes and other organs.

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

At the time of designation, plasminogen deficiency affected approximately 0.02 in 10,000 people in the European Union (EU). This was equivalent to a total of around 1,000 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, no satisfactory methods were authorised in the EU for treating plasminogen deficiency. Patients were managed with surgery and with medicines to reduce inflammation and scarring.

*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).

How is this medicine expected to work?

This medicine contains purified plasminogen extracted from human blood. When given by injection to patients who lack working plasminogen of their own, it is expected to replace the missing protein in their blood and so correct the symptoms of the condition.

What is the stage of development of this medicine?

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

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with plasminogen deficiency were ongoing.

At the time of submission, human plasminogen was not authorised anywhere in the EU for plasminogen deficiency. Orphan designation of the medicine had been granted in the United States for type 1 plasminogen deficiency (hypoplasminogenaemia).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 June 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.

For more information

Sponsor's contact details:

ProMetic BioTherapeutics Ltd
Horizon Park, Barton Road
Comberton
Cambridge CB23 7AJ
United Kingdom
Tel. +44 (0)1223 433 822
Fax +44 (0)1223 420 270
E-mail: regaffairsuk@prometic.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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Human plasminogen	Treatment of plasminogen deficiency
Bulgarian	Човешки плазминоген	Лечение на дефицит на плазминоген
Croatian	Ljudski plazminogen	Liječenje nedostatka plazminogena
Czech	Lidský plazminogen	Léčba deficitu plasminogenu
Danish	Humant plasminogen	Behandling af plasminogen mangel
Dutch	Humaan plasminogen	Behandeling van plasminogeendeficiëntie
Estonian	Inimese plasminogeen	Plasminogeeni puudulikkuse ravi
Finnish	Ihmisen plasminogeeni	Plasminogeenin puutoksen hoito
French	Plasminogène humain	Traitement du déficit en plasminogène
German	Humanes Plasminogen	Behandlung von Plasminogen-Mangel
Greek	Ανθρώπινο πλασμινογόνο	Θεραπεία της ανεπάρκειας πλασμινογόνου
Hungarian	Humán plazminogén	Plazminogén-hiány kezelésére
Italian	Plasminogeno umano	Trattamento della plasminopenemia
Latvian	Cilvēka plazminogēns	Plazminogēna deficīta ārstēšana
Lithuanian	Žmogaus plazminogenas	Plazminogeno stokos gydymas
Maltese	Plasminogen uman	Kura ta' nuqqas ta' plasminogen
Polish	Plazminogen ludzki	Leczenie niedoboru plazminogenu
Portuguese	Plasminogénio humano	Tratamento de deficiência de plasminogénio
Romanian	Plasminogen uman	Tratamentul deficitului de plasminogen
Slovak	Ľudský plazminogén	Liečba plasminogenového nedostatku
Slovenian	Človeški plazminogen	Zdravljenje pomanjkanje plazminogena
Spanish	Plasminógeno humano	Tratamiento de la deficiencia de plasminógeno
Swedish	Human plasminogen	Behandling av plasminogen brist
Norwegian	Human plasminogen	Behandling av plasminogen-mangel
Icelandic	Forplasmín úr mönnum	Meðferð plasmínógen skorts

¹ At the time of designation