European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF human plasminogen for the treatment of ligneous conjunctivitis

On 3 August 2007, orphan designation (EU/3/07/461) was granted by the European Commission to Kedrion S.p.A., Italy, for human plasminogen for the treatment of ligneous conjunctivitis.

What is ligneous conjunctivitis?
Ligneous conjunctivitis is a rare chronic inflammation of the eyes, which is caused by plasminogen deficiency and results in the formation of layers of different materials on the surface of the eyes (pseudomembranes). Plasminogen is a protein that is present in the blood and in the other tissues, including the eye, and also plays a role in the coagulation process. Ligneous conjunctivitis is chronically debilitating due to the risk of corneal involvement and subsequent blindness; it can occasionally be life-threatening due to associated lung disease.

What are the methods of treatment available?
No satisfactory methods exist that were authorised at the time of application. Surgery may be necessary in certain cases, but the lesions usually recur after surgery. Therapy with plasminogen, while not authorised, has been described in medical journals in a few patients with ligneous conjunctivitis, with apparent good results.

What is the estimated number of patients affected by the condition[*]?
According to the information provided by the sponsor, ligneous conjunctivitis was considered to affect less than 5,000 persons in the European Union.

How is this medicinal product expected to act?
Human plasminogen is expected to replace the missing protein in the eye and prevent pseudomembrane formation. Plasminogen is converted to plasmin, which helps to heal the lesions that develop in these patients.

What is the stage of development of this medicinal product?
At the time of submission of the application for orphan designation, no studies in experimental models, or clinical trials in patients, had been initiated.

Human plasminogen was not authorised anywhere worldwide for ligneous conjunctivitis, or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

[*] Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 27 June 2007 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

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Translations of the active ingredient and indication in all EU languages and Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Human plasminogen	Treatment of ligneous conjunctivitis
Bulgarian	Човешки плазминоген	Лечение на лигнеозен конюнктивит
Czech	Lidský plazminogen	Léčba konjuktivitidy lignea
Danish	Humant plasminogen	Behandling af conjunctivitis lignosa
Dutch	Humaan plasminogen	Behandeling van conjunctivitis lignosa
Estonian	Inimese plasminogeen	Ligneoosse sidekestapõletiku ravi
Finnish	Ihmisen plasminogeeni	Conjunctivitis lignosan hoitoon
French	Plasminogène humain	Traitement de la conjonctivite ligneuse
German	Humanes Plasminogen	Behandlung der Konjunktivitis lignosa
Greek	Ανθρωπινο πλασμινογονο	Θεραπεία Ξυλώδους Επιπεφυκίτιδας
Hungarian	Humán plazminogén	Conjunctivitis lignosa kezelése
Italian	Plasminogeno umano	Trattamento delle congiuntivite lignea
Latvian	Cilvēka plazminogēns	Fibroza konjunktivīta ārstēšana
Lithuanian	Žmogaus plazminogenas	Medžių žiedadulkių sukelto konjunktyvito gydymas
Maltese	Plasminogen uman	Kura tal-konġuntivite linjuża
Polish	Plazminogen ludzki	Leczenie zapalenia spojówek z tworzeniem pseudobłony
Portuguese	Plasminogénio humano	Tratamento da conjuntivite lenhosa
Romanian	Plasminogen uman	Tratamentul conjunctivitei lemnoase
Slovak	Ľudský plazminogén	Liečba ligneóznej konjunktivitídy
Slovenian	Človeški plazminogen	Tretma lesastega vnetja očesnih veznic
Spanish	Plasminógeno humano	Tratamiento de la conjuntivitis leñosa
Swedish	Human plasminogen	Behandling av träartad konjunktivit
Norwegian	Human plasminogen	Behandling av conjunctivitis lignosa
Icelandic	Forplasmín úr mönnum	Meðferð við trjákenndri tárubólgu (iigneous conjunctivitis)