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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF *E. Coli* heat-shock protein 70 with bovine retinal S-antigen for the treatment of autoimmune uveitis

On 24 January 2006, orphan designation (EU/3/05/346) was granted by the European Commission to Biotech Tools SA, Belgium, for *E. Coli* heat-shock protein 70 with bovine retinal S-antigen for the treatment of autoimmune uveitis.

What is autoimmune uveitis?

The term uveitis covers clinical symptoms of inflammation of the uvea, the middle layer of the eye - between the sclera and retina. Autoimmune uveitis is a type of uveitis where a reaction to an individual's own tissues (in this case in the eye) occurs because the body's defence system mistakenly recognises it as foreign tissue and tries to destroy it. This may occur in association with other autoimmune illnesses that include, amongst others, various types of arthritis and some bowel diseases. In many cases the cause of the uveitis cannot be found.

Uveitis can affect one eye or both eyes and may cause varying degrees of discomfort or pain, with or without blurring of vision. The eye may be red or appear white. Autoimmune uveitis is chronically debilitating as it may lead to structural damage to the tissues in the eye, cataracts (opacity of the transparent structures of the eyes impairing vision or causing blindness) and sight loss.

What are the methods of treatment available?

Various medicinal products were available for the treatment of autoimmune uveitis and were authorised in some Member States in the Community at the time of submission of the application for orphan drug designation.

E. Coli heat-shock protein 70 with bovine retinal S-antigen might be of potential significant benefit for the treatment of autoimmune uveitis. This medicinal product might help to achieve better overall control of the condition. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

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

According to the information provided by the sponsor, autoimmune uveitis was considered to affect about 207,000 persons in the European Union.

How is this medicinal product expected to act?

E. Coli heat-shock protein 70 with bovine retinal S-antigen mimicks a portion of one of the eye natural proteins against which the patient's immune system reacts thus resulting in autoimmune uveitis. Once administered to the patient, *E. Coli* heat-shock protein 70 with bovine retinal S-antigen is expected to induce tolerance of the body's defence system to the natural proteins of the eye that are mistakenly recognised as foreign tissues.

What is the stage of development of this medicinal product?

The evaluation of the effects of *E. Coli* heat-shock protein 70 with bovine retinal S-antigen in experimental models is ongoing.

At the time of submission of the application for orphan designation, no clinical trials in patients with autoimmune uveitis were initiated.

E. Coli heat-shock protein 70 with bovine retinal S-antigen was not authorised anywhere worldwide for autoimmune uveitis or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 7 December 2005 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.

For more information:

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

Language	Active Ingredient	Indication
English	<i>E. Coli</i> heat-shock protein 70 with bovine retinal S-antigen	Treatment of autoimmune uveitis
Czech	<i>E. Coli</i> heat-shock protein s boviním retinálním S-antigenem	Léčba autoimunní uveitidy
Danish	<i>E. Coli</i> heat-shock protein 70 med bovint retinalt S-antigen	Behandling af autoimmun uveitis
Dutch	<i>E. Coli</i> hitte shock proteïne 70 met bovien retinaal S-antigen	Behandeling van auto-immune uveïtis
Estonian	<i>E. Coli</i> heat-shock valk 70 koos looma reetina S-antigeeniga-	Autoimmuunse uveiidi ravi
Finnish	<i>E. Coli</i> heat shock-proteiini 70, jossa on nautaperäinen retinaalinen S-antigeeni	Autoimmuuniuveiitin hoito
French	Protéine de choc thermique 70 de <i>E. Coli</i> avec S-antigène de rétine bovine	Traitement de l'uvéïte auto-immune
German	<i>E. Coli</i> Hitzeschockprotein 70 mit bovin-retinalem S-Antigen	Behandlung der Autoimmunuveitis
Greek	<i>E. Coli</i> πρωτεΐνη θερμικής προσβολής (heat-shock) 70 με S-αντιγόνο βόειου αμφιβληστροειδούς	Θεραπεία της αυτοάνοσης φλεγμονής χοριοειδούς χιτώνας
Hungarian	<i>E. Coli</i> hőszokk 70kD fehérje szarvasmarha retina S-antigénnel	Autoimmun uveitis kezelése
Italian	<i>E. Coli</i> proteina heat-shock 70 con S-Antigene di retina bovina	Trattamento dell'uveite autoimmune
Latvian	<i>E. Coli</i> karstuma šoka proteīns 70 ar vērša retinālo S-antigēnu	Autoimūna uveīta ārstēšanai
Lithuanian	<i>E. Coli</i> šilumos smūgiu paveiktas baltymas 70 su galvijų tinklainės S – antigenu	Autoimuninio uveito gydymas
Polish	Białko szoku termicznego <i>E. Coli</i> z wolim antygenem S naczyńiówkowym	Leczenie autoimmunologicznego zapalenia błony naczyniowej oka
Portuguese	Proteína 70 de choque térmico da <i>E. Coli</i> com antígeno S de retina de bovino	Tratamento da uveíte autoimmune
Slovak	<i>E. Coli</i> proteín teplotného šoku 70 s boviným retinálnym S antigénom	Liečba autoimúnnej uveitídy
Slovenian	<i>E. Coli</i> vročinsko šokovni protein 70 z bovinim mrežničnim S-antigenom	Zdravljenje avtoimunskega uveitisa
Spanish	Proteína 70 del shock térmico	Tratamiento de la uveítis autoimmune

	de <i>E. Coli</i> con antígeno S de retina bovinade	
Swedish	<i>E. Coli</i> värmechock protein 70 med bovint retinalt S-antigen	Behandling av autoimmun uveitis
Norwegian	<i>E. coli</i> heat shock protein 70 med bovint retinalt S-antigen	Behandling av autoimmun uveitt
Icelandic	Kóliðgerla hita-losts prótein 70 með nauta sjónu S-mótefnavaka	Meðferð sjálfsofnæmis æðahjúpsbólgu