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Public summary of opinion on orphan designation

HLA-B27-derived peptide (amino acid 125-138) for the treatment of autoimmune uveitis

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Rev.1: transfer of sponsorship	9 June 2006
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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 2 September 2004, orphan designation (EU/3/04/219) was granted by the European Commission to Lynkeus BioTech GmbH, Germany, for HLA-B27-derived peptide (amino acid 125-138) for the treatment of autoimmune uveitis.

The sponsorship was transferred to Dr Gerhild Wildner, Germany, in August 2005.

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 is never 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 is the estimated number of patients affected by the condition?

At the time of designation, autoimmune uveitis affected approximately 4.5 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 209,000 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?

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.

HLA-B27-derived peptide (amino acid 125-138) 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.

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?

HLA-B27-derived peptide (amino acid 125-138) 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, HLA-B27-derived peptide 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 medicine?

The evaluation of the effects of HLA-B27-derived peptide (amino acid 125-138) in experimental models is ongoing. At the time of submission of the application for orphan designation, clinical trials in patients with autoimmune uveitis were ongoing.

HLA-B27-derived peptide (amino acid 125-138) was not marketed anywhere worldwide for autoimmune uveitis or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 22 July 2004 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 25), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 464,200,000 (Eurostat 2004).

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

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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	HLA-B27 derived peptide (amino acid 125-138)	Treatment of autoimmune uveitis
Czech	HLA-B27 derivát peptidu (aminokyselina 125-138)	Léčba autoimunní uveitidy
Danish	HLA-B27 afledt peptid (aminosyre 125 - 138)	Behandling af autoimmun uveitis
Dutch	HLA-B27-afgeleid peptide (aminozuur 125-138)	Behandeling van auto-immune uveïtis
Estonian	HLA-B27-st tulenev peptiid (aminohape 125-138)	Autoimmuunse uveiidi ravi.
Finnish	HLA-B27 -johdettu peptidi (aminohappo 125-138)	Autoimmuuniuveiitin hoito
French	Peptide dérivé du HLA-B27 (acide aminé 125-138)	Traitement de l'uvéïte auto-immune
German	HLA-B27 abgeleitetes Peptid (Aminosäure 125-138)	Behandlung der autoimmun Uveitis
Greek	HLA-B27 παράγωγο πεπτιδίο (αμινοξύ 125-138)	Θεραπεία της αυτοάνοσης φλεγμονής χοριοειδούς χιτώνος
Hungarian	HLA-B27 derivált peptid (aminosav 125-138)	Autoimmun uveitis kezelése
Italian	Peptide derivato da HLA-B27 (aminoacidi 125-138)	Trattamento dell'uveite autoimmune
Latvian	No HLA-B27 atvasinātais peptīds (aminoskābe 125 – 138)	Autoimūna uveiņa ārstēšanai
Lithuanian	ŽLA-B27 gautas peptidas (amino rūgštis 125-138)	Autoimuninio uveito gydymas
Maltese	HLA-B27 derived peptide (amino acid 125-138)	Treatment of autoimmune uveitis
Polish	Peptyd pochodny z HLA-B27 (aminokwas 125-138)	Autoimmunologiczne zapalenie błony naczyniowej oka
Portuguese	Derivado do péptido HLA-B27 (aminoácido 125 - 138)	Tratamento da uveíte auto-imune
Slovak	HLA-B27 odvodený peptid (aminokyselina 125 – 138)	Liečba autoimúnnej uveitídy
Slovenian	Peptid (aminokislina 125-138), ki izvira iz HLA-B27	Avtoimunski uveitis
Spanish	Péptido derivado del HLA-B27 (aminoácidos 125-138)	Tratamiento de la uveítis autoimmune
Swedish	HLA-B27 deriverad peptid (aminosyra 125-138)	Behandling av autoimmun uveitis
Norwegian	HLA-B27 avledet peptid (aminosyre 125 –138)	Behandling av autoimmun uveitt

¹ At the time of designation

Language	Active Ingredient	Indication
Icelandic	HLA-B27 afleidd peptíð (amínósýra 125-138)	sjálfnæm æðahjúpsbólga