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

Document Date: London, 8 August 2006
Doc.Ref.: EMEA/COMP/315311/2005

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF levamisol hydrochloride for the treatment of nephrotic syndrome

On 28 October 2005, orphan designation (EU/3/05/324) was granted by the European Commission to ACE Pharmaceuticals BV, The Netherlands, for levamisol hydrochloride for the treatment of nephrotic syndrome.

What is nephrotic syndrome?

Nephrotic syndrome is a type of kidney disorder that is characterised by oedema (presence of abnormal large amounts of fluid in the body) and some abnormal amounts of certain substances such as "low serum albumin" (a major type of protein in the blood) and a large amount of protein in the urine. Other typical signs or symptoms of the disease apart from oedema, are weight gain, high blood pressure and an uncontrolled lack or loss of appetite. Nephrotic syndrome might occur without apparent causes (especially in children) or as a result of a number of illnesses that can damage the kidney.
Nephrotic syndrome is a chronically debilitating and life-threatening condition.

What are the methods of treatment available?

Medicinal products, such as corticosteroids, were authorised for the condition within the Community at the time of submission of the application for orphan drug designation.
Satisfactory argumentation has been submitted by the sponsor to justify the assumption that the medicinal product might be of potential significant benefit for the treatment of nephrotic syndrome, particularly in terms of its corticosteroid-sparing effect. 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, nephrotic syndrome was considered to affect about 46,000 persons in the European Union.

How is this medicinal product expected to act?

Nephrotic syndrome is considered as an immunologic disease (involving a reaction by which the body harms itself). Levamisol hydrochloride is proposed to act by changing the body's immune response against the disease. As a result the need for treatment with immunosuppressants, e.g. corticosteroids, could be reduced.

What is the stage of development of this medicinal product?

The effects of levamisole hydrochloride were evaluated in experimental models.
At the time of submission of the application for orphan designation, no clinical trials in patients with nephrotic syndrome were initiated.

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The medicinal product was not authorised anywhere worldwide for nephrotic syndrome 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 9 September 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.

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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	Levamisol hydrochloride	Treatment of nephrotic syndrome
Czech	Hydrochlorid levamisolu	Léčba nefrotického syndromu
Danish	Levamisol hydrochlorid	Behandling af nefrotisk syndrom
Dutch	Levamisol hydrochloride	Behandeling van nefrotisch syndroom
Estonian	Levamisoolhüdrokloriid	Nefrootilise sündroomi ravi
Finnish	Levamisoli hydrokloridi	Nefroottisen syndrooman hoito
French	Chlorhydrate de Levamisole	Traitement du syndrome néphrotique
German	Levamisolhydrochlorid	Behandlung des nephrotischen Syndroms
Greek	Υδροχλωρική Λεβαμισόλη	Θεραπεία του νεφροτικού συνδρόμου
Hungarian	Levamizol hidroklorid	Nephrosis szindróma kezelése
Italian	Levamisolo idrocloruro	Trattamento della sindrome nefrotica
Latvian	Levamizola hidrohlorīds	Nefrotiskā sindroma ārstēšana
Lithuanian	Levamizolio hidrochloridas	Nefrozinio sindromo gydymas
Polish	Lewamisolu chlorowodorek	Leczenie zespołu nerczycowego
Portuguese	Cloridrato de levamisol	Tratamento do síndrome nefrótico
Slovak	Levamizol hydrochlorid	Liečba nefrotického syndrómu
Slovenian	Levamisol hidroklorid	Zdravljenje nefrotičnega sindroma
Spanish	Clorhidrato de levamisol	Tratamiento del síndrome nefrótico
Swedish	Levamisol hydroklorid	Behandling av nefrotisk syndrom
Norwegian	Levamisolhydroklorid	Behandling av nefrotisk syndrom
Icelandic	Levamísól hýdróklóríð	Meferð við nýrungaheilkenni