



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Diphenylcyclopropenone for the treatment of alopecia universalis

On 29 June 2006, orphan designation (EU/3/06/380) was granted by the European Commission to Orfagen, France, for diphenylcyclopropenone for the treatment of alopecia universalis.

What is alopecia universalis?

Alopecia universalis is a disease characterised by complete hair loss of the whole body. It affects patients in an acute form (loss of hair within weeks) or in a slower form (loss of hair can progress for up to two years). Usually patients do not recover from the hair loss and the condition becomes chronic. Alopecia universalis can affect patients of both sexes and of any age. Patients suffering alopecia universalis report psychological consequences (distress, sadness) that are particularly severe in children and women.

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

At the time of designation, alopecia universalis affected not more than 2.5 in 10,000 people in the European Union (EU)*. This is equivalent to a total of not more than 161,000 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?

No satisfactory methods were authorised at the time of application.

How is this medicine expected to work?

Hair loss seen in alopecia universalis is thought to be caused by cells of the immune system (body's own defence mechanism against infection and disease) attacking the hair follicles. Diphenylcyclopropenone can act as local irritant and trigger local sensitization, which is an allergic reaction to oneself. By doing this, it is thought to mount an immune response and produce populations

*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 Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).



of immune cells that oppose the action of the autoreactive cells that destroy hair loss. This way it is thought to allow for hair growth locally.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, clinical trials in patients with alopecia universalis were completed. The sponsor of the application plans to conduct further clinical studies.

Diphenylcyclopropenone was not authorised anywhere worldwide for alopecia universalis 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 16 May 2006 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:

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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	Diphenylcyclopropenone	Treatment of alopecia universalis
Czech	Difenylcyclopropenone	Léčba alopecie universalis
Danish	Difenylcyklopropenon	Behandling af alopecia universalis
Dutch	Diphenylcyclopropenon	Behandeling van alopecia universalis
Estonian	Difenüülsüklopropenoon	<i>Alopecia universalise ravi</i>
Finnish	Difenyylisyklopropenooni	Kaikkialla esiintyvän alopesian (<i>alopecia universalis</i>) hoitoon
French	Diphénylcyclopropénone	Traitement de l'alopecie universelle
German	Diphenylcyclopropenon	Behandlung der Alopecia universalis
Greek	Διφαινυλοκυκλοπροπενόνη	Αγωγή κατά της καθολικής αλωπεκίας
Hungarian	Difenilciklopropenon	Alopecia universalis kezelése
Italian	Difenilciclopropenone	Trattamento dell'alopecia universale
Latvian	Difenilciklopropenons	Vispārējas matu izkrišanas [<i>alopecia universalis</i>] ārstēšana
Lithuanian	Difenilciklopropenonas	Bendro nuplikimo gydymas
Polish	Difenylcyclopropenon	Leczenie łysienia uogólnionego
Portuguese	Difenilciclopropenona	Tratamento da alopecia universalis
Slovak	Difenylcyklopropenón	Liečba alopecie universalis
Slovenian	Difenilciklopropenon	Zdravljenje alopecije universalis
Spanish	Difenilciclopropenona	Tratamiento de la alopecia universal
Swedish	Difenylcyklopropenon	Behandling av alopecia universalis
Norwegian	Difenylcyklopropenon	Behandling av alopecia universalis
Icelandic	Tvífénýlcýklóprópenón	Til meðferðar á hárleysi (alopecia universalis)

¹ At the time of designation