



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

16 June 2011
EMA/COMP/405290/2006 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Budesonide (oral use) for the treatment of Graft-versus-Host disease

On 3 November 2006, orphan designation (EU/3/06/413) was granted by the European Commission to Dr Falk Pharma GmbH, Germany, for budesonide (oral use) for the treatment of Graft-versus-Host disease.

What is Graft-versus-Host disease?

The bone marrow is the spongy tissue inside the large bones in the body. Bone marrow produces red blood cells (which carry oxygen and other materials to all tissues of the body), white blood cells (that fight infection), and platelets (that make the blood clot). Bone marrow transplantation (replacing with healthy marrow) is a treatment used against certain diseases of the bone marrow. Development of Graft-versus-Host disease (GvHD) is a frequent complication of bone marrow transplantation. This disease involves a reaction between the donor cells and the recipient's native tissues leading to injury of the recipient's tissues. GvHD occurs in acute and chronic form. The organs most commonly affected in acute GvHD are the stomach and the intestines, the skin, and the liver. Chronic GvHD involves a much wider range of tissues than the acute form. The condition is chronically debilitating and life threatening.

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

At the time of designation, Graft-versus-Host disease affected less than 1 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 46,000 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?

The methods of treatment authorised for GvHD in the Community, at the time of submission of the application for orphan designation, consisted of certain steroid hormones (corticosteroids),

*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).



administered at high doses. Other therapies included drugs that inhibit the immune response (immunosuppressants).

How is this medicine expected to work?

Budesonide belongs to the group of corticosteroids, chemicals that affect the activity of certain organs and of the immune system. This medicinal product contains budesonide, which is intended for use as an oral rinse and it is expected to be absorbed through the oral mucosa. This way, the product is expected to act locally, while its side effects are expected to be limited.

What is the stage of development of this medicine?

The effects of budesonide (oral use) were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with GvHD were ongoing.

Budesonide (oral use) was not authorised anywhere worldwide for Graft versus Host Disease, 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 4 October 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:

Dr Falk Pharma GmbH
Leinenweberstrasse 5
D-79041 Freiburg i. Br
Germany
Telephone: +49 76 11 51 40
Telefax: +49 76 11 51 43 21
E-mail: zentrale@drfalkpharma.de

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	Budesonide (oral use)	Treatment of Graft-versus-Host disease
Czech	Budesonid (perorální podání)	Léčba reakce štěpu proti hostiteli
Danish	Budesonid (oral anvendelse)	Behandling af graft versus host reaktion
Dutch	Budesonide (oraal gebruik)	Behandeling van graft versus host ziekte
Estonian	Budesoniid (peroraalne)	Graft versus host haiguse ravi
Finnish	Budesonidi (ssuun kautta)	Käänteishyljintäreaktion hoito
French	Budésonide (voie orale)	Traitement de la réaction du greffon contre l'hôte
German	Budesonid (zum Einnehmen)	Behandlung der Graft-versus-Host-Reaktion
Greek	Budesonid (Από στόματος χρήση)	Θεραπεία της αντίδρασης του μοσχεύματος
Hungarian	Budezonid (oralis alkalmazás)	Graft-versus-host betegség kezelése
Italian	Budesonide (uso orale)	Trattamento della reazione del trapianto contro l'ospite
Latvian	Budezonīds (iekšķīgai lietošanai)	Saimnieka-transplantāta slimības ārstēšana
Lithuanian	Budezonidas (gerti)	Transplantato atmetimo ligos gydymas
Polish	Budesonid (podanie doustne)	Leczenie choroby przeszczep przeciw gospodarzowi
Portuguese	Budesonida (via oral)	Tratamento da reacção do enxerto contra o hospedeiro
Slovak	Budesonid (perorálne použitie)	Liečba reakcie štepů proti hostiteľovi
Slovenian	Budezonid (peroralna uporaba)	Zdravljenje bolezni presadka proti gostitelju
Spanish	Budesonida (vía oral)	Tratamiento de la enfermedad de injerto contra huésped
Swedish	Budesonid (oral användning)	Behandling av graft-värd host reaktion
Norwegian	Budesonid (oral bruk)	Behandling av graft-versus-host -reaksjon
Icelandic	Búdesóníð (til notkunar í munni)	Til meðferðar á hýsilssótt í munni

¹ At the time of designation