



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Beclomethasone 17,21-dipropionate (oral use) for the treatment of intestinal graft-versus-host disease

On 13 March 2002, orphan designation (EU/3/02/093) was granted by the European Commission to Voisin Consulting, France, for beclomethasone 17,21-dipropionate (oral use) for the treatment of intestinal graft versus host disease (GvHD).

The sponsorship was transferred to DOR BioPharma UK Ltd, United Kingdom, in May 2008.

DOR BioPharma UK Ltd has changed its name to Soligenix UK Ltd in May 2011.

#### **What is intestinal graft versus host disease?**

Bone marrow transplantation is primarily utilised in the treatment of patients with malignant tumours or with blood diseases, such as leukemias and marrow dysplasias. A frequent complication of transplantation is the development of GvHD. This disease process involves a reaction between the donor cells and the recipient's native tissues. GvHD occurs in acute and chronic form. The anatomical sites most likely affected in acute GvHD are the gastrointestinal tract, the skin, and the liver. Chronic GvHD involves a much wider range of tissues than the acute form. Any portion of the gastro-intestinal tract may be involved. The condition is chronically debilitating and life-threatening.

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

At the time of designation, intestinal graft versus host disease affected between 0.1 and 0.2 in 10,000 people in the European Union (EU)\*. This is equivalent to a total of between 3,800 and 7,500 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).

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\*Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition. This estimate is based on available information and calculations presented by the sponsor at the time of the application.



## **What treatments are available?**

The methods of treatment were authorised for GvHD in the Community, at the time of submission of the application for orphan designation, consisted of systemic corticosteroids administered at high doses. Other therapies include various immunosuppressants. 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 intestinal GvHD, particularly in terms of limiting toxicity.

## **How is this medicine expected to work?**

Corticosteroids exert their anti-inflammatory effects by causing a decrease in the numbers of circulating white blood cells. Several immune factors are inhibited. Beclomethasone 17,21-dipropionate is a corticosteroid with potent local anti-inflammatory effect.

## **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 GvHD with gastrointestinal symptoms were ongoing.

Beclomethasone 17,21-dipropionate (oral use) had not been marketed anywhere worldwide for intestinal GvHD, at the time of submission.

Orphan designation of oral beclomethasone 17,21-dipropionate had been granted by the United States Food and Drug Administration (FDA) for oral administration in the treatment of intestinal GvHD.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 23 January 2002 recommending the granting of this designation.

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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:

Soligenix UK Ltd  
BioPark  
Broadwater Road  
Welwyn Garden City  
Hertfordshire AL7 3AX  
United Kingdom  
Telephone: +44 1707 35 86 05  
Telefax: +44 1707 35 86 05

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 languages<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Beclomethasone 17, 21-dipropionate (oral use)	Treatment of intestinal graft-versus-host disease
Bulgarian	Беклометазон 17, 21-дипропионат (за перорално приложение)	Лечение на чревна форма на реакция на отхвърляне на присадката
Czech	Beclomethasone 17, 21-dipropionat (orální aplikace)	Léčba intestinální formy nemoci reakce štěpu proti hostiteli
Danish	Beclometason-17, 21-dipropionat (oral anvendelse)	Behandling af intestinal transplanteret organs reaktion mod vært (graft-versus-host disease)
Dutch	Beclomethasone 17,21-dipropionaat (oraal gebruik)	Behandeling van intestinaal "graft versus host" reactie
Estonian	Beklometasoon-17, 21-dipropionaat (suukaudne)	Soole graft versus host haiguse ravi
Finnish	Beklometasoni-17,21-dipropionaatti (suun kautta)	Intestinaalisen hylkimisreaktion (graft-versus host) hoito
French	Béclométhasone-17, 21-dipropionate (voie orale)	Traitement des manifestations intestinales de la réaction du greffon contre l'hôte
German	Beclometason-17, 21-dipropionat (zum Einnehmen)	Behandlung der intestinalen Transplantat-Wirt-Reaktion
Greek	21-διπροπιονική βεκλομεθαζόνη-17 (Από στόματος χρήση)	Θεραπεία εντερικής νόσου του μοσχεύματος έναντι του ξενιστή
Hungarian	Beclometasone 17, 21 dipropionát (orális alkalmazásra)	Intesztinális graft-versus-host betegség kezelése
Italian	beclometazone 17,21 dipropionato (uso orale)	Trattamento della reazione intestinale del trapianto contro l'ospite
Latvian	Beklometazona 17, 21-dipropionāts (perorāla lietošana)	Transplantāta atgrūšanas slimības zarnu simptomātikas ārstēšana
Lithuanian	Beklometazono 17, 21 -dipropionatas (vartoti per burną)	Transplantato prieš šeimininką ligos žarnyno simptomatikos gydymas
Maltese	Beclomethasone 17, 21-dipropionate (użu orali)	Kura tal-marda intestinali tat-tessut għat-trapjant kontra dak li jirċievih
Polish	Beklometazonu 17, 21-dipropionian (podanie doustne)	Leczenie choroby przeszczep przeciw gospodarzowi
Portuguese	Beclometasona-17, 21-dipropionato (via oral)	Tratamento de manifestações intestinais da reacção do órgão transplantado contra o hóspede
Romanian	17, 21- dipropionat de beclometazonă (administrare orală)	Tratamentul bolii grefă contra gazdă la nivel intestinal
Slovak	Beklometazón-17, 21-dipropionát (perorálne použitie)	Liečba intestinálnej formy reakcie štepů proti hostiteľovi

<sup>1</sup> At the time of transfer of sponsorship

Language	Active ingredient	Indication
Slovenian	Beklometazon-17, 21-dipropionat (peroralno)	Zdravljenje bolezní presadka proti gostitelju
Spanish	Beclometasona-17, 21-dipropionato (vía oral)	Tratamiento de manifestaciones intestinales de la reacción del receptor contra el trasplante
Swedish	Beclometason-17, 21- dipropionat (oral användning)	Behandling av intestinal transplantat-kontra-värdreaktion ("graft-versus-host disease")