



**COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS**

**PUBLIC SUMMARY OF  
POSITIVE OPINION FOR ORPHAN DESIGNATION  
OF  
R-salbutamol sulphate  
for the treatment of cutaneous forms of lupus erythematosus**

On 14 September 2007, orphan designation (EU/3/07/481) was granted by the European Commission to Astion Pharma A/S, Denmark, for R-salbutamol sulphate for the treatment of cutaneous forms of lupus erythematosus.

**What are cutaneous forms of lupus erythematosus?**

Lupus erythematosus is a disease where the defence system of the body (immune system) damages its own tissues, such as the skin and those forming the joints, and organs, such as the kidneys. Inflamed lesions of the skin caused by lupus erythematosus can be divided into three subgroups: discoid lupus erythematosus (DLE), subacute cutaneous lupus erythematosus (SCLE), and other local lupus erythematosus (lupus erythematosus profundus and lupus panniculitis). Lesions occur predominantly in areas exposed to sunlight, such as the face, scalp, neck and upper extremities. A small number of patients develop scattered lesions, affecting widespread areas including the trunk and limbs. Generalized involvement is milder in SCLE, and in the other forms there is involvement of the deeper tissues beneath the skin. The condition may persist for years, with repeated recurrences, and may be chronically debilitating.

**What are the methods of treatment available?**

Several products with anti-inflammatory activity and some antimalarial drugs were authorised for the condition in some countries in the Community at the time of submission of the application for orphan drug designation. R-salbutamol sulphate could be of potential significant benefit for the treatment of cutaneous forms of lupus erythematosus, in particular with regards to improved efficacy. This assumption of significant benefit will have to be confirmed at the time of marketing authorisation, as this is necessary to maintain the orphan status.

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

Based on the information provided by the sponsor and previous knowledge of the Committee, cutaneous forms of lupus erythematosus was considered to affect not more than 5 in 10,000 persons in the European Union, which, at the time of designation, corresponded to about 249,000 persons in total.

**How is this medicinal product expected to act?**

R-salbutamol sulphate selectively binds to and activates  $\beta_2$ -adrenoceptors, which are molecules on the surface of many cells. This results in a decreased function of genes that induce inflammation. In short, the product has a general anti-inflammatory effect, and is administered directly on the skin as a cream.

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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 27), Norway, Iceland and Lichtenstein. This represents a population of 498,000,000 (Eurostat 2006). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

**What is the stage of development of this medicinal product?**

The effects of R-salbutamol sulphate were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with cutaneous forms of lupus erythematosus were ongoing.

R-salbutamol sulphate was not authorised anywhere in the world for the treatment of cutaneous forms of lupus erythematosus, nor designated as an orphan medicinal product 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 25 July 2007 a positive opinion recommending the grant of the above-mentioned designation.

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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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**Translations of the active ingredient and indication in all EU languages  
and Norwegian and Icelandic**

<b>Language</b>	<b>Active Ingredient</b>	<b>Indication</b>
English	R-salbutamol sulphate	Treatment of cutaneous forms of lupus erythematosus
Bulgarian	R-салбутамол сулфат	Лечение на кожни форми на lupus erythematosus
Czech	R-salbutamol sulphate	Léčba kožní formy lupus erythematosus
Danish	R-salbutamol sulfat	Behandling af kutan lupus erythematosus
Dutch	R-salbutamolsulfaat	Behandeling van cutane lupus erythematosus
Estonian	R-salbutamool-sulfaat	Erütematoosse luupuse nahavormide ravi
Finnish	R-salbutamolisuulfaatti	Iho-oireina ilmenevän punahukan hoito
French	Sulfate de R-salbutamol	Traitement des formes cutanées du lupus érythémateux
German	R-Salbutamolsulfat	Behandlung der kutanen Form des Lupus erythematosus
Greek	R- Σαλβουταμόλη θειϊκή	Θεραπεία των δερματικών μορφών του ερυθηματώδη λύκου
Hungarian	R-salbutamol szulfát	Cutan lupus erythematosus kezelése
Italian	R-salbutamolo solfato	Trattamento del lupus eritematoso cutaneo
Latvian	R-salbutamola sulfāts	Sarkanās vilkēdes ādas formu ārstēšana
Lithuanian	R-salbutamolio sulfatas	Odos pažeidimo, sergant raudonaja vilklige, gydymas
Maltese	R-salbutamol sulphate	Kura ta' forom tal-lupus erythematosus li jolqtu l-ġilda
Polish	Siarczan R-salbutamolu	Leczenie skórnych postaci tocznia rumieniowatego
Portuguese	Sulfato de R-salbutamol	Tratamento das manifestações cutâneas de lúpus eritematoso
Romanian	Sulfat de R-salbutamol	Tratamentul formelor cutanate de lupus eritematos
Slovak	R-salbutamoliumsulfát	Liečba kožných foriem lupus erythematosus
Slovenian	R-salbutamol sulfat	Zdravljenje kožnih oblik lupusa eritematozusa
Spanish	Sulfato de R-salbutamol	Tratamiento de la formas cutáneas de lupus eritematoso
Swedish	R-salbutamol sulfat	Behandling av kutan form av lupus erythematosus
Norwegian	R-salbutamolsulfat	Behandling av kutan lupus erythematosus
Icelandic	R-salbútamól sulfat	Meðferð á rauðum úlfum í húð