



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Paquinimod for the treatment of systemic sclerosis

On 23 February 2011, orphan designation (EU/3/10/836) was granted by the European Commission to Active Biotech AB, Sweden, for paquinimod for the treatment of systemic sclerosis.

What is systemic sclerosis?

Systemic sclerosis is a complex disease in which the immune system (the body's natural defences) is overactivated, causing inflammation and overproduction of various proteins, particularly collagen. The reason why the immune system is activated is not known. Collagen is an important component of connective tissue (the tissue that supports the skin and internal organs).

The overproduction of collagen leads to the abnormal growth of connective tissue, causing the skin to become thick and hard. It also damages the tissues in the blood vessel walls of the internal organs, such as the heart, lungs and kidneys. This makes it more difficult for the blood to move through the vessels, causing tissue damage, circulation problems and high blood pressure.

Systemic sclerosis is a debilitating disease that is long lasting and may be life threatening because of its effects on the gut, heart, lungs and kidneys.

What is the estimated number of patients affected by systemic sclerosis?

At the time of designation, systemic sclerosis affected approximately 3.5 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 177,000 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?

At the time of designation, there were no treatments for systemic sclerosis that could stop the build-up of collagen. Treatments authorised in the EU were aimed at relieving the symptoms of the disease and

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union (EU 27), Norway, Iceland and Liechtenstein. This represents a population of 506,500,000 (Eurostat 2010).



limiting the damage it causes. Several medicines were used to reduce inflammation and circulation problems.

The sponsor has provided sufficient information to show that paquinimod might be of significant benefit for patients with systemic sclerosis because it works in a different way to existing treatments and early studies in experimental models show that it might improve the treatment of patients with this condition. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Paquinimod is expected to work by modulating the activity of S100A9, a protein involved in the inflammatory process. In systemic sclerosis, it is expected to reduce inflammation and the abnormal growth of connective tissue.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, the evaluation of the effects of paquinimod in experimental models was ongoing.

At the time of submission, no clinical trials with paquinimod in patients with systemic sclerosis had been started.

At the time of submission, paquinimod was not authorised anywhere in the EU for systemic sclerosis or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 November 2010 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	Paquinimod	Treatment of systemic sclerosis
Bulgarian	Пакинимод	Лечение на системна склероза
Czech	Paquinimod	Léčba systémové sklerodermie
Danish	Paquinimod	Behandling af systemisk sklerose
Dutch	Paquinimod	Behandeling van Systeem Sclerose
Estonian	Paquinimod	Süsteemse sklerodermia ravi
Finnish	Pakinimodi	Systeemisen skleroosin hoito
French	Paquinimod	Traitement de la sclérose systémique
German	Paquinimod	Behandlung der systemischen Sklerose
Greek	Πακινιμόδη	Θεραπεία της συστηματικής σκλήρυνσης
Hungarian	Paquinimod	Szisztémás scleroderma kezelése
Italian	Paquinimod	Trattamento della sclerosi sistemica
Latvian	Pakvinimods	Sistēmiskas sklerozes ārstēšana
Lithuanian	Pakvinimodas	Sisteminės sklerozės gydymas
Maltese	Paquinimod	Kura tas-sklerosi sistemika
Polish	Pachinimod	Leczenie twardziny narządowej
Portuguese	Paquinimod	Tratamento da esclerose sistémica
Romanian	Pachinimod	Tratamentul sclerozei sistemice
Slovak	Paquinimod	Liečba systémovej sklerózy
Slovenian	Pakvinimod	Zdravljenje sistemske skleroze
Spanish	Paquinimod	Tratamiento de la esclerosis sistémica
Swedish	Pakinimod	Behandling av systemisk skleros
Norwegian	Pakinimod	Behandling av systemisk sklerose
Icelandic	Paquínimód	Meðferð við dreifðum herslismeinum

¹ At the time of designation