



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

Document Date: London, 15 June 2009
Doc.Ref.: EMEA/COMP/271/2004

Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of halofuginone hydrobromide for the treatment of systemic sclerosis

On, 11 December 2001 orphan designation (EU/3/01/074) was granted by the European Commission to PPD Global Ltd., United Kingdom, for halofuginone hydrobromide for the treatment of systemic sclerosis.

What is systemic sclerosis?

Systemic sclerosis (also called scleroderma) is a disease of unknown cause, characterised by the accumulation of collagen leading to fibrosis (scar tissue formation) of various severity in different organs such as the skin, blood vessels, digestive tract, lungs, heart and kidneys.

The condition is chronically debilitating and life threatening, in particular due to lung and kidney damage. The progressive deterioration of the lung function remains life-threatening.

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

At the time of designation systemic sclerosis affected approximately 0.3 to 1.3 in 10,000 in the European Union (EU) *. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of around 11,300 to 49,000 people.

What treatments are available?

Several products with anti-inflammatory activity had been authorised for the condition in some countries in the Community at the time of submission of the application for orphan drug designation. Other products were also authorised in some countries in the Community for the treatment of high blood pressure in the lung vessels (pulmonary hypertension), which is one of the major consequences of the disease.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that halofuginone hydrobromide might be of potential significant benefit for the treatment of systemic sclerosis. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Halofuginone hydrobromide is a chemical compound. According to the sponsor, it acts as an inhibitor (a molecule which represses or prevents another molecule from engaging in a reaction) on the build-up of one type of collagen (type I). Thus, halofuginone hydrobromide could have a potential influence on the excessive deposition of collagen type I in patients with systemic sclerosis.

What is the stage of development of this medicine?

The effects of halofuginone hydrobromide were evaluated in experimental models.

*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. This represents a population of 377,000,000 (Eurostat 2001).

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

Halofuginone hydrobromide was not marketed anywhere worldwide for the treatment of systemic sclerosis or designated as orphan medicinal product elsewhere 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 26 October 2001 a positive opinion recommending the grant of the above-mentioned 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;
- and either the rarity of the condition (affecting not more than five in 10,000 people in the Community) or the 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 the quality, safety and efficacy is necessary before a 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**

Language	Active Ingredient	Indication
English	Halofuginone Hydrobromide	Treatment of Systemic Sclerosis
Danish	Halofuginon hydrobromid	Behandling af systemisk sclerose
Dutch	Halofuginone hydrobromide	Behandeling van sclerodermie
Finnish	Halofuginoni hydrobromidi	Systeemisen skleroosin hoito
French	Halofuginone hydrobromide	Traitement de la sclérodermie systémique
German	Halofuginon hydrobromid	Therapie der systemischer Sklerose
Greek	Αλοφουγινόνη Υδροβρωμίδη	Θεραπεία της προοδευτικής συστηματικής σκλήρωσης
Italian	Alofuginone idrobromuro	Trattamento della sclerosi Sistemica Progressiva
Portuguese	Halofuginona bromohidrato	Tratamento da esclerose sistémica
Spanish	Hidrobromuro de Halofuginona	Tratamiento de esclerosis sistémica
Swedish	Halofuginon hydrobromid	Behandling av sytemisk skleros