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

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COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF iodine (^{123}I) serum amyloid P for the diagnosis of the extent of histologically proven amyloidosis

On 14 February 2003, orphan designation (EU/3/03/134) was granted by the European Commission to Mediam, France, for iodine (^{123}I) serum amyloid P for the diagnosis of the extent of histologically proven amyloidosis.
The sponsorship was transferred to Laboratoire français du fractionnement et des biotechnologies, France, in January 2006.

What is systemic amyloidosis?

Amyloid is the name of a group of proteins that can be found in the body. These proteins attached to sugar units, and are similar to starch (a protein found in plants). Normally, these proteins easily melt in water and liquids of the body, like in the blood. In some cases, however, the amyloid proteins loose their shape and become solid. The solid proteins deposit in certain parts of the body. These deposits can be harmful to organs in the body and this condition is called amyloidosis. To make the diagnosis, a small piece of tissue is taken from the body and studied with a microscope (this type of study is called histology). If the deposits are seen, this is called histologically proven amyloidosis. The damage from amyloidosis depends on what is the organ affected, and how big is the deposit. When the deposits are found throughout the body this is called systemic amyloidosis. Systemic amyloidosis is a life-threatening condition.

What are the methods of diagnosis available?

There were no authorised medicinal products for the diagnosis of the condition in the Community. However, several methods have been used in the past. Typically, small pieces of tissue are taken from the body (biopsy) and then coloured with a dye called Congo red. The colour helps to locate amyloid when looking at the tissues through a microscope. Iodine (^{123}I) serum amyloid P might be of potential significant benefit for the diagnosis of the extent of histologically proven amyloidosis. The assumption of benefits is yet to be validated and will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

What is the estimated number of patients at risk of developing the condition*?

Based on the information provided by the sponsor and previous knowledge of the Committee, systemic amyloidosis was considered to affect approximately 3 in 10,000 persons in the European Union, which, at the time of designation, corresponded to about 113,000 persons.

* 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.

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How is this medicinal product expected to act?
The product contains a substance called serum amyloid P. This is a protein that can mix with amyloid deposits in the body. The protein is also labelled with some elements that give off rays of energy (radiation) (radiolabelled with iodine 123). Such energy can create an image on a film, using a special type of camera. This technique is called scintigraphy. Thus, once given to the patient, it is expected that the protein will mix with the deposits and give off rays of energy, allowing to create a picture of where and how big the amyloid deposits are. This could help to study the extent of the disease.

What is the stage of development of this medicinal product?
At the time of submission of the application for orphan designation, clinical trials in patients with systemic amyloidosis were completed.

Iodine (^{123}I) serum amyloid P was not marketed anywhere worldwide for systemic amyloidosis, at the time of submission. Orphan designation of iodine (^{123}I) serum amyloid P has not been granted in any country for systemic amyloidosis.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 10 January 2003 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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