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 aviptadil for the treatment of sarcoidosis

On 14 September 2007, orphan designation (EU/3/07/473) was granted by the European Commission to mondoBIOTECH Laboratories Anstalt, Liechtenstein, for aviptadil for the treatment of sarcoidosis.

What is sarcoidosis?

Sarcoidosis is a chronic disease of unknown cause that affects many organs and tissues, most commonly the lungs. The disease is characterized by specific microscopic lesions called granulomas. Sarcoidosis is usually diagnosed in asymptomatic patients by routine chest radiography and is confirmed by examination of samples from affected organs using microscopic techniques. Sarcoidosis may sometimes be suspected by the patient's history of fever, fatigue, malaise, weight loss, cough and respiratory difficulties.
In general, two thirds of cases resolve spontaneously and one third of cases are long-term; in a minority of patients the disease can be life-threatening.

What are the methods of treatment available?

Oral prednisone was authorised in the European Union for the treatment of sarcoidosis at the time of submission of the application for orphan drug designation. Methotrexate, used when glucocorticoids are ineffective or not tolerated, requires up to 6 months to be effective. Lung transplantation (usually unilateral) was reserved for end-stage disease. Inhaled aviptadil may be of potential significant benefit over currently authorised drugs, due to its different mechanism of action and alternative route of administration.

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

According to the information provided by the sponsor, sarcoidosis was considered to affect about 100,000 persons in the European Union.

How is this medicinal product expected to act?

Aviptadil is a chemically produced substance that is identical to a natural hormone (a protein that circulates in the human blood), which is called Vasoactive Intestinal Peptide or VIP. Aviptadil is a vasodilator (i.e. it is able to enlarge small blood vessels) and lowers blood pressure if administered intravenously. However its mechanism of action in sarcoidosis is related to its ability to influence the immune system. This may decrease the inflammatory processes seen in sarcoidosis by acting on the white blood cells (lymphocytes and monocytes) involved in the formation of the granulomas. Since the lung is primarily affected in sarcoidosis, aviptadil will be administered by inhalation, directly to the lungs.

* 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.

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What is the stage of development of this medicinal product?
The effects of aviptadil were evaluated in experimental models.

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

Aviptadil was not authorised anywhere in the world for sarcoidosis, at the time of submission. Orphan designation has been granted in the European Union to aviptadil for the treatment of acute lung injury.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 30 July 2007 a positive opinion recommending the grant of the above-mentioned designation.

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.

For more information:

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

Language	Active Ingredient	Indication
English	Aviptadil	Treatment of sarcoidosis
Bulgarian	Авиптадил	Лечение на саркоидоза
Czech	Aviptadil	Léčba sarkoidózy
Danish	Aviptadil	Behandling af sarkoidose
Dutch	Aviptadil	Behandeling van sarcoïdose
Estonian	Aviptadil	Sarkoidoosi ravi
Finnish	Aviptadiili	Sarkoidoosin hoito
French	Aviptadil	Traitement de la sarcoïdose
German	Aviptadil	Behandlung der Sarkoidose
Greek	Aviptadil	Θεραπευτική αγωγή της Σαρκοείδωσης
Hungarian	Aviptadil	Sarcoidosis kezelése
Italian	Aviptadil	Trattamento della sarcoidosi
Latvian	Aviptadils	Sarkoidozes ārstēšana
Lithuanian	Aviptadilis	Sarkoidozės gydymas
Maltese	Aviptadil	Kura tas-sarkojidożi
Polish	Awiptadil	Leczenie sarkoidozy
Portuguese	Aviptadil	Tratamento da sarcoidose
Romanian	Aviptadil	Tratamentul sarcoidozei
Slovak	Aviptadil	Liečba sarkoidózy
Slovenian	Aviptadil	Zdravljenje sarkoidoze
Spanish	Aviptadil	Tratamiento de la sarcoidosis
Swedish	Aviptadil	Behandling av Sarkoidos
Norwegian	Aviptadil	Behandling av sarkoidose
Icelandic	Aviptadíl	Meðferð á sarklíki