



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

29 October 2013  
EMA/COMP/554715/2013  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser for the treatment of sarcoidosis

On 7 October 2013, orphan designation (EU/3/13/1191) was granted by the European Commission to Araim Pharma Europe Ltd, United Kingdom, for L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser for the treatment of sarcoidosis.

### What is sarcoidosis?

Sarcoidosis is a disease causing inflammation and injury of body tissues. It can appear in almost any body organ, but it affects most often the lungs. It is characterised by abnormal collections of inflammatory cells forming nodules in various organs which are called granulomas. Symptoms vary depending on which organs are affected. When sarcoidosis affects the nervous system the symptoms include nerve pain, hypersensitivity to heat and cold, numbness and tingling.

Sarcoidosis is a long-term debilitating disease and may be life threatening because of it affects the function of the body's organs.

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

At the time of designation, sarcoidosis affected approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 102,000 people<sup>\*</sup>, 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, corticosteroids (substances that reduce the activity of the immune system) were authorised in the EU to treat sarcoidosis. Treatment for nerve pain associated with neurosarcoidosis (sarcoidosis affecting the nerves) included gabapentin. In some patients with neurosarcoidosis surgery and radiotherapy were also used.

---

<sup>\*</sup>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 28), Norway, Iceland and Liechtenstein. This represents a population of 512,200,000 (Eurostat 2013).



The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with sarcoidosis because early studies suggest that it can improve symptoms affecting the nervous system. 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?**

This medicine attaches to and activates a receptor in body's cells called innate repair receptor (IRR), which controls inflammatory processes in the body. Once activated, IRR blocks the production of messenger molecules called cytokines which are involved in the process of inflammation. The medicine also promotes the repair of injured cells and tissues. Together, these actions are expected to reduce inflammation thereby improving the symptoms of sarcoidosis.

### **What is the stage of development of this medicine?**

The effects of the medicine have been evaluated in experimental models.

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

At the time of submission, the medicine was not authorised anywhere in the EU for sarcoidosis. Orphan designation had been granted in the United States for the treatment of neuropathic pain due to sarcoidosis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 September 2013 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:

Araim Pharma Europe Ltd  
The Coach House  
The Square  
Sawbridgeworth  
Herts CM21 9AE  
United Kingdom  
Tel. +44 012 796 003 60  
Fax +44 012 796 003 25  
E-mail: [info@araimpharmaeurope.co.uk](mailto:info@araimpharmaeurope.co.uk)

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 languages<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Treatment of sarcoidosis
Bulgarian	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Лечение на саркоидоза
Czech	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Léčba sarkoidózy
Croatian	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Liječenje sarkoidoze
Danish	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Behandling af sarkoidose
Dutch	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Behandeling van sarcoïdose
Estonian	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Sarkoidoosi ravi
Finnish	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Sarkoidoosin hoito
French	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Traitement de la sarcoïdose
German	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Behandlung der Sarkoidose
Greek	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Θεραπευτική αγωγή της Σαρκοειδωσης
Hungarian	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Sarcoidosis kezelése
Italian	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Trattamento della sarcoidosi
Latvian	L-Pir-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Sarkoidozes ārstēšana
Lithuanian	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Sarkoidozės gydymas
Maltese	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Kura tas-sarkojidoži
Polish	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Leczenie sarkoidozy
Portuguese	L-Pir-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Tratamento da sarcoïdose
Romanian	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Tratamentul sarcoidozei
Slovak	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Liečba sarkoidózy

<sup>1</sup> At the time of designation

Language	Active ingredient	Indication
Slovenian	L-Pir-L-Glu-L-Gln-L-Lev-L-Glu-L-Arg-L-Ala-L-Lev-L-Asn-L-Ser-L-Ser	Zdravljenje sarkoidoze
Spanish	L-Pir-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Tratamiento de la sarcoidosis
Swedish	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Behandling av Sarkoidos
Norwegian	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Behandling av sarkoidose
Icelandic	L-Pyr-L-Glu-L-Gln-L-Leu-L-Glu-L-Arg-L-Ala-L-Leu-L-Asn-L-Ser-L-Ser	Meðferð á sarklíki