



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Brostallicin for the treatment of soft tissue sarcoma

First publication	8 August 2006
Rev.1: transfer of sponsorship	28 August 2009
Rev.2: withdrawal from the Community Register	1 July 2013
Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in April 2013 on request of the Sponsor.

On 23 December 2005, orphan designation (EU/3/05/336) was granted by the European Commission to Nerviano Medical Sciences Srl, Italy, for brostallicin for the treatment of soft tissue sarcoma.

The sponsorship was transferred to AAIPharma S.A., France, in February 2009.

What is soft tissue sarcoma?

Soft tissue sarcomas are cancers of the supporting tissues of the body. They can occur in muscle, fat, blood vessels or in any of the other tissues that support, surround and protect the organs of the body. There are many types of soft tissue sarcoma, which tend to behave differently. The cause of soft tissue sarcoma is largely unknown. Soft tissue sarcoma is a serious condition, potentially debilitating and life-threatening.

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

At the time of designation, soft tissue sarcoma affected approximately 2.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 112,000 people^{*}, and is below the

^{*}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 25), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 466,600,000 (Eurostat 2005).



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?

Surgery is currently the main choice of therapy for early stage soft tissue sarcomas. For larger sarcomas, and where it is thought there is a possibility of cancer cells being left behind, after a surgical intervention, radiotherapy (using high-dose x-rays or other high-energy rays to kill cancer cells) and chemotherapy (using drugs to kill cancer cells) are usually used. Several products for treatment of soft tissue sarcoma were authorised for the condition in some countries in the Community at the time of submission of the application for orphan drug designation.

Brostallicin could be of potential significant benefit for the treatment of soft tissue sarcomas, mainly because it might improve the long-term outcome of the patients. 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?

Brostallicin is expected to bind to the fundamental genetic material (DNA, the cellular structure which carries the genetic information). Since the DNA is necessary for the division and survival of both normal and abnormal (i.e. tumour) cells, brostallicin might slow down or kill cancer cells which are actively dividing cells.

What is the stage of development of this medicine?

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

The medicinal product was not authorised anywhere worldwide for soft tissue sarcoma or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 November 2005 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	Brostallicin	Treatment of soft tissue sarcoma
Bulgarian	Бросталицин	Лечение на саркома на меките тъкани
Czech	Brostallicínu	Léčba sarkomu měkkých tkání
Danish	Brostallicin	Behandling af bløddelssarkom
Dutch	Brostallicine	Behandeling weke delen sarcoom
Estonian	Brostallitsiin	Pehmeete kudede sarkoomi ravi
Finnish	Brostallisiini	Pehmytkudossarkooman hoito
French	Brostallicine	Traitement des sarcomes des tissus mous
German	Brostallizin	Behandlung des Weichteilsarkoms
Greek	Βροσταλλικίνη	Θεραπεία του σαρκώματος των μαλακών ιστών
Hungarian	Brostallicin	Lágy szöveti sarcoma kezelése
Italian	Brostallicina	Trattamento dei sarcomi dei tessuti molli
Latvian	Brostallicīns	Mīksto audu sarkomas ārstēšana
Lithuanian	Brostallicino	Minkštųjų audinių sarkomos gydymas
Maltese	Brostallicin	Kura tas-sarkoma tat-tessuti rotob
Polish	Brostallicyna	Leczenie mięsaków tkanek miękkich
Portuguese	Brostallicina	Tratamento do sarcoma dos tecidos moles
Romanian	Brostallicin	Tratamentul sarcoamelor țesuturilor moi
Slovak	Brostallicin	Liečba sarkómu mäkkých tkanív
Slovenian	Brostallicin	Zdravljenje sarkoma mehkih tkiv
Spanish	Brostallicina	Tratamiento del sarcoma de tejidos blandos
Swedish	Brostallicin	Behandling av mjukdelssarkom
Norwegian	Brostallicin	Behandling av bløtvevssarkom
Icelandic	Brostallicín	Meðferð við mjúkvefjasarkmeini

¹ At the time of transfer of sponsorship