10 June 2014
EMA/COMP/69581/2011 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Ombrabulin for the treatment of soft tissue sarcoma

First publication	20 April 2011
Rev.1: sponsor's name and address change	5 April 2012
Rev.2: withdrawal from the Community Register	10 June 2014
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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in April 2014 on request of the Sponsor.

On 15 April 2011, orphan designation (EU/3/11/853) was granted by the European Commission to Sanofi-Aventis, France, for ombrabulin for the treatment of soft tissue sarcoma.

In October 2012, Sanofi Aventis changed name to Sanofi-Aventis Groupe.

What is soft tissue sarcoma?

Soft tissue sarcoma is a type of cancer that affects the soft, supportive tissues of the body. It can occur in muscles, blood vessels, fat tissue or in other tissues that support, surround and protect organs. Patients with soft tissue sarcoma do not usually have symptoms in the early stages of the disease. First symptoms appear when the tumour grows large enough to cause swelling and pain.

Soft tissue sarcoma is a serious and life-threatening disease particularly when the cancer has spread to other parts of the body.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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 122,000 people*, 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, the main treatment for early-stage soft tissue sarcoma was surgery. For large sarcomas, surgery was usually followed by radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer) to kill any cancerous cells that were left behind. Several medicines were authorised in the EU for the treatment of soft tissue sarcoma.

The sponsor has provided sufficient information to show that ombrabulin might be of significant benefit for patients with soft tissue sarcoma because it works in a different way to existing treatments, and early studies show that it might be used in combination with chemotherapy to improve the outcome of patients with this condition. These assumptions 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?

Ombrabulin is derived from combretastatin, a natural substance extracted from a bark of a South African tree. It is expected to work in soft tissue sarcoma mainly by disrupting the vessels that bring blood to the tumour, blocking the normal flow of the blood. Cancer cells grow rapidly and thus require a large amount of blood. By blocking the blood flow, ombrabulin is expected to stop the growth of cancer cells, which eventually die.

Ombrabulin is also expected to disrupt the division of cancer cells by attaching to a protein in cells called ‘tubulin’, which is important in the formation of the internal ‘skeleton’ that cells need to assemble when they divide.

What is the stage of development of this medicine?

The effects of ombrabulin have been evaluated in experimental models.

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

At the time of submission, ombrabulin was not authorised anywhere in the EU for soft tissue sarcoma or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 January 2011 recommending the granting of this designation.

_____

*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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 507,700,000 (Eurostat 2011).

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:

Sanofi-Aventis Groupe
54 rue de la Boétie
75008 Paris
France
Tel. +33 1 53 77 40 00
Fax +33 1 53 77 41 33
www.sanofi-aventis.com/contact/contact.asp

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[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Ombrabulin	Treatment of soft tissue sarcoma
Bulgarian	Омрабулин	Лечение на сарком на меките тъкани
Czech	Ombrabulin	Léčba sarkomu měkkých tkání
Danish	Ombrabulin	Behandling af bløddelssarkom
Dutch	Ombrabuline	Behandeling weke delen sarcoom
Estonian	Ombrabuliin	Pehmete kudede sarkoomi ravi
Finnish	Ombrabuliini	Pehmytkudossarkooman hoito
French	Ombrabuline	Traitement des sarcomes des tissus mous
German	Ombrabulin	Behandlung des Weichteilsarkoms
Greek	Ομπραμπουλίνη	Θεραπεία του σαρκώματος των μαλακών ιστών
Hungarian	Ombrabulin	Lágy szöveti sarcoma kezelése
Italian	Ombrabulina	Trattamento dei sarcomi dei tessuti molli
Latvian	Ombrabulīns	Mīksto audu sarkomas ārstēšana
Lithuanian	Ombrabulinas	Minkštųjų audinių sarkomos gydymas
Maltese	Ombrabulin	Kura tas-sarkoma tat-tessuti rotob
Polish	Ombrabulina	Leczenie mięsaków tkanek miękkich
Portuguese	Ombrabulina	Tratamento do sarcoma dos tecidos moles
Romanian	Ombrabulină	Tratamentul sarcomului ţesuturilor moi
Slovak	Ombrabulin	Liečba sarkómu mäkkých tkanív
Slovenian	Ombrabulin	Zdravljenje sarkoma mehkih tkiv
Spanish	Ombrabulina	Tratamiento del sarcoma de tejidos blandos
Swedish	Ombrabulin	Behandling av mjukdelssarkom
Norwegian	Ombrabulin	Behandling av bløtvevssarkom
Icelandic	Ombrabúlín	Meðferð við mjúkvefjasarkmeini

[1] At the time of designation