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EMA/COMP/25527/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Olaratumab for the treatment of soft tissue sarcoma

On 12 February 2015, orphan designation (EU/3/15/1447) was granted by the European Commission to Elli Lilly Nederland B.V., the Netherlands, for olaratumab for the treatment of soft tissue sarcoma.

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 long-term debilitating and life-threatening disease, particularly when the cancer has spread to other parts of the body.

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

At the time of designation, soft tissue sarcoma affected not more than 3 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 154,000 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 including doxorubicin.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with soft tissue sarcoma because early studies indicate that it might improve 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 28), Norway, Iceland and Liechtenstein. This represents a population of 512,900,000 (Eurostat 2015).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

outcome of patients when combined with doxorubicin. 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?

Olaratumab is a monoclonal antibody, a type of protein that has been designed to recognise and attach to a protein called platelet-derived growth factor receptor alpha (PDGFRα). This protein is often found on the surface of cells where it is thought to play a role in regulating cell multiplication. In cancers such as soft tissue sarcoma these proteins are present in high levels or overactive, causing cells to become cancerous. When olaratumab attaches to PDGFRα on sarcoma cells, it is expected to block its activity, thereby slowing down the growth of the cancer.

What is the stage of development of this medicine?

The effects of olaratumab have been evaluated in experimental models.

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

At the time of submission, olaratumab was not authorised anywhere in the EU for soft tissue sarcoma. Orphan designation of olaratumab had been granted in the United States of America for the treatment of soft tissue sarcoma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 January 2015 recommending the granting of this designation.

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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:

Eli Lilly Nederland B.V.
Grootslag 1-5
NL-3991 RA Houten
The Netherlands
Tel. +31 30 60 25 800
Fax +31 30 60 25 888
E-mail: eu_orphan@lilly.com

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

[1] At the time of designation