5 September 2016
EMA/COMP/451315/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sirolimus for the treatment of sporadic lymphangioleiomyomatosis

On 14 July 2016, orphan designation (EU/3/16/1704) was granted by the European Commission to Best Regulatory Consulting Ltd, United Kingdom, for sirolimus for the treatment of sporadic lymphangioleiomyomatosis.

What is sporadic lymphangioleiomyomatosis?

Lymphangioleiomyomatosis is a disease that affects mostly women and is caused by the spread of immature muscle-like cells (known as LAM cells) to the lungs, lymph nodes and the kidneys. Patients with lymphangioleiomyomatosis often have problems with breathing, accumulation of fluid in the chest and abdomen (belly), and masses in the abdomen, which can cause bleeding. The sporadic form of lymphangioleiomyomatosis has more severe effects on breathing.

Sporadic lymphangioleiomyomatosis is life-threatening and debilitating in the long term because of worsening lung function and other lung problems, as well as bleeding and complications arising from the presence of masses in the abdomen and pelvis.

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

At the time of designation, sporadic lymphangioleiomyomatosis affected approximately 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 5,000 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 the orphan designation, no satisfactory methods of treatment were authorised for sporadic lymphangioleiomyomatosis. Treatment for severe symptoms of the disease included surgery and lung transplantation.

[*]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 513,700,000 (Eurostat 2016).

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How is this medicine expected to work?

Sirolimus blocks the activity of mTOR, a protein involved in the proliferation of LAM cells. By blocking mTOR, the medicine is expected to reduce the number of LAM cells in the lungs, thereby helping to relieve patients' symptoms.

What is the stage of development of this medicine?

The effects of sirolimus have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with sirolimus in patients with sporadic lymphangioleiomyomatosis were ongoing.

At the time of submission, sirolimus was authorised in the EU for preventing rejection of transplanted kidneys. Oral (by mouth) sirolimus has also been authorised in the United States for the treatment of lymphangioleiomyomatosis.

At the time of submission, sirolimus was not authorised anywhere in the EU for sporadic lymphangioleiomyomatosis. Orphan designation had been granted in the United States for sirolimus for the treatment of lymphangioleiomyomatosis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 16 June 2016 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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	Sirolimus	Treatment of sporadic lymphangioleiomyomatosis
Bulgarian	Сиролимус	Лечение на спорадична лимфангиолейомиоматоза
Croatian	Sirolimus	Liječenje sporadične limfangiolejomiomatoze
Czech	Sirolimus	Léčba sporadické lymfangioleiomyomatózy
Danish	Sirolimus	Behandling af sporadisk lymphangioleiomyomatosis
Dutch	Sirolimus	Behandeling van sporadisch lymfangioleiomyomatose
Estonian	Siroliimus	Sporaadilise lümfangioleiomüomatoos ravi
Finnish	Sirolimuusi	Sporadisen lymfangioleiomyomatoosin hoito
French	Sirolimus	Traitement de la lymphangioléiomyomatose sporadique
German	Sirolimus	Behandlung von sporadischer Lymphangioleiomyomatose
Greek	Σιρόλιμους	Αντιμετώπιση της σποραδικής λεμφαγγειολειομυομάτωσης
Hungarian	Szirolimusz	Sporadikus lymphangioleiomyomatosis kezelésére
Italian	Sirolimus	Trattamento della linfangioleiomiomatosi sporadica
Latvian	Sirolimus	Sporādiskas limfangioleiomiomatozes ārstēšana
Lithuanian	Sirolimuzas	Sporadinės limfangiolejomiomatozės gydymas
Maltese	Sirolimus	Kura tal-limfanġolejomijomatosi sporadika
Polish	Syrolimus	Leczenie limfangioleiomiomatozy
Portuguese	Sirolimus	Tratamento da linfangioleiomiomatose esporádica
Romanian	Sirolimus	Tratamentul limfangioleiomiomatozei sporadice
Slovak	Sirolimus	Liečba sporadickej lymfangioleiomyomatózou
Slovenian	Sirolimus	Zdravljenje sporadične limfangioleiomiomatozo
Spanish	Sirolimus	Tratamiento de la linfangioleiomiomatosis esporádica
Swedish	Sirolimus	Behandling av sporadisk lymfangioleiomyomatos
Norwegian	Sirolimus	Behandling av sporadisk lymfoangioleiomyomatose
Icelandic	Sírólímus	Meðferð sléttvöðvahnúta í lungum (lymphangioleiomyomatosis)

[1] At the time of designation