



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Crenolanib besylate for the treatment of soft tissue sarcoma

On 14 October 2016, orphan designation (EU/3/16/1749) was granted by the European Commission to Arog Pharmaceuticals Europe Ltd, Ireland, for crenolanib besylate for treatment of soft tissue sarcoma.

#### What is soft tissue sarcoma?

Soft tissue sarcoma is a cancer that affects the soft 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 approximately 2.8 in 10,000 people in the European Union (EU). This was equivalent to a total of around 144,000 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 cancer 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 this medicine might be of significant benefit for patients with soft tissue sarcoma, with early data showing improvement in patients with

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\*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).



advanced cancer who had received other treatments previously. 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?**

Crenolanib besylate is a 'tyrosine-kinase inhibitor'. This means that it blocks the activity of enzymes known as tyrosine kinases found in certain receptors in cancer cells. This medicine blocks receptors called PDGFRs, which are involved in stimulating soft tissue sarcoma cells to divide uncontrollably. By blocking these receptors, crenolanib besylate is expected to stop cell division and lead to cell death, and thus slow down the progression of the disease.

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

The effects of crenolanib besylate 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 soft tissue sarcoma were ongoing.

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 September 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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Crenolanib besylate	Treatment of soft tissue sarcoma
Bulgarian	Креноланиб безилат	Лечение на сарком на меките тъкани
Croatian	Crenolanib besilat	Liječenje sarkoma mekih tkiva
Czech	Crenolanib besilat	Léčba sarkomu měkkých tkání
Danish	Crenolanib besilat	Behandling af bløddelsarkom
Dutch	Crenolanib besilaat	Behandeling weke delen sarcoom
Estonian	Crenolanib besülaat	Pehmeete kudede sarkoomi ravi
Finnish	Krenolanibi besilaatti	Pehmytkudossarkooman hoito
French	Crenolanib bésylate	Traitement des sarcomes des tissus mous
German	Crenolanib besilat	Behandlung des Weichteilsarkoms
Greek	Κρενολάνιμπη βεσυλική	Θεραπεία του σαρκώματος των μαλακών ιστών
Hungarian	Crenolanib bezilát	Lágy szöveti sarcoma kezelése
Italian	Crenolanib besilato	Trattamento dei sarcomi dei tessuti molli
Latvian	Krenolaniba besilāts	Mīksto audu sarkomas ārstēšana
Lithuanian	Krenolanibo besilatas	Minkštųjų audinių sarkomos gydymas
Maltese	Crenolanib besylate	Kura tas-sarkoma tat-tessuti rotob
Polish	Krenolanib bezylanu	Leczenie mięsaków tkanek miękkich
Portuguese	Besilato de crenolanibe	Tratamento do sarcoma dos tecidos moles
Romanian	Crenolanib besilat	Tratamentul sarcomului țesuturilor moi
Slovak	Crenolanib besilát	Liečba sarkómu mäkkých tkanív
Slovenian	Krenolanib bezilat	Zdravljenje sarkoma mehkih tkiv
Spanish	Besilato de crenolanib	Tratamiento del sarcoma de tejidos blandos
Swedish	Crenolanib besilat	Behandling av mjukdelssarkom
Norwegian	Krenolanibbesilat	Behandling av bløtvevssarkom
Icelandic	Crenólanib besýlat	Meðferð við mjúkvefjasarkmeini

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