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Public summary of opinion on orphan designation

Larotrectinib for the treatment of salivary gland cancer

On 21 March 2018, orphan designation (EU/3/18/1995) was granted by the European Commission to Loxo Oncology Limited, United Kingdom, for larotrectinib for the treatment of salivary gland cancer.

What is salivary gland cancer?

Salivary gland cancer is a cancer of the glands that make saliva. Tumours often start off as painless lumps and can form in the mouth, tongue, nose, ear and neck. Salivary gland cancer is a life-threatening disease that can spread and worsen quickly.

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

At the time of designation, salivary gland cancer affected approximately 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 26,000 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, no satisfactory methods were authorised in the EU for the treatment of salivary gland cancer. Patients underwent surgery to remove the cancer if possible.

How is this medicine expected to work?

Some patients with salivary gland cancer have genetic mutations (changes) called *NTRK*-fusion mutations which result in the production of altered TRK proteins that can cause cancer.

This medicine blocks the activity of the altered TRK proteins, thus preventing or slowing down the growth of salivary gland cancer.

*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 517,400,000 (Eurostat 2018).

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

What is the stage of development of this medicine?

The effects of larotrectinib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with larotrectinib in patients with solid tumours were ongoing.

At the time of submission, larotrectinib was not authorised anywhere in the EU for salivary gland cancer. Orphan designation of larotrectinib had been granted in the US for treatment of solid tumors with NTRK-fusion proteins.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 February 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	Larotrectinib	Treatment of salivary gland cancer
Bulgarian	Ларотректиниб	Лечение на рак на слюнчените жлези
Croatian	Larotrectinib	Liječenje raka žlijezde slinovnice
Czech	Larotrectinib	Léčba karcinomu slinné žlázy
Danish	Larotrectinib	Behandling af spytkirtelcancer
Dutch	Larotrectinib	Behandeling van speekselklierkanker
Estonian	Larotrektiniib	Süljenäärmevähi ravi
Finnish	Larotrektinibi	Sylkirauhassyövän hoito
French	Larotrectinib	Traitement du cancer des glandes salivaires
German	Larotrectinib	Behandlung des Speicheldrüsenkarzinoms
Greek	Λαροτρεκτινίμπη	Θεραπεία καρκίνου των σιελογόνων αδένων
Hungarian	Larotrectinib	Nyálmirigy-karcinóma kezelése
Italian	Larotrectinib	Trattamento del carcinoma delle ghiandole salivari
Latvian	Larotrektinibs	Siekalu dziedzeru vēža ārstēšana
Lithuanian	Larotrektinibas	Seilių liaukų vėžio gydymas
Maltese	Larotrektinib	Kura tal-kanċer tal-glandoli salivarji
Polish	Larotrektynib	Leczenie raka ślinianek
Portuguese	Larotrectinib	Tratamento do cancro das glândulas salivares
Romanian	Larotrectinib	Tratamentul cancerului glandelor salivare
Slovak	Larotrektinib	Liečba rakoviny slinných žliaz
Slovenian	Larotrektinib	Zdravljenje raka žlez slinavk
Spanish	Larotrectinib	Tratamiento del cáncer de glándulas salivales
Swedish	Larotrektinib	Behandling av spottkörtelcancer
Norwegian	Larotrektinib	Behandling av spyttkjertelkreft
Icelandic	Larótrektíníb	Meðferð við krabbameini í munnvatnskirtli

[1] At the time of designation