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Public summary of opinion on orphan designation

Selumetinib for the treatment of neurofibromatosis type 1

On 31 July 2018, orphan designation (EU/3/18/2050) was granted by the European Commission to AstraZeneca AB, Sweden, for selumetinib for the treatment of neurofibromatosis type 1.

What is neurofibromatosis type 1?

Neurofibromatosis type 1 is an inherited disease in which the patient develops benign (non-cancerous) tumours along the nerves. The severity of the disease varies from patient to patient, and symptoms include pale, coffee-coloured patches, freckles in unusual places (such as the armpits, groin and under the breasts), high blood pressure, problems with the bones, eyes and nervous system, learning difficulty and short stature. Patients can also develop cancer, including cancer of the optic nerve (the nerve that sends signals from the eye to the brain).

The disease is caused by mutations (changes) in a gene called *NF1*, which leads to uncontrolled growth of cells in the nervous system.

Neurofibromatosis type 1 is a debilitating disease because of the damage caused by the tumours. The disease may also be life threatening due to the increased risk of developing cancer.

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

At the time of designation, neurofibromatosis type 1 affected approximately 3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 155,000 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, there were no treatments authorised in the EU for neurofibromatosis type 1. Surgery was used to remove tumours, and chemotherapy (medicines for treating cancer) was used for cancers caused by the condition.

[*]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

How is this medicine expected to work?

Selumetinib blocks enzymes called MEK1/2 which are involved in stimulating cells to grow. MEK1/2 are overactive in certain types of cancer, which makes cells grow uncontrollably. By blocking these enzymes, the medicine is expected to slow down growth of the tumour cells in neurofibromatosis type 1.

What is the stage of development of this medicine?

The effects of selumetinib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with selumetinib in patients with neurofibromatosis type 1 were ongoing.

At the time of submission, selumetinib was not authorised anywhere in the EU for neurofibromatosis type 1. Orphan designation of the medicine 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 21 June 2018 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	Selumetinib	Treatment of neurofibromatosis type 1
Bulgarian	Селуметиниб	Лечение на неврофиброматоза тип 1
Croatian	Selumetinib	Liječenje neurofibromatoze tipa 1
Czech	Selumetinib	Léčba neurofibromatózy typu 1
Danish	Selumetinib	Behandling af neurofibromatose type 1
Dutch	Selumetinib	Behandeling van neurofibromatose type 1
Estonian	Selumetiniib	1.tüüpi neurofibromatoosi ravi
Finnish	Selumetinibi	Tyypin 1 neurofibromatoosin hoito
French	Sélumétinib	Traitement de la neurofibromatose de type 1
German	Selumetinib	Die Behandlung der Neurofibromatose-Typ-1
Greek	Σελουμετινίμπη	Θεραπεία της νευροϊνωμάτωσης-τύπου 1
Hungarian	Selumetinib	1-es típusú neurofibromatózis kezelése
Italian	Selumetinib	Trattamento di neurofibromatosi di tipo 1
Latvian	Selumetinibs	1. tipa neirofibromatozes ārstēšana
Lithuanian	Selumetinibas	Neurofibromatozės (1 tipo) gydymas
Maltese	Selumetinib	Trattament tan-newrofibromatosi tat-tip 1
Polish	Selumetinib	Leczenie nerwiakowłókniakowatości typu 1
Portuguese	Selumetinib	Tratamento da neurofibromatose de tipo 1
Romanian	Selumetinib	Tratamentul neurofibromatozei tip 1
Slovak	Selumetinib	Liečba neurofibromatózy typu 1
Slovenian	Selumetinib	Zdravljenje neurofibromatoze tipa 1
Spanish	Selumetinib	Tratamiento de la neurofibromatosis de tipo 1
Swedish	Selumetinib	Behandling av neurofibromatos typ 1
Norwegian	Selumetinib	Behandling av nevrofibromatose type 1
Icelandic	Selumetinib	Meðferð við taugatrefjaæxlageri-gerð 1

[1] At the time of designation