6 April 2018
EMA/52868/2018

Public summary of opinion on orphan designation

Seletalisib for the treatment of activated phosphoinositide 3-kinase delta syndrome

On 22 February 2018, orphan designation (EU/3/18/1986) was granted by the European Commission to UCB Biopharma SPRL, Belgium, for seletalisib for the treatment of activated phosphoinositide 3-kinase delta syndrome.

What is activated phosphoinositide 3-kinase delta syndrome?

Activated phosphoinositide 3-kinase delta syndrome is an inherited disorder where the patient is unable to fight infections because the immune system (the body's natural defences) does not work properly.

It is caused by defects in the gene which makes a protein called phosphoinositide 3-kinase delta. This protein is essential for the development of B and T cells, white blood cells that play a key role in the immune system. The defect makes phosphoinositide 3-kinase delta overactive, which interferes with the normal development of B and T cells and their ability to fight infections. Patients with the disorder are highly susceptible to infections. The main symptoms usually occur in the first two years of life, and include repeated lung infections and a failure to grow and develop normally.

Activated phosphoinositide 3-kinase delta syndrome is a long-term debilitating and life-threatening condition due to repeated lung infections that can lead to bronchiectasis (enlargement and inflammation of part of the airways) as well a reduction in the white blood cell count.

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

At the time of designation, activated phosphoinositide 3-kinase delta syndrome affected approximately 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of around 500 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).

*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).

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What treatments are available?

At the time of designation of the review of the orphan designation, no satisfactory treatments were authorised in the EU for patients affected by activated phosphoinositide 3-kinase delta syndrome. The main treatment included medicines to help control infection, such as immunoglobulin replacement therapy.

How is this medicine expected to work?

The medicine attaches to phosphoinositide 3-kinase delta and blocks its action. This reduces the excessive activity of the protein, which is expected to help to restore normal development of B and T cells and their ability to fight infections.

What is the stage of development of this medicine?

The effects of seletalisib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with seletalisib in patients with activated phosphoinositide 3-kinase delta syndrome were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for activated phosphoinositide 3-kinase delta syndrome. 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 18 January 2018 recommending the granting of this designation.

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	Seletalisib	Treatment of activated phosphoinositide 3-kinase delta syndrome
Bulgarian	Селеталисиб	Лечение на синдрома на активирана фосфоинозитид 3-киназа делта
Croatian	Seletalisib	Liječenje sindroma aktivirane fosfatidilinozitol 3 kinaze delta
Czech	Seletalisib	Léčba syndromu aktivované PI3 kinázy
Danish	Seletalisib	Behandling af aktiveret fosfoinositide 3-kinase deltasyndrom
Dutch	Seletalisib	Behandeling van geactiveerd PI3 kinase delta syndroom
Estonian	Seletalisiib	Aktiveeritud fosfoinositiid-3-kinaas delta sündroomi ravi
Finnish	Seletalisibi	Aktivoidun fosfatidyyli-inositoli-3-kinaasi delta oireyhtymän hoito
French	Sélétalisib	Traitement du syndrome phosphoinositide 3-kinase delta activé
German	Seletalisib	Aktivierendes PIK3-delta-Syndrom
Greek	Σελεταλισίμπη	Θεραπεία του συνδρόμου ενεργοποιημένης 3-κινάσης δέλτα των φωσφοϊνοσιτιδίων
Hungarian	Szeletalizib	Aktivált foszfoinozitid-3-kináz delta szindróma kezelése
Italian	Seletalisib	Trattamento della sindrome da attivazione della fosfatidilinositol-3chinasi-delta
Latvian	Seletalisibs	Aktivizētā fosfoinositīda 3-kināzes delta sindroma ārstēšana
Lithuanian	Seletalisibas	Aktyvuoto fosfoinozitido 3-kinazės delta sindromo gydymas
Maltese	Seletalisib	Trattament ta' sindrome ta’ fosfojnositidu 3-kinaži delta attivat
Polish	Seletalisib	Leczenie zespołu aktywacji kinazy 3-fosfatydyloinozytolu-delta
Portuguese	Seletalisib	Tratamento do síndrome delta fosfatidilinositol 3-quinase ativada
Romanian	Seletalisib	Tratamentul sindromului fosfoinozitid 3-kinazei delta activate
Slovak	Seletalisib	Liečba syndrómu aktivovanej fosfoinozitid 3-kinázy delta
Slovenian	Seletalisib	Zdravljenje aktiviranega fosfoinositid 3-kinaza delta sindroma
Spanish	Seletalisib	Tratamiento del síndrome de PI3K-delta activado
Swedish	Seletalisib	Behandling av aktiverat PI3K-deltasyndrom
Norwegian	Seletalisib	Behandling av aktivert fosfoinositid 3-kinase deltasyndrom
Icelandic	Seletaísíb	Meðhöndlun gegn heilkenni virkjaðra fosfóinósítíð 3-kínasa delta

[1] At the time of designation