

7 May 2018
EMA/116423/2018

Public summary of opinion on orphan designation

Patidegib for the treatment of naevoid basal-cell carcinoma syndrome (Gorlin syndrome)

On 21 March 2018, orphan designation (EU/3/18/1998) was granted by the European Commission to Blue-Reg Europe, France, for patidegib for the treatment of naevoid basal-cell carcinoma syndrome (Gorlin syndrome).

What is naevoid basal-cell carcinoma syndrome?

Naevoid basal-cell carcinoma syndrome (also known as Gorlin syndrome) is a genetic condition where patients develop various cancerous and non-cancerous (benign) tumours in many areas of the body.

In people with Gorlin syndrome, the most common cancer is basal-cell carcinoma, a slow-growing type of skin cancer, which usually develops on the face, chest and back. Most people also develop non-cancerous tumours of the jaw which cause face swelling and problems with teeth. Skeletal problems in the spine, ribs and skull are also common.

Gorlin syndrome is a long-term debilitating condition because of the multiple tumours which can lead to deformities.

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

At the time of designation, Gorlin syndrome affected approximately 0.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 10,000 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, fluorouracil, imiquimod, sonidegib, tretinoin and vismodegib were authorised in the EU for treating basal-cell carcinoma.

*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).

The sponsor has provided sufficient information to show that patidegib might be of significant benefit for patients with Gorlin syndrome. Preliminary data showed that it reduced the number of new tumours. The fact that the medicine will be applied to the skin may make long-term treatment easier for patients who cannot take the authorised treatments.

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?

Patidegib is expected to work by blocking 'the Hedgehog signalling pathway', which controls several processes in the body such as cell growth. In Gorlin syndrome, the Hedgehog signalling pathway becomes abnormally active and leads to the growth and spread of cancerous cells. Patidegib is thought to attach to a protein called 'SMO', which is involved in activating the Hedgehog signalling pathway. By attaching to SMO, patidegib is expected to block this pathway, thereby slowing down the growth and spread of the cancer cells.

What is the stage of development of this medicine?

The effects of patidegib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with patidegib in patients with Gorlin syndrome were ongoing.

At the time of submission, patidegib was not authorised anywhere in the EU for Gorlin syndrome. Orphan designation of patidegib had been granted in United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 February 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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Patidegib	Treatment of naevoid basal-cell carcinoma syndrome (Gorlin syndrome)
Bulgarian	Патидегиб	Лечение на синдром на Gorlin-невоидна базалноклетъчна карцинома
Croatian	Patidegib	Liječenje sindroma nevoidnog karcinoma bazalnih stanica (ili Gorlinovog sindroma)
Czech	Patidegib	Léčba nevoidního basalioidního karcinomu (Gorlin syndrome)
Danish	Patidegib	Behandling af nevoid basalcelle carcinom syndrom (Gorlin syndrom)
Dutch	Patidegib	Behandeling van naevoid basaalcelcarcinoom syndroom (Gorlin syndroom)
Estonian	Patidegib	Nevoidse basaarakulise kartsinoomi sündroomi (Gorlini sündroom) ravi
Finnish	Patidegibi	Nevoidi tyvisolukarsinoomaoireyhtymän (Gorlinin oireyhtymän) hoito
French	Patidegib	Traitement de la naevomatose basocellulaire (syndrome de Gorlin)
German	Patidegib	Behandlung des naevoiden Basalioms (Gorlin-Syndrom)
Greek	Πατιδεγίμπη	Σύνδρομο σπιλοειδούς βασικοκυτταρικού καρκινώματος (σύνδρομο Gorlin)
Hungarian	Patidegib	Basalsejtes naevoid carcinoma szindróma (Gorlin-szindróma) kezelése
Italian	Patidegib	Trattamento della sindrome del carcinoma nevoide a cellule basali, (sindrome di Gorlin)
Latvian	Patidegibs	Bazālo nevus šūnu karcinomas sindroma (Gorlin sindroma) ārstēšanai
Lithuanian	Patidegibas	Apgamo pamatinio sluoksnio ląstelių karcinomos sindromo (Gorlin sindromo) gydymas
Maltese	Patidegib	Kura tas-sindrome ta' karċinoma taċ-ċelluli bażali nevojdi (sindrome ta' Gorlin)

¹ At the time of designation

Language	Active ingredient	Indication
Polish	Patidegib	Leczenie zespołu znamionowych nabłoniaków podstawnkomórkowych (Zespół Gorlina)
Portuguese	Patidegib	Síndrome dos carcinomas basocelulares nevóides (síndrome de Gorlin)
Romanian	Patidegib	<i>Tratamentul sindromului</i> carcinomului nevilor bazocelulari (sindromul Gorlin)
Slovak	Patidegib	Liečba syndrómu névoidného bazaliómu (Gorlinovho syndrómu)
Slovenian	Patidegib	Zdravljenje sindroma nevoidnih bazalnoceličnih karcinomov (Gorlinov karcinom),
Spanish	Patidegib	Síndrome del carcinoma nevoide de células basales (SCNCB) (síndrome de Gorlin)
Swedish	Patidegib	behandling av nevoid basalcell karcinom syndrom (Gorlins syndrom)
Norwegian	Patidegib	Behandling av nevoid basalcellekarsinom syndrom (Gorlins syndrom)
Icelandic	Patidegib	Meðferð fæðingarbletta grunnfrumukrabbameins heilkennis (Gorlin heilkenni)