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Public summary of opinion on orphan designation

Pemigatinib for the treatment of myeloid/lymphoid neoplasms with eosinophilia and rearrangement of PDGFRA, PDGFRB, or FGFR1, or with PCM1-JAK2

On 17 October 2019, orphan designation EU/3/19/2216 was granted by the European Commission to Incyte Biosciences Distribution B.V., the Netherlands, for pemigatinib for the treatment of myeloid/lymphoid neoplasms with eosinophilia and rearrangement of PDGFRA, PDGFRB, or FGFR1, or with PCM1-JAK2.

What are myeloid/lymphoid neoplasms with eosinophilia and rearrangement of PDGFRA, PDGFRB, or FGFR1, or with PCM1-JAK2?

Myeloid/lymphoid neoplasms with eosinophilia and rearrangement of PDGFRA, PDGFRB, or FGFR1, or with PCM1-JAK2 are a group of cancers affecting the bone marrow and the white blood cells. The condition is often characterised by high levels of a type of white blood cells called eosinophils, and is caused by changes (rearrangements) in the genes for some proteins called receptor tyrosine kinases (PDGFRA, PDGFRB, FGFR1, and PCM1-JAK2). These proteins play a key role in the transmission of signals within cells that control cell growth, division and survival. As a result of the changes, these proteins are overactive and increase cell division, survival and the formation of new blood vessels, leading to cancer.

The condition is debilitating in the long term and life threatening due to its aggressive nature, with a high number of patients developing leukaemia that is resistant to conventional chemotherapy. It is associated with poor long-term survival.

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

At the time of designation, the condition affected less than 0.7 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 36,000 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).

*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 518,400,000 (Eurostat 2019).

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What treatments are available?

At the time of designation, the main treatments for this condition were chemotherapy (medicines to treat cancer) and haematopoietic (blood) stem-cell transplantation (a procedure where the patient's bone marrow is cleared of cells and replaced by stem cells to form new bone marrow that produces healthy blood cells).

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with the condition. Early studies show that patients affected by myeloid or lymphoid neoplasms associated with FGFR1 rearrangement who received previous treatment responded to this medicine.

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?

Pemigatinib belongs to a group of medicines called protein kinase inhibitors. It works by blocking protein kinases, including the overactive receptor tyrosine kinases in patients with the condition.

By blocking the overactive proteins, pemigatinib is expected to reduce the growth and spread of the cancer.

What is the stage of development of this medicine?

The effects of pemigatinib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with pemigatinib in patients with the condition were ongoing.

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

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 12 September 2019, 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.

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	Pemigatinib	Treatment of myeloid/lymphoid neoplasms with eosinophilia and rearrangement of PDGFRA, PDGFRB, or FGFR1, or with PCM1-JAK2
Bulgarian	Пемигатиниб	Лечение на миелоидни/лимфоидни неоплазии, с еозинофилия и пренареждане на PDGFRA, PDGFRB или FGFR1 или с PCM1-JAK2
Croatian	Pemigatinib	Liječenje mijeloidnih / limfoidnih novotvorina s eozinofilijom i preuređivanjem PDGFRA, PDGFRB ili FGFR1 ili PCM1-JAK2
Czech	Pemigatinib	Léčba myeloidní/lymfoidní neoplázie s eozinofilií a přestavbou PDGFRA, PDGFRB nebo FGFR1, nebo s PCM1-JAK2
Danish	Pemigatinib	Behandling af myeloide/lymfoide neoplasmer med eosinophili og rearrangement af PDGFRA, PDGFRB eller FGFR1, eller med PCM1-JAK2
Dutch	Pemigatinib	Behandeling van myeloid/ lymfoïd neoplasma met eosinophilia en herschikking van PDGFRA, PDGFRB, or FGFR1, of met PCM1-JAK2
Estonian	Pemigatiniib	PCM1-JAK2 ümberkorraldusest või PDGFRA, PDGFRB või FGFR1 ümberkorraldusest põhjustatud ja kaasuva eosinofiiliaga müeloid- või lümfoidkasvaja ravi
Finnish	Pemigatinibi	Myeeloisen / lymfaattisen neoplasman hoito, johon liittyy eosinofilia ja uudelleenjärjest PDGFRA:n, PDGFRB:n, tai FGFR1:n, tai yhdessä PCM1-JAK2:n kanssa
French	Pemigatinib	Traitement des néoplasmes myéloïdes/lymphoïdes avec éosinophiles et anomalies PDGFRA, PDGFRB, FGFR1, ou PCM1-JAK2
German	Pemigatinib	Behandlung von myeloischen/lymphatischen Neoplasien mit Eosinophilie und Gen-Rearrangement von PDGFRA, PDGFRB, oder FGFR1, PCM1-JAK2
Greek	Πεμιγκατινίμπη	Θεραπεία των μυελοειδών/λεμφοειδών νεοπλασμάτων με ιωσινοφιλία και αναδιάταξη του PDGFRA, του PDGFRB, ή του FGFR1, ή με PCM1-JAK2
Hungarian	Pemigatinib	Eozinofiliával és PDGFRA, PDGFRB vagy FGFR1 átrendeződéssel, vagy PCM1-JAK2-vel járó myeloid és lymphoid neoplazmák kezelése
Italian	Pemigatinib	Trattamento delle neoplasie mieloidi o linfoidi con eosinofilia associate a riarrangiamento di PDGFRA, PDGFRB, o FGFR1, o con PCM1-JAK2
Latvian	Pemigatinibs	Mieloīdo / limfoīdo audzēju ar eozinofīliju un PDGFRA, PDGFRB vai FGFR1 pārkārtošanos vai ar PCM1-JAK2 ārstēšana

[1] At the time of designation

Language	Active ingredient	Indication
Lithuanian	Pemigatinibas	Mieloidinių/limfoidinių neoplazijų su eozinofilija ir struktūriniais *PPDGFRA, PDGFRB ir FGFR1*, ir su *PCM1-JAK2* persitvarkymais, gydymas
Maltese	Pemigatinib	Kura ta' neoplażmi ta' mijelojde/limfojde b'eosinofilja u arranġament mill-ġdid tal-PDGFRA, PDGFRB, jew FGFR1, jew b'PCM1-JAK2
Polish	Pemigatynib	Leczenie nowotworów szpiku kostnego lub limfatycznych związanych z eozynofilią oraz rearanżacją PDGFRA, PDGFRB, FGFR1, lub PCM1-JAK2
Portuguese	Pemigatinib	Tratamento de neoplasia mieloide ou linfoide com eosinofilia associada a rearranjo do PDGFRA, PDGFRB, ou FGFR1, ou com PCM1-JAK2
Romanian	Pemigatinib	Tratamentul neoplaziilor mieloide sau limfoide asociate cu eozinofilie şi cu rearanjarea PDGFRA, PDGFRB sau FGFR1, sau cu PCM1-JAK2
Slovak	Pemigatinib	Liečba myeloidnej alebo lymfoidnej neoplázie spojenej s eozinofíliou a preskupením PDGFRA, PDGFRB, alebo FGFR1, alebo s PCM1-JAK2
Slovenian	Pemigatinib	Zdravljenje mieloidne/ limfoidne neoplazme z eozinofilijo in prerazporeditvijo PDGFRA, PDGFRB ali FGFR1 ali s PCM1-JAK2
Spanish	Pemigatinib	Tratamiento de la neoplasia mieloide y linfoide con eosinophilia y asociada a la reordenación PDGFRA, PDGFRB, or FGFR1, o con PCM1-JAK2
Swedish	Pemigatinib	Behandling av myeloida/lymfoida tumörer med eosinofili och rearrangering av PDGFRA, PDGFRB, eller FGFR1 eller med PCM1-JAK2
Norwegian	Pemigatinib	Behandling av myeloide og lymfoide neoplasmer assosiert med eosinofili og rearrangering av PDGFRA/-B eller FGFR1, eller med PCM1-JAK2-fusjon
Icelandic	Pemigatíníb	Meðferð við mergæxli/eitilæxli með rauðkyrningafjölgun og umröðun á PDGFRA, PDGFRB, eða FGFR1, eða með PCM1-JAK2