



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

8 April 2021 Rev. 1¹
EMADOC-628903358-1131

Public summary of opinion on orphan designation

Parsaclisib for the treatment of marginal zone lymphoma

On 25 July 2019, orphan designation EU/3/19/2185 was granted by the European Commission to Incyte Biosciences Distribution B.V., the Netherlands, for parsaclisib for the treatment of marginal zone lymphoma.

What is marginal zone lymphoma?

Marginal zone lymphoma is a cancer of a type of white blood cell called B lymphocytes or B cells. In marginal zone lymphoma, abnormal B cells multiply quickly and live for too long. The abnormal B cells affect various organs. Patients usually have fever, weight loss, tiredness and night sweats.

Marginal zone lymphoma is a life-threatening and long-term debilitating disease due to its effects on the spleen, lymph nodes and bone marrow, as well as the increased risk of infection.

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

At the time of designation, marginal zone lymphoma affected approximately 1.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 78,000 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, the main treatments for marginal zone lymphoma available in the EU included chemotherapy (cancer medicines), immunotherapy (using the body's own immune system to kill cancer cells) with the medicine rituximab, radiotherapy (treatment with radiation) and surgery to remove affected lymph nodes. In some patients, marginal zone lymphoma affecting the stomach is associated with infection by the bacterium *Helicobacter pylori*, and treatment with antibiotics was used to resolve the infection.

¹ Orphan designation number EU/3/19/1285 was corrected: EU/3/19/2185.

*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).



The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with marginal zone lymphoma because early studies indicated a beneficial effect in patients whose disease had come back after or did not respond to previous treatment. 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?

Parsaclisib is expected to work by blocking the effects of an enzyme called PI3K that plays a role in the growth and survival of white blood cells. PI3K is overactive in marginal zone lymphoma cells, where it helps the cancer cells to survive. By blocking the effects of PI3K, parsaclisib is expected to cause death of the cancer cells, thereby delaying or stopping the progression of the cancer.

What is the stage of development of this medicine?

The effects of parsaclisib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with parsaclisib in patients with marginal zone lymphoma were ongoing.

At the time of submission, parsaclisib was not authorised anywhere in the EU for the treatment of marginal zone lymphoma or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 20 June 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 languages², Norwegian and Icelandic

Language	Active ingredient	Indication
English	Parsaclisib	Treatment of marginal zone lymphoma
Bulgarian	Парсаклисиб	Лечение на маргинално зонален лимфом
Croatian	Parsaklisib	Liječenje limfoma marginalne zone
Czech	Parsaclisib	Léčba lymfomu z marginální zóny
Danish	Parsaclisib	Behandling af marginalzonelymfom
Dutch	Parsaclisib	Behandeling van marginale zone lymfoom
Estonian	Parsatsilisiib	Marginaaltsooni lümfoomi ravi
Finnish	Parsaklisibi	Marginaalivyöhykkeen lymfooman hoito
French	Parsaclisib	Traitement du lymphome de la zone marginale
German	Parsaclisib	Behandlung des Marginalzonenlymphoms
Greek	Παρσακλισίμπη	Θεραπεία του λεμφώματος μεθοριακής ζώνης
Hungarian	Parsaclisib	Marginális zóna lymphoma kezelése
Italian	Parsaclisib	Trattamento del linfoma della zona marginale
Latvian	Parsaklizibs	Marginālo zonu limfomas ārstēšana
Lithuanian	Parsaklisibas	Marginalinės zonos limfomos gydymas
Maltese	Parsaklisib	Kura ta' limfoma taż-żona marginali
Polish	Parsaklizyb	Leczenie chłoniaka strefy brzeżnej
Portuguese	Parsaclisib	Tratamento do linfoma da zona marginal
Romanian	Parsaclisib	Tratamentul limfomului de zonă marginală
Slovak	Parsaklisib	Liečba lymfómu z marginálnej zóny
Slovenian	Parsaklisib	Zdravljenje limfoma marginalne cone
Spanish	Parsaclisib	Tratamiento del linfoma de la zona marginal
Swedish	Parsaklisib	Behandling av marginalzonelymfom
Norwegian	Parsaklisib	Behandling av marginalsonelymfom
Icelandic	Parsaklisib	Meðferð við jaðarsvæðiseitlkrabbameini

² At the time of designation