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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Acalabrutinib for the treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma

On 21 March 2016, orphan designation (EU/3/16/1624) was granted by the European Commission to Acerta Pharma, BV, the Netherlands, for acalabrutinib for the treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma.

What is chronic lymphocytic leukaemia / small lymphocytic lymphoma?

Chronic lymphocytic leukaemia (CLL) is cancer of a type of white blood cell called B lymphocytes or B cells. In this disease, the B cells multiply too quickly and live for too long, so that there are too many of them circulating in the blood. The cancerous B cells look normal, but they are not fully developed and do not work properly. Over time, the cancer cells replace the normal white cells, red cells and platelets in the bone marrow (the spongy tissue inside the large bones in the body, where blood cells are produced).

The disease known as 'small lymphocytic lymphoma' (SLL) is the same disease as CLL. The name SLL is used when the cancer cells are located mainly in the lymph nodes.

CLL/SLL is the most common type of leukaemia and mainly affects older people. It is rare in people under the age of 40 years. CLL/SLL is a long-term debilitating and life-threatening disease because some patients develop severe infections.

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

At the time of designation, CLL/SLL affected approximately 4.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 231,000 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 513,700,000 (Eurostat 2016).

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What treatments are available?

Treatment for CLL/SLL is complex and depends on a number of factors, including the extent of the disease, whether it has been treated before, and the patient's age, symptoms and general state of health. Patients whose CLL/SLL is not causing any symptoms or is getting worse only very slowly may not need treatment. Treatment for CLL/SLL is started only if symptoms become troublesome. At the time of designation, the main treatment for CLL/SLL was chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that acalabrutinib might be of significant benefit for patients with CLL/SLL because early studies showed that this medicine improved survival. 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?

Acalabrutinib is expected to work by blocking an enzyme called Bruton's tyrosine kinase (Btk), which is found in B cells. Btk promotes growth and survival of B cells. By blocking Btk, acalabrutinib is expected to slow down the build-up of cancerous B cells in CLL/SLL, thereby delaying or stopping the progression of the disease.

The medicine is expected to be taken by mouth.

What is the stage of development of this medicine?

The effects of acalabrutinib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with acalabrutinib in patients with CLL/SLL were ongoing.

At the time of submission, acalabrutinib was not authorised anywhere in the EU for CLL/SLL. Orphan designation of acalabrutinib had been granted in the United States for chronic lymphocytic leukaemia.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 February 2016 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	Acalabrutinib	Treatment of chronic lymphocytic leukaemia / small lymphocytic lymphoma
Bulgarian	Акалабрутиниб	Лечение на хронична лимфоцитна левкемия / Дребноклетъчен лимфоцитен лимфом
Croatian	Akalabrutinib	Liječenje kronične limfocitne leukemije / limfoma malih limfocita
Czech	Acalabrutinib	Léčba chronické lymfatické leukémie / lymfom z malých lymfocytů
Danish	Acalabrutinib	Behandling af kronisk lymfatisk leukæmi / småcellet lymfocytisk lymfom
Dutch	Acalabrutinib	Behandeling van chronische lymfocytaire leukemie / kleincellig lymfocytair lymfoom
Estonian	Akalabrutiniib	Kroonilise lümfoidleukeemia ja väikerakk-lümfotsüütlümfoomi ravi
Finnish	Akalabrutinibi	Kroonisen lymfaattisen leukemian ja pienilymfosyyttisen lymfooman hoito
French	Acalabrutinib	Traitement de la leucémie lymphoïde chronique / du lymphome lymphocytaire à petites cellules
German	Acalabrutinib	Behandlung der chronischen lymphatischen Leukämie / des kleinzelligen lymphozytischen Lymphoms
Greek	Ακαλαβρουτινίμπη	Θεραπεία της χρόνιας λεμφοκυτταρικής λευχαιμίας / του Λεμφώματος από Μικρά Λεμφοκύτταρα
Hungarian	Akalabrutinib	Krónikus lymphoid leukaemia / kissejtes lymphocytás lymphoma kezelése
Italian	Acalabrutinib	Trattamento della leucemia linfocitica cronica / linfoma a piccoli linfociti
Latvian	Akalabrutinibs	Hroniskas limfoleikozes / mazo limfocītu limfomas ārstēšana
Lithuanian	Akalabrutinibas	Lėtinės limfocitinės leukemijos / smulkių limfocitų limfomos gydymas
Maltese	Acalabrutinib	Kura tal-lewkimja limfoċitika kronika / limfoma limfoċitika żgħira
Polish	Akalabrutynib	Leczenie przewlekłej białaczki limfatycznej / chłoniaka z małych limfocytów
Portuguese	Acalabrutinib	Tratamento da leucemia linfocítica crónica/linfoma de pequenos linfocitos
Romanian	Acalabrutinib	Tratamentul leucemiei limfoide cronice / limfomului limfocitar cu celule mici
Slovak	Akalabrutinib	Liečba chronickej lymfocytovej leukémie / malobunkového lymfocytového lymfómu
Slovenian	Akalabrutinib	Zdravljenje kronične limfatične levkemije / drobnoceličnega limfocitnega limfoma
Spanish	Acalabrutinib	Tratamiento de la leucemia linfocítica crónica / linfoma linfocítico pequeño

[1] At the time of designation

Language	Active ingredient	Indication
Swedish	Acalabrutinib	Behandling av kronisk lymfatisk leukemi / småcelligt lymfocytärt lymfom
Norwegian	Acalabrutinib	Behandling av kronisk lymfatisk leukemi / småcellet lymfocytært lymfom
Icelandic	Acalabrútíníb	Meðferð á langvinnu eitilfrumuhvítblæði / smáeitilfrumu eitlakrabbameini