



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/COMP/121542/2005 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Acadesine for the treatment B-cell chronic lymphocytic leukemia

First publication	29 June 2005
Rev.1: sponsor's name and address change	11 August 2009
Rev.2: sponsor's change of address	22 July 2013
Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 27 May 2005, orphan designation (EU/3/05/280) was granted by the European Commission to Advanced In vitro Cell technologies S.L., Spain, for acadesine for the treatment of B-cell chronic lymphocytic leukemia.

In September 2008, Advanced In vitro Cell technologies S.L. changed name to Advancell - Advanced In Vitro Cell Technologies S.A.

What is B-cell chronic lymphocytic leukemia?

B-cell chronic lymphocytic leukemia is a disease in which cancer cells are found in the blood and the bone marrow. The bone marrow is the spongy tissue inside the large bones in the body. Normally, the bone marrow makes cells called "blasts" that mature into several different types of blood cells that have specific functions in the body. These include red cells, white cells and platelets. Red blood cells carry oxygen and other materials to all tissues of the body. White blood cells fight infection. Platelets support blood clotting. When leukemia develops, the bone marrow produces large numbers of abnormal blood cells. There are several types of leukemias. B-cell chronic lymphocytic leukaemia is a cancer of a type of white blood cells called B-lymphocytes. The lymphocytes multiply too quickly and live too long, so there are too many of them circulating in the blood. These leukemic lymphocytes look normal, but they are not fully developed and do not work properly. Over a period of time these abnormal cells replace the normal white cells, red cells and platelets in the bone marrow.. B-cell chronic lymphocytic leukemia is the most common type of leukaemia and mainly affects older people and is rare in people under the age of 40. B-cell chronic lymphocytic leukemia is chronically debilitating and life-threatening due to the severe prognosis and the poor long-term survival for high-risk patients.



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

At the time of designation, B-cell chronic lymphocytic leukemia affected approximately 2.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 126,000 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?

Treatment for leukemia is complex and depends on a number of factors including the type of leukemia, the extent of the disease and whether the leukemia has been treated before. It also depends on the age, the symptoms, and the general health of the patient. Some people with B-cell chronic lymphocytic leukemia never have treatment if their illness is not causing any symptoms and is progressing only very slowly. Treatment is only started if and when the symptoms become troublesome. Current main treatment of B-cell chronic lymphocytic leukemia is chemotherapy (using drugs to kill cancer cells). Several products were authorised for the condition in the Community at the time of submission of the application for orphan drug designation.

Acadesine could be of potential significant benefit for the treatment of B-cell chronic lymphocytic leukemia because it might act differently from other medicinal products. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

The so-called programmed cell death (apoptosis) is a process that is very important for the natural death of cells. In B-cell chronic lymphocytic leukemia the cells live longer than normal. It is not completely understood how acadesine works in the human body but it might help to specifically destroy the cancer cells through activation of some specific reactions inside the cancer cell itself, which trigger the apoptosis.

What is the stage of development of this medicine?

The evaluation of the effects of acadesine in experimental models is ongoing.

At the time of submission of the application for orphan designation, no clinical trials in patients with B-cell chronic lymphocytic leukemia were initiated.

Acadesine was not marketed anywhere worldwide for B-cell chronic lymphocytic leukemia or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 April 2005 recommending the granting of this designation.

*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 25), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 466,600,000 (Eurostat 2005).

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:

Advancell - Advanced In Vitro Cell Technologies S.A.
Via Augusta 59
3rd floor, door 313
08006 Barcelona
Spain
Telephone: +34 930 130 561
Telefax: +34 932 380 766

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	Acadesine	Treatment of B-cell chronic lymphocytic leukemia (B-CLL)
Czech	Acadesine	Léčba chronické lymfatické leukémie
Danish	Acadesin	Behandling af B-celle kronisk lymfocytær leukæmi
Dutch	Acadesine	Behandeling van B-cel chronische lymfocyttaire leukemie (B-CLL)
Estonian	Akadesiin	B-rakulise kroonilise lümfoïdleukeemia ravi
Finnish	Akadesiini	Kroonisen B-solu (B-CLL) lymfosyyttileukemian hoito
French	Acadésine	Traitement de la leucémie lymphoïde chronique à cellules B
German	Acadesin	Behandlung der chronisch-lymphatischen B-Zell Leukämie (B-CLL)
Greek	Ακαδεσίνη	Θεραπεία της χρόνιας λεμφοκυτταρικής λευχαιμίας Β-λεμφοκυττάρων (B-CLL)
Hungarian	Acadesin	Krónikus B-sejtes lymphoid leukémia kezelése
Italian	Acadesina	Trattamento della leucemia linfocitica cronica a cellule B
Latvian	Akadezīns	Hroniskas B šūnu limfocitiskās leikēmijas ārstēšana
Lithuanian	Akadezinas	Lėtinės B-ląstelių limfocitinės leukemijos gydymas
Polish	Acadezyna	Leczenie przewlekłej białaczki limfatycznej
Portuguese	Acadesina	Tratamento da leucemia linfocítica crónica das células B (LLC-B)
Slovak	Acadesin	Liečba chronickej B-bunkovej lymfocytovej leukémie
Slovenian	Akadazin	Zdravljenje kronične limfocitne
Spanish	Acadesina	Tratamiento de la leucemia linfocítica crónica de células B
Swedish	Akadesin	Behandling av B-cell kronisk lymfatisk leukemi.
Norwegian	Akadesin	Behandling av kronisk lymfatisk leukemi
Icelandic	Akadesín	Meðferð á langvinnu eitilfrumuhvítblæði (B-CLL)

¹ At the time of designation