16 June 2011
EMA/COMP/449452/2008 Rev.3
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Vincristine sulphate liposomes for the treatment of acute lymphoblastic leukaemia

On 8 July 2008, orphan designation (EU/3/08/555) was granted by the European Commission to QuadraMed Limited, United Kingdom, for vincristine sulphate liposomes for the treatment of acute lymphoblastic leukaemia.

The sponsorship was transferred to Fulcrum Pharma (Europe) Limited, United Kingdom, in April 2009 and subsequently to NDA Regulatory Science Ltd, United Kingdom, in May 2011.

What is acute lymphoblastic leukaemia?

Acute lymphoblastic leukaemia (ALL) is a cancer of the white blood cells called lymphocytes. In this disease, the lymphocytes multiply too quickly and live for too long, so there are too many of them circulating in the blood. These leukaemic 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 and red blood cells and platelets in the bone marrow (the spongy tissue inside the large bones in the body that produces blood cells). ALL is the most common type of leukaemia in young children but the disease also affects adults, especially those aged 65 and older. Many people with acute leukaemia can be cured. However, despite the available treatments, ALL remains a serious and life-threatening condition in some patients.

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

At the time of designation, ALL affected approximately 0.5 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 25,000 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 27), Norway, Iceland and Liechtenstein. This represents a population of 506,300,000 (Eurostat 2011).

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What treatments are available?

Treatment for ALL 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. The main treatment is chemotherapy (medicines used to kill cancer cells) followed by or combined with radiotherapy (using radiation to kill cancer cells). Bone marrow transplantation is also used.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that vincristine sulphate liposomes might be of potential significant benefit for the treatment of ALL because it has a new delivery method. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain orphan status.

How is this medicine expected to work?

Vincristine is a medicine that is already used widely as chemotherapy for the treatment of blood cancers including ALL. It is a 'vinca alkaloid' (a substance obtained from the *Vinca* periwinkle flower) that blocks the cell's ability to break down the 'skeleton' that allows cells to divide and multiply. With the skeleton still in place the cells cannot divide, and they eventually die. In this medicine, vincristine is contained in liposomes (tiny fatty particles). This is expected to improve the way the medicine works compared with the conventional form of the medicine, by slowing down the clearance of the medicine from the body.

What is the stage of development of this medicine?

The effects of vincristine sulphate liposomes have been evaluated in experimental models.

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

Vincristine sulphate liposomes were not authorised anywhere worldwide for ALL at the time of submission but orphan designation of vincristine sulphate liposomes has been granted in the United States of America for the treatment of ALL.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 14 May 2008 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:

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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	Vincristine sulphate liposomes	Treatment of acute lymphoblastic leukaemia
Bulgarian	Липозоми с винкристин сулфат	Лечение на остра лимфобластна левкемия
Czech	Liposomální vinkristin sulfát	Léčba akutní lymfoblastické leukémie
Danish	Vincristinsulfat, liposomer	Behandling af akut lymfoblastær leukæmi
Dutch	Vincristinesulfaat liposomen	Behandeling van acute lymfoblastaire leukemie
Estonian	Vinkristiinsulfaadi liposoomid	Ägeda lümfoblastilise leukeemia ravi
Finnish	Vinkristiinisulfaattiliposomi	Akuutin lymfoblastileukemian hoito
French	Liposomes de sulfate de vincristine	Traitement de la leucémie lymphoblastique aiguë
German	Vincristinsulfat Liposomen	Behandlung der akuten lymphatischen Leukämie
Greek	Λιποσώματα Θειικής βινκριστίνης	Θεραπεία της οξείας λεμφοβλαστικής λευχαιμίας
Hungarian	Vinkrisztin-szulfát liposzóma	Akut lymphoblastos leukaemia kezelése
Italian	Vincristina solfato liposomiale	Trattamento della leucemia linfoblastica acuta
Latvian	Vinkristīna sulfāta liposomas	Akūtas limfoblastiskas leikozes ārstēšana
Lithuanian	Vinkristino sulfato liposomos	Ūmios limfoblastinės leukemijos gydymas
Maltese	Liposomi tal-vincristine sulphate	Kura tal-lewkimja limfoblastika akuta
Polish	Siarczan winkrystyny w liposomach	Leczenie ostrej białaczki limfoblastycznej
Portuguese	Lipossomas de Sulfato de Vincristina	Tratamento da leucémia linfoblástica aguda
Romanian	Sulfat de vincristină inclus în lipozomi	Tratamentul leucemiei limfoblastice acute
Slovak	Lipozómy s vinkristíniumsulfátom	Liečba akútnej lymfoblastickej leukémie
Slovenian	Liposomi z vinkristin sulfatom	Zdravljenje akutne limfoblastne levkemije
Spanish	Sulfato de vincristina liposomal	Tratamiento de la leucemia linfoblástica aguda
Swedish	Vinkristinsulfatliposomer	Behandling av akut lymfatisk leukemi
Norwegian	Vinkristinsulfatliposomer	Behandling av akutt lymfoblastisk leukemi
Icelandic	Vínkristínsúlfat fitukorn	Meðferð við bráðu eitlifrumuhvítblæði

[1] At the time of designation