



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pixantrone dimaleate for the treatment of diffuse large B-cell lymphoma

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in December 2010 on request of the sponsor.

On 2 February 2010, orphan designation (EU/3/09/722) was granted by the European Commission to CTI Life Sciences Ltd, United Kingdom, for pixantrone dimaleate for the treatment of diffuse large B-cell lymphoma.

What is diffuse large B-cell lymphoma?

Diffuse large B-cell lymphoma is the most common cancer of the lymphatic system, a network of vessels that transport lymph from tissues through the lymph nodes and into the bloodstream. Diffuse large B-cell lymphoma affects a type of white blood cell called B lymphocytes, or B cells. In diffuse large B-cell lymphoma, the B cells multiply too quickly and live for too long, so there are too many of them in the lymph nodes. The first sign of the disease is usually a lump in the neck, under the arm or in the groin area, which is caused by an enlarged lymph node. Patients with diffuse large B-cell lymphoma may also have fever, tiredness, night sweats or weight loss that have no obvious cause.

Although some people with diffuse large B-cell lymphoma can be cured, it remains a serious and life-threatening disease, particularly when the disease is found late or has come back after initial treatment.

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

At the time of designation, diffuse large B-cell lymphoma affected approximately 2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 101,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and 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 504,800,000 (Eurostat 2009).



What treatments are available?

At the time of designation, several medicines were authorised for the treatment of diffuse large B-cell lymphoma in the EU. The main treatment was chemotherapy (medicines to treat cancer), sometimes in combination with radiotherapy (treatment with radiation). Bone marrow transplantation was also used in patients at risk of the disease coming back after treatment. This is a complex procedure where the bone marrow of the patient is destroyed and replaced with healthy bone marrow from the same patient or a matched donor.

The sponsor has provided sufficient information to show that pixantrone dimaleate might be of significant benefit for patients with diffuse large B-cell lymphoma because early studies show that it might improve the treatment of patients with this condition, particularly patients whose disease has come back after 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?

Pixantrone dimaleate is a cytotoxic (a medicine that kills cells that are dividing rapidly, such as cancer cells). It is expected to work by interfering with the DNA, preventing the cells from making more copies of their DNA and from making proteins. This means that cancer cells cannot divide or multiply, slowing down the progression of the disease.

What is the stage of development of this medicine?

The effects of pixantrone dimaleate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with pixantrone dimaleate in patients with diffuse large B-cell lymphoma were ongoing.

At the time of submission, pixantrone dimaleate was not authorised anywhere in the EU for the treatment of diffuse large B-cell lymphoma or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 3 December 2009 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 Community) 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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Patient associations' contact points

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	Pixantrone dimaleate	Treatment of diffuse large B-cell lymphoma
Bulgarian	Пиксантрон дималеат	Лечение на дифузен В-едроклетъчен лимфом
Czech	Pixantron dimaleát	Léčba velkobuněčného difuzního B-lymfomu
Danish	Pixantron dimaleat	Behandling af diffust storcellet B-celle lymfom
Dutch	Pixantrone dimaleate	Behandeling van diffuus grootcellig B-cel-lymfoom
Estonian	Piksantrone dimaleaat	Diffuusse suure β -rakulise lümfoomi ravi
Finnish	Piksantronidimaleaatti	Diffuusin suurisoluisen B-solulymfooman hoito
French	Dimaléate de pixantrone	Traitement du lymphome diffus à grandes cellules B
German	Pixantron-Dimaleat	Behandlung des diffusen großzelligen B-Zell-Lymphoms
Greek	Διμαλεϊκή Πιξαντρόνη	Θεραπεία του διάχυτου μεγαλοκυτταρικού λεμφώματος B-κυττάρου (DLBCL)
Hungarian	Pixantron dimaleát	Diffúz nagy B-sejtes lymphoma kezelése
Italian	Pixantrone dimaleato	Terapia del linfoma non-Hodgkin diffuso a grandi cellule di tipo B (DLBCL)
Latvian	Piksantrona dimaleāts	Difūzas lielo B šūnu limfomas ārstēšana
Lithuanian	Piksantrono dimaleatas	Difuzinės stambiujų B ląstelių limfomos gydymas
Maltese	Pixantrone dimaleate	Kura tal-linfoma taċ-ċelluli tat-tip B kbar mxerrda
Polish	Dimaleinian piksantronu	Leczenie rozlanego chłoniaka z dużych limfocytów B
Portuguese	Dimaleato de pixantrona	Tratamento do linfoma difuso de grandes células B
Romanian	Dimaleat de pixantronă	Tratamentul limfomului difuz cu celule B mari
Slovak	Pixantrón dimaleát	Liečba difúzneho veľkobunkového lymfómu z B-buniek
Slovenian	Piksantron dimaleat	Zdravljenje razširjenega limfoma velikih B celic
Spanish	Dimaleato de pixantrona	Tratamiento del linfoma difuso de células B grandes
Swedish	Pixantron-dimaleat	Behandling av diffusa storcelliga B-cells lymfom
Norwegian	Piksantrondimaleat	Behandling av diffust storcellet B-celle lymfom
Icelandic	Pixantrón-dímaleat	Til meðferðar á dreifðu stórfrumu B frumu eitlakraðbameini

¹ At the time of designation