



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of daunorubicin (liposomal) for the treatment of acute myeloid leukaemia

On 3 December 2008, orphan designation (EU/3/08/585) was granted by the European Commission to Diatos S.A, France, for daunorubicin (liposomal) for the treatment of acute myeloid leukaemia.

What is acute myeloid leukaemia?

Acute myeloid leukaemia (AML) is a cancer of the white blood cells (cells that fight infections). In patients with AML, the bone marrow produces large numbers of abnormal, immature white blood cells called 'blasts'. These abnormal cells accumulate in the bone marrow and are found in the blood. AML is a life-threatening disease because these immature cells take the place of the normal white blood cells, reducing the patient's ability to fight infections.

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

At the time of designation, AML affected less than 2 in 10,000 people in the European Union (EU)*. This is below the threshold for orphan designation which is 5 in 10,000, and is equivalent to a total of fewer than 100,000 people. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

Treatment for AML 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 primary treatment of AML is chemotherapy (medicines to treat cancer). At the time of designation, several medicines were authorised in the EU to treat this condition.

The sponsor has provided sufficient information to show that daunorubicin (liposomal) might be of significant benefit for patients with AML because early studies indicate that it might improve treatment of patients with this condition. 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?

Daunorubicin (liposomal) consists of an existing anticancer medicine called daunorubicin, which is contained in small fatty particles called 'liposomes'. Daunorubicin belongs to the group 'anthracyclines'. It works by interfering with the DNA within cells, preventing them from making more copies of DNA and making proteins. This means that cancer cells cannot divide and eventually die.

*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 502,282,000 (Eurostat 2008).

Daunorubicin has been used for several years in the treatment of leukaemia in many European countries. When it is contained within liposomes, it is expected to remain in the patient's body for longer than 'free' daunorubicin.

What is the stage of development of this medicine?

The effects of daunorubicin (liposomal) have been evaluated in experimental models.

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

At the time of submission, daunorubicin (liposomal) had not been authorised anywhere in the EU for AML or designated as 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 8 October 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 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 marketing authorisation.

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**Translations of the active ingredient and indication in all official EU languages,
Norwegian and Icelandic**

| Language | Active Ingredient | Indication |
|-----------------|-------------------------------------|--|
| English | Daunorubicin (liposomal) | Treatment of acute myeloid leukaemia |
| Bulgarian | Даунорубицин, (липозомален) | Лечение на остра миелоидна левкемия |
| Czech | Daunorubicin (liposomální) | Léčba akutní myeloidní leukémie |
| Danish | Daunorubicin (liposomal) | Behandling af akut myeloid leukæmi |
| Dutch | Daunorubicine (liposomaal) | Behandeling van acute myeloïde leukemie |
| Estonian | Daunorubitsiin (liposomaalne) | Akuutse müeloidse leukeemia ravi |
| Finnish | Daunorubisiini (liposomaalinen) | Akuutin myelooisen leukemian hoito |
| French | Daunorubicine (liposomale) | Traitement de la leucémie aiguë myéloïde |
| German | Daunorubicin (liposomal) | Behandlung der akuten myeloischen Leukämie |
| Greek | Δαουνορουβικίνη, (Λιποσωμική) | Θεραπεία της οξείας μυελοειδούς λευχαιμίας |
| Hungarian | Daunorubicin (liposzómban) | Akut myeloid leukaemia kezelése |
| Italian | Daunorubicina (liposomiale) | Trattamento della leucemia mieloide acuta |
| Latvian | Daunorubicīns (liposomu) | Akūtas mieloleikozes ārstēšana |
| Lithuanian | Daunorubicinas (liposominis) | Ūmios mieloleukozės gydymas |
| Maltese | Daunorubicin (liposomal) | Kura tal-lewkimja mjeloidja akuta |
| Polish | Daunorubicyna (liposomalna) | Leczenie ostrej białaczki szpikowej |
| Portuguese | Daunorubicina (liposomal) | Tratamento da leucémia mieloide aguda |
| Romanian | Daunorubicină (inclusă în lipozomi) | Tratamentul leucemiei mieloide acute |
| Slovak | Daunorubicín (lipozomálny) | Liečba akútnej myeloickej leukémie |
| Slovenian | Daunorubicin (liposomski) | Zdravljenje akutne mieloične levkemije |
| Spanish | Daunorubicina (liposomal) | Tratamiento de la leucemia mieloide aguda |
| Swedish | Daunorubicin (liposomal) | Behandling av akut myeloid leukemi |
| Norwegian | Daunorubicin (liposomal) | Behandling av akutt myelogen leukemi |
| Icelandic | Daunórubicín (í lípósómum) | Meðferð við bráðu kyrningahvítblæði |