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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

Liposomal combination of cytarabine and daunorubicin for the treatment of acute myeloid leukaemia

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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 11 January 2012, orphan designation (EU/3/11/942) was granted by the European Commission to Celator (UK) Ltd, United Kingdom, for liposomal combination of cytarabine and daunorubicin 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 against infections). In patients with AML, the bone marrow (the spongy tissue inside the large bones, where blood cells are produced) produces large numbers of abnormal, immature white blood cells. These abnormal cells quickly build up in large numbers in the bone marrow and are found in the blood.

AML is a long-term debilitating and life-threatening disease because these abnormal 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 not more than 1.2 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 61,000 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. At the time of designation, this represented a population of 509,000,000 (Eurostat 2012).



## **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. At the time of designation, the main treatments for AML were chemotherapy (medicines to treat cancer) and haematopoietic (blood) stem-cell transplantation (a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow).

The sponsor has provided sufficient information to show that liposomal combination of cytarabine and daunorubicin might be of significant benefit for patients with AML because early studies have shown that the medicine may improve the 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?**

This medicine is a combination of two existing anticancer medicines, cytarabine and daunorubicin, which have been used in combination to treat AML and other types of cancer for several years. Cytarabine interferes with the enzymes involved in the production of new cell DNA, slowing down the growth of cancer cells and eventually killing them. Daunorubicin interferes with the DNA within cells, preventing them from making more copies of DNA and making proteins, which means they are unable to divide and they eventually die.

In this medicine, cytarabine and daunorubicin are contained in microscopic fat particles called 'liposomes'. The liposomes are expected to remain in the patient's body for longer than 'free' cytarabine and daunorubicin and accumulate in the patient's bone marrow. The liposomes protect the anticancer medicines from being broken down early, which is expected to enhance their effect on cancer cells.

## **What is the stage of development of this medicine?**

The effects of liposomal combination of cytarabine and daunorubicin have been evaluated in experimental models.

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

At the time of submission, this medicine was not authorised anywhere in the EU for AML. Orphan designation of the medicine had been granted in the United States of America for the treatment of AML.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 November 2011 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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Liposomal combination of cytarabine and daunorubicin	Treatment of acute myeloid leukaemia
Bulgarian	Липозомна комбинация от цитарабин и даунорубицин	Лечение на остра миелоидна левкемия
Czech	Liposomální kombinace cytarabinu a daunorubicinu	Léčba akutní myeloidní leukémie
Danish	Liposomal kombination af cytarabin og daunorubicin	Behandling af akut myeloid leukæmi
Dutch	Liposomale combinatie van cytarabine en daunorubicine	Behandeling van acute myeloïde leukemie
Estonian	Tsütarabiini ja daunorubitsiini liposomaalne kombinatsioon	Akuutse müeloidse leukeemia ravi
Finnish	Liposomivalmiste, jossa on sytarabiinia ja daunorubisiinia	Akuutin myelooisen leukemian hoito
French	Combinaison liposomale de cytarabine et daunorubicine	Traitement de la leucémie aiguë myéloïde
German	Liposomale Kombination von Cytarabin und daunorubicin	Behandlung der akuten myeloischen Leukämie
Greek	Λιποσωμικός συνδυασμός κυταραβίνης και νταουνορουμπικίνης	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	A citarabin és a daunorubicin liposomális kombinációja	Akut myeloid leukaemia kezelése
Italian	combinazione liposomale di citarabina e daunorubicina	Trattamento della leucemia mieloide acuta
Latvian	Citarabīna un daunorubicīna liposomālā kombinācija	Akūtas mieloleikozes ārstēšana
Lithuanian	Liposominis citarabino ir daunorubicino derinys	Ūmios mieloleukozės gydymas
Maltese	Kombinazzjoni liposomal ta' cytarabine u daunorubicin	Kura tal-lewkimja mjelojda akuta
Polish	Liposomalne połączenie cytarabiny z daunorubicyną	Leczenie ostrej białaczki szpikowej
Portuguese	Combinação liposomal de Citarabina e daunorubicina	Tratamento da leucémia mieloide aguda
Romanian	Combinățile lipozomală de citarabină și daunorubicină	Tratamentul leucemiei mieloide acute
Slovak	Lipozómová kombinácia cytarabínu a daunorubicínu	Liečba akútnej myeloickej leukémie
Slovenian	Liposomska kombinacija citarabina in daunorubicina	Zdravljenje akutne mieloične levkemije

<sup>1</sup> At the time of designation

Language	Active ingredient	Indication
Spanish	Combinación liposomal de citarabina y daunorrubicina	Tratamiento de la leucemia mieloide aguda
Swedish	Liposomal kombination av cytarabine och daunorubicin	Behandling av akut myeloisk leukemi
Norwegian	Liposomal kombinasjon eller cytarabine og daunorubicin	<b>Behandling av akutt myelogen leukemi</b>
Iceland	Blanda af cýtarabíni og daunórúbícíni í lípósómum	Meðferð við bráðu kyrningahvítblæði