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EMA/COMP/615285/2012
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Liposomal daunorubicin for the treatment of acute myeloid leukaemia

On 10 October 2012, orphan designation (EU/3/12/1056) was granted by the European Commission to Galen Limited, United Kingdom, for liposomal 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 is 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).

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).

[*]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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The sponsor has provided sufficient information to show that liposomal daunorubicin might be of significant benefit for patients with AML because it is a new formulation of daunorubicin (an anticancer medicine that has been used to treat AML and other types of cancer for several years) which may help daunorubicin to reach the cancer cells more selectively, thereby improving the outcome 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 contains the anticancer medicine daunorubicin. 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, daunorubicin is contained in microscopic fat particles called 'liposomes'. The liposomes are expected to keep the daunorubicin in the patient's body for longer than 'free' daunorubicin and to accumulate in the patient's bone marrow. The liposomes protect the anticancer medicine from being broken down early and enhance its effect on cancer cells.

What is the stage of development of this medicine?

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

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

At the time of submission, liposomal daunorubicin was not authorised anywhere in the EU for AML. Orphan designation of liposomal daunorubicin had been granted previously in the EU and in the United States of America for AML.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 September 2012 recommending the granting of this designation.

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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	Liposomal daunorubicin	Treatment of acute myeloid leukaemia
Bulgarian	Даунорубицин, (липозомален)	Лечение на остра миелоидна левкемия
Czech	Daunorubicin (liposomální)	Léčba akutní myeloidní leukémie
Danish	Liposomal daunorubicin	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	Νταουνορουβικίνη, (Λιποσωμική)	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Daunorubicin (liposzómában)	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 (liposomali)	Kura tal-lewkimja mjelojda akuta
Polish	Daunorubicyna (liposomalna)	Leczenie ostrej białaczki szpikowej
Portuguese	Daunorrubicina (liposomal)	Tratamento da leucémia mielóide aguda
Romanian	Daunorubicină (inclusă în lipozomi)	Tratamentul leucemiei mieloide acute
Slovak	Lipozomálny daunorubicín	Liečba akútnej myeloickej leukémie
Slovenian	Daunorubicin (liposomski)	Zdravljenje akutne mieloične levkemije
Spanish	Daunorrubicina (liposomal)	Tratamiento de la leucemia mieloide aguda
Swedish	Daunorubicin (liposomal)	Behandling av akut myeloisk leukemi
Norwegian	Daunorubicin (liposomalt)	Behandling av akutt myelogen leukemi
Icelandic	Daunórúbicín (í lípósómum)	Meðferð við bráðu kyrningahvítblæði

[1] At the time of designation