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EMA/COMP/433087/2015
Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Doxorubicin for the treatment of hepatoblastoma

On 28 July 2015, orphan designation (EU/3/15/1513) was granted by the European Commission to Double Bond Pharmaceutical AB, Sweden, for doxorubicin for the treatment of hepatoblastoma.

### What is hepatoblastoma?

Hepatoblastoma is a cancer of the liver that usually affects young children. Children often present with a mass in the abdomen, though there may also be generalised symptoms. In advanced stages, the cancer spreads to other parts of the body, particularly the lungs.

Hepatoblastoma is a seriously debilitating and life-threatening disease that is associated with reduced life expectancy.

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

At the time of designation, hepatoblastoma affected less than 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 5,000 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?

At the time of designation, there were no satisfactory treatments in the EU for hepatoblastoma. Standard treatment was surgery to remove the abdominal mass, with chemotherapy sometimes given before or after surgery.

### How is this medicine expected to work?

Doxorubicin is an anti-cancer agent belonging to the class 'anthracyclines'. It is thought to work 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 they 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 28), Norway, Iceland and Liechtenstein. This represents a population of 512,900,000 (Eurostat 2015).

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Doxorubicin is commonly used in the treatment of several types of cancers, including breast, ovarian, bladder, thyroid and blood cancers. In this medicine, doxorubicin is to be combined with a substance known as a surfactant, which is expected to cause the doxorubicin to accumulate in the liver where it is most needed.

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

The effects of this doxorubicin medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with hepatoblastoma had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for hepatoblastoma 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 18 June 2015 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 | Doxorubicin | Treatment of hepatoblastoma |
| Bulgarian | Доксорубицин | Лечение на хепатобластом |
| Croatian | Doksorubicin | Liječenje hepatoblastoma |
| Czech | Doxorubicin | Léčba hepatoblastomu |
| Danish | Doxorubicin | Behandling af hepatoblastom |
| Dutch | Doxorubicine | Behandeling van hepatoblastoom |
| Estonian | Doksorubitsiin | Hepatoblastoomi ravi |
| Finnish | Doksorubisiini | Hepatoblastooman hoito |
| French | Doxorubicine | Traitement de l'hépatoblastome |
| German | Doxorubicin | Die Behandlung des Hepatoblastoms |
| Greek | Δοξορουβικίνη | Θεραπεία ηπατοβλαστώματος |
| Hungarian | Doxorubicin | Hepatoblastoma kezelésére |
| Italian | Doxorubicina | Trattamento dell' epatoblastoma |
| Latvian | Doksorubicīns | Hepatoblastomas ārstēšana |
| Lithuanian | Doksorubicinas | Hepatoblastomos gydymas |
| Maltese | Doxorubicin | Kura tal-epatoblastoma |
| Polish | Doksorubicyny | Leczenie hepatoblastomy |
| Portuguese | Doxorrubicina | Tratamento de hepatoblastoma |
| Romanian | Doxorubicină | Tratamentul hepatoblastomului |
| Slovak | Doxorubicín | Liečba hepatoblastómu |
| Slovenian | Doksorubicin | Zdravljenje hepatoblastoma |
| Spanish | Doxorubicina | Tratamiento del hepatoblastoma |
| Swedish | Doxorubicin | Behandling av hepatoblastom |
| Norwegian | Doksorubicin | Behandling av hepatoblastom |
| Icelandic | Doxórúbisín | Meðferð hepatoblastoma |

[1] At the time of designation