17 March 2014
EMA/COMP/473073/2007 Rev.2
Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Picoplatin for the treatment of small-cell lung cancer

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| First publication | 1 July 2008 |
| Rev.1: sponsor's change of address | 6 April 2011 |
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| Disclaimer<br>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. | |

***Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in March 2014 on request of the Sponsor.***

On 6 December 2007, orphan designation (EU/3/07/502) was granted by the European Commission to Kendle International, UK, for picoplatin for the treatment of small-cell lung cancer.

### What is small-cell lung cancer?

Small-cell lung cancer is a disease in which cancer (malignant) cells develop in the lungs. This type of lung cancer accounts only for about 20% of all lung cancer cases. Small-cell cancer usually develops in the central part of the lungs, and the cancer cells are typically small compared with the other types of lung cancer. Small-cell cancer is difficult to detect in early stages of the disease, and the majority of the patients are diagnosed when the disease has spread and cannot be surgically removed. Small-cell lung cancer is a life-threatening disease.

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

At the time of designation, small-cell lung cancer affected less than 1.5 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 75,000 people*, and is below the

*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 500,300,000 (Eurostat 2007).

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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?

The main treatment of small-cell lung cancer consists of chemotherapy (i.e drugs that kill cancer cells) and radiotherapy (i.e using high-dose x-rays or other high-energy rays to kill cancer cells).

Several medicinal products were authorised in the European Community, for the treatment of small-cell lung cancer, at the time of submission of the application for orphan designation. Picoplatin might be of potential significant benefit for the treatment of small-cell lung cancer, because it might improve the long-term outcome. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

## How is this medicine expected to work?

Platinum-containing products are already authorized for the treatment of small-cell lung cancer. Picoplatin is a platinum-derivative that is able to bind to the genetic material of cells (DNA) and to interfere with its normal function, leading to lack of DNA replication, absence of protein production from DNA and, ultimately, cell death. Picoplatin has been designed to overcome two of the common problems observed during treatment with other platinum compounds; namely, the onset of resistance to drug treatment (tumour growth continues or re-starts) and the triggering of important adverse reactions. These characteristics of picoplatin may be of potential significant benefit over the existing authorized medicinal products. This assumption will have to be confirmed at the time of marketing authorisation, and this will be necessary to maintain the orphan status.

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

The effects of picoplatin were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with small-cell lung cancer were ongoing.

Picoplatin was not authorised anywhere in the world for the treatment of small-cell lung cancer, at the time of submission. Orphan designation was granted in the United States on 2 November 2005 to cis-Amminedichloro(2-methylpyridine)platinum(II) (the chemical name of picoplatin) for the treatment of small cell lung cancer.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 October 2007 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 | Picoplatin | Treatment of small cell lung cancer |
| Bulgarian | Пикоплатин | Лечение на дребноклетъчен карцином на белия дроб |
| Czech | Pikoplatin | Léčba malobuněčného karcinomu plic |
| Danish | Picoplatin | Behandling af småcellet lungecancer |
| Dutch | Picoplatine | Behandeling van kleincellig longcarcinoom |
| Estonian | Pikoplatiin | Väikeserakulise kopsuvähi ravi. |
| Finnish | Pikoplatiini | Keuhkojen pienisolusyövän hoito |
| French | Picoplatine | Traitement du cancer du poumon à petites cellules |
| German | Picoplatin | Behandlung des kleinzelligen Lungenkarzinoms |
| Greek | Πικοπλατίνη | Θεραπεία του μικροκυτταρικού καρκίνου του πνεύμονα |
| Hungarian | Picoplatin | Kissejtes tüdőrák kezelése |
| Italian | Picoplatino | Trattamento del cancro del polmone a piccole cellule (microcitoma) |
| Latvian | Pikoplatīns | Sīkšūnu plaušu vēža ārstēšana |
| Lithuanian | Pikoplatina | Smulkialąstelinio plaučių vėžio gydymas |
| Maltese | Picoplatin | Kura tal-kanċer tal-pulmun b'ċelloli żgħar |
| Polish | Pikoplatyna | Leczenie raka drobnokomórkowego pluc |
| Portuguese | Picoplatina | Tratamento do carcinoma de pequenas células do pulmão |
| Romanian | Picoplatin | Tratamentul cancerului pulmonar cu celule mici |
| Slovak | Pikoplatina | Liečba malobunkového karcinómu pľúc |
| Slovenian | Pikoplatin | Zdravljenje drobnocelicnega raka pljuc |
| Spanish | Picoplatino | Carcinoma de pulmón de células pequeñas |
| Swedish | Pikoplatin | Behandling av småcellig lungcancer |
| Norwegian | Pikoplatin | Behandling av småcellet lungekreft |
| Icelandic | Píkóplatín | Til meðferðar við lungnakrabbameini af smáfrumugerð |

[1] At the time of designation