4 March 2015
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Committee for Orphan Medicinal Products

# Public summary of opinion on orphan designation

## Nanoliposomal irinotecan for the treatment of pancreatic cancer

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| First publication | 20 December 2011 |
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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 9 December 2011, orphan designation (EU/3/11/933) was granted by the European Commission to Merrimack Pharmaceuticals UK Limited, United Kingdom, for nanoliposomal irinotecan for the treatment of pancreatic cancer.

The sponsorship was transferred to Baxter Innovations GmbH, Austria, in December 2014.

### What is pancreatic cancer?

Pancreatic cancer is cancer of the pancreas, a small organ that lies behind the stomach. The pancreas has two functions: to produce a juice that helps with the digestion of food, and to produce hormones such as insulin. Due to the absence of symptoms in the early stages of pancreatic cancer, the majority of patients are diagnosed when the cancer has spread locally or to other parts of the body.

Pancreatic cancer is a very severe and life-threatening disease that is associated with shortened life expectancy.

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

At the time of designation, pancreatic cancer affected approximately 1.4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 71,000 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 507,700,000 (Eurostat 2011).

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## What methods of treatment are available?

At the time of designation, several medicines were authorised in the EU for treating pancreatic cancer. The choice of treatment depended on several factors, including how far the disease had advanced. Treatments included surgery, radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer).

The sponsor has provided sufficient information to show that nanoliposomal irinotecan might be of significant benefit for patients with pancreatic cancer because it is a new formulation of irinotecan (an anticancer medicine authorised for the treatment of colorectal cancer), which is expected to 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?

Irinotecan is an anticancer medicine that belongs to the group 'topoisomerase inhibitors'. It blocks an enzyme called topoisomerase I, which is involved in the division of cell DNA. When the enzyme is blocked, the DNA strands break. This prevents the cancer cells from dividing and they eventually die.

Free irinotecan is already authorised for the treatment of colorectal cancer. In this medicine, irinotecan is contained within tiny fat particles called 'nanoliposomes'. The nanoliposomes are expected to accumulate within the tumour and release the medicine slowly over time, thereby decreasing the rate at which the irinotecan is removed from the body and allowing it to act for longer.

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

The effects of nanoliposomal irinotecan have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with nanoliposomal irinotecan in patients with pancreatic cancer were ongoing.

At the time of submission, nanoliposomal irinotecan was not authorised anywhere in the EU for the treatment of pancreatic cancer. Orphan designation of nanoliposomal irinotecan had been granted in United States of America for pancreatic cancer.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 October 2011 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 | Nanoliposomal irinotecan | Treatment of pancreatic cancer |
| Bulgarian | Нанолипозомен иринотекан | Лечение на рак на панкреаса |
| Croatian | Nanoliposomalni irinotekan | Liječenje raka gušterače |
| Czech | Nanoliposomální irinotekan | Léčba karcinomu pankreatu |
| Danish | Nanoliposomal irinotecan | Behandling af pancreascancer |
| Dutch | Nanoliposomale irinotecan | Behandeling van pancreaskanker |
| Estonian | Nanoliposomaalne irinotekaan | Pankreasevähi ravi |
| Finnish | Nanoliposomaalinen irinotekaani | Haimasyövän hoito |
| French | Irinotécan nanoliposomal | Traitement du cancer pancréatique |
| German | In Nanoliposomen verkapseltes Irinotecan | Behandlung des Pankreaskarzinoms |
| Greek | Νανολιποσωμιακή ιρινοτεκάνη | Θεραπεία καρκίνου του παγκρέατος |
| Hungarian | Nano-liposzómális irinotecan | Hasnyálmirigyrák kezelése |
| Italian | Irinotecan nano-liposomiale | Trattamento del cancro pancreatico |
| Latvian | Nanoliposomālais irinotekāns | Aizkuņģa dziedzera vēža ārstēšana |
| Lithuanian | Nanoliposominis irinotekanas | Kasos vėžio gydymas |
| Maltese | Irinotecan nanoliposomali | Kura tal-kanċer tal-frixa |
| Polish | Irynotekan nanoliposomalny | Leczenie raka trzustki |
| Portuguese | Irinotecano nanoliposomal | Tratamento do carcinoma do pâncreas |
| Romanian | Irinotecan nanolipozomal | Tratamentul cancerului pancreatic |
| Slovak | Nanolipozómový irinotekan | Liečba rakoviny pankreasu |
| Slovenian | Nanoliposomski irinotekan | Zdravljenje raka trebušne slinavke |
| Spanish | Irinotecán nanoliposomal | Tratamiento del cáncer de páncreas |
| Swedish | Nanoliposomalt irinotekan | Behandling av pankreascancer |
| Norwegian | Nanoliposomalt irinotekan | Behandling av pankreascancer |
| Icelandic | Nanólípósómal írínótekan | Meðferð briskrabbameins |

[1] At the time of transfer of sponsorship