



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

Polyinosine-polycytidylic acid coupled with the polycationic polyethyleneimine for the treatment of pancreatic cancer

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| First publication                                                                                                                                                                                                                                                                                                       | 2 July 2012       |
| Rev.1: withdrawal from the Community Register                                                                                                                                                                                                                                                                           | 12 September 2013 |
| <b>Disclaimer</b><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***Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in September 2013 on request of the Sponsor.***

On 6 June 2012, orphan designation (EU/3/12/1000) was granted by the European Commission to Bioncotech Therapeutics S.L., Spain, for polyinosine-polycytidylic acid coupled with the polycationic polyethyleneimine for the treatment of pancreatic cancer.

### 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 not more than 1.4 in 10,000 people in the European Union (EU)<sup>\*</sup>. This is equivalent to a total of not more than 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).

## **What treatments 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 the medicine 'polyinosine-polycytidylic acid coupled with the polycationic polyethyleneimine' might be of significant benefit to patients with pancreatic cancer because it works in a different way to existing treatments and may target the condition in a different manner. Early studies have shown that, when used alone or in combination with a 'MEK inhibitor' (another anticancer agent), the medicine reduces the number of viable cancer cells. It has also been shown in experimental models to reduce tumour size.

This assumption of significant benefit 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?**

The medicine is expected to act against tumour cells by activating an enzyme within the cell called helicase MDA-5, which will cause the cell to digest and kill itself. Moreover, the medicine is expected to stimulate the immune system to recognise and attack tumour cells.

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

At the time of submission of the application for orphan designation, the evaluation of the effects of the medicinal product in experimental models was ongoing.

At the time of submission, no clinical trials with the medicinal product in patients with pancreatic cancer had been started.

At the time of submission, the medicinal product was not authorised anywhere in the EU for pancreatic cancer 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 12 April 2012 recommending the granting of this designation.

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<sup>\*</sup>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).

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:

Bioncotech Therapeutics S.L.  
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Paterna (Valencia)  
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Telephone: +34 96 110 99 55  
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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    | Polyinosine-polycytidylic acid coupled with the polycationic polyethyleneimine   | Treatment of pancreatic cancer      |
| Bulgarian  | Полиинозин-полицитидилова киселина свързана с поликатионен полиетиленимин        | Лечение на рак на панкреаса         |
| Czech      | Polyinosin-polycytidylová kyselina vázaná na polykationický polyethylenimin      | Léčba karcinomu pankreatu           |
| Danish     | Polyinosin-polycytidylsyre, der er koblet med polykationisk polyethylenimin      | Behandling af pancreascancer        |
| Dutch      | Polyinosine-polycytidinezuur gekoppeld met polykationisch polyethyleenimine      | Behandeling van pancreaskanker      |
| Estonian   | Polüinosiin-polütsütidüülhape koos polükatioonse polüetüleenimiiniga             | Pankreasevähi ravi                  |
| Finnish    | Polyinosiini-polysytidyylihapo yhdistettynä polyetyleeni-imiiniin                | Haimasyövän hoito                   |
| French     | Acide polyinosine-polycytidylrique couplé au polyéthylène-imine polycationique   | Traitement du cancer pancréatique   |
| German     | Polysinosin-polycytidylinsäure gekoppelt mit polykationischem Polyethylenimin    | Behandlung des Pankreaskarzinoms    |
| Greek      | Πολυϊνοσίνη-πολυκυτιδικό οξύ σε συνδυασμό με την πολυκατιονική πολυαιθυλενοϊμίνη | Θεραπεία καρκίνου του παγκρέατος    |
| Hungarian  | Polyethyleneimine polykationhoz kapcsolt polyinosine-polycytidylic-sav           | Hasnyálmirigyrák kezelése           |
| Italian    | Acido poli-inosino- poli-citidinico accoppiato con polietilenimina policationica | Trattamento del cancro pancreatico  |
| Latvian    | Poliinozīna-policitidīna skābe kopā ar polikatjonu polietilēnimīnu               | Aizkuģa dziedzerā vēža ārstēšana    |
| Lithuanian | Poliinozino - policitidilo rūgštis, sujungta su polikatijoniniu polietileniminu  | Kasos vėžio gydymas                 |
| Maltese    | Aċidu polyinosine-polycytidylic akkoppjat ma' polycationic polyethyleneimine     | Kura tal-kanċer tal-frixa           |
| Polish     | Kwas poliinozyno-cytydylowy związany z polikationową polietylenoiminą            | Leczenie raka trzustki              |
| Portuguese | Ácido poliinosina-policitidílico associado ao polietileneimino policatiónico     | Tratamento do carcinoma do pâncreas |

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

| Language  | Active ingredient                                                              | Indication                         |
|-----------|--------------------------------------------------------------------------------|------------------------------------|
| Romanian  | Acid poliinosin policitidilic cuplat cu polietilenimină policationică          | Tratamentul cancerului pancreatic  |
| Slovak    | Kyselina polyinozín - polycytidylová viazaná s polykationovým polyetylénimínom | Liečba rakoviny pankreasu          |
| Slovenian | Polinozin-policitidilična kislina, vezana s polikationskim polietileniminom    | Zdravljenje raka trebušne slinavke |
| Spanish   | Complejo de ácido poliinosínico-polycitidílico y policación polietilenimina    | Tratamiento del cáncer de páncreas |
| Swedish   | Polyinosine-polycytidylsyra kopplad till polykatjonisk polyetylenimin          | Behandling av pankreascancer       |
| Norwegian | Polyinosin-polycytidyl syre koblet til polykationisk polyetylenimin            | Behandling av pankreascancer       |
| Icelandic | Pólýínósín-pólýsítidilík-sýra með pólýkasjónísku pólýepýlínímín                | Meðferð briskrabbameins            |