



5 January 2016  
EMA/COMP/696429/2015  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Azacitidine for the treatment of nasopharyngeal carcinoma

On 11 November 2015, orphan designation (EU/3/15/1570) was granted by the European Commission to Celgene Europe Limited, United Kingdom, for azacitidine for the treatment of nasopharyngeal carcinoma.

#### **What is nasopharyngeal carcinoma?**

Nasopharyngeal carcinoma is a type of cancer of the head and neck that begins in the upper part of the throat, behind the nose. It can have a variety of causes, including certain viral infections, exposure to cancer-causing chemicals and inherited genetic changes (mutations). It readily spreads to surrounding tissues and other parts of the body such as bones, liver and lungs.

The condition is long-term debilitating due to symptoms such as nose bleeds, blockage of the nose or mouth, hearing problems and tinnitus, headache, double vision and numbness, tingling or pain in the face. Nasopharyngeal carcinoma is also life threatening because it may come back after treatment and spread to other parts of the body.

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

At the time of designation, nasopharyngeal carcinoma affected approximately 0.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 10,000 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 the main method of treatment for nasopharyngeal carcinoma was radiotherapy (treatment with radiation), alone or combined with chemotherapy (medicines to treat cancer) and surgery. Various medicines were authorised in the EU for the treatment of head and neck cancer, which includes nasopharyngeal carcinoma.

---

\*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).



The sponsor has provided sufficient information to show that azacitidine might be of significant benefit for patients with nasopharyngeal carcinoma because preliminary results suggest that it may reduce growth of the cancer in patients whose disease has come back after, or not responded to, other treatments. 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?**

Azacitidine is an analogue of cytidine, a molecule found in the genetic material of cells (DNA and RNA). In the cell, azacitidine is incorporated into the DNA and RNA. It is thought to work by altering the way the cell turns genes on and off and also by interfering with the production of new RNA and DNA, which ultimately results in cell death. This is expected to reduce the growth of the cancer.

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

The effects of azacitidine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with azacitidine in patients with nasopharyngeal carcinoma were ongoing.

At the time of submission, azacitidine was authorised in the EU as Vidaza for the treatment of blood disorders known as myelodysplastic syndromes, and for certain leukaemias.

At the time of submission, azacitidine was not authorised anywhere in the EU for nasopharyngeal carcinoma 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 8 October 2015 recommending the granting of this designation.

---

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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's [rare disease designations page](#).

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	Azacididine	Treatment of nasopharyngeal carcinoma
Bulgarian	Азациитидин	Лечение на назофарингеален карцином
Croatian	Azacididin	Liječenje nazofaringealnog karcinoma
Czech	Azacididin	Léčba nazofaryngeálního karcinomu
Danish	Azacididin	Behandling af nasofaryngealt karcinom
Dutch	Azacididine	Behandeling van nasofaryngeaal carcinoom
Estonian	Asatsitidiin	Nasofarüngaalse kartsinoomi ravi
Finnish	Atsasiitidiini	Nenäielukarsinooman hoito
French	Azacididine	Traitement du carcinome nasopharyngé
German	Azacididin	Behandlung des nasopharyngealen Karzinoms
Greek	Αζακτιδίνη	Θεραπεία καρκινώματος ρινοφάρυγγα
Hungarian	Azacididin	Nasopharyngealis carcinoma kezelése
Italian	Azacidina	Trattamento del carcinoma nasofaringeo
Latvian	Azacidīns	Nazofaringeāla vēža ārstēšana
Lithuanian	Azacidinas	Nosiaryklės karcinomos gydymas
Maltese	Azacididine	Kura tal-karċinoma nażofaringali
Polish	Azacytydyna	Leczenie raka części nosowej gardła
Portuguese	Azacidina	Tratamento do carcinoma nasofaringeo
Romanian	Azacidină	Tratamentul carcinomului nazofaringian
Slovak	Azacidín	Liečba karcinómu nosohltanu
Slovenian	Azacididin	Zdravljenje nazofaringealnega karcinoma
Spanish	Azacidina	Tratamiento del carcinoma de nasofaringe
Swedish	Azacididin	Behandling av nasofaryngealt karcinom
Norwegian	Azacytidin	Behandling av nasofaryngealt karsinom
Icelandic	Azacítidín	Meðferð við krabbameini í nefkoki

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