



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

2 March 2011  
EMA/COMP/740415/2010  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Nimorazole for the treatment of squamous cell carcinoma of the head and neck in patients undergoing radiotherapy

On 23 February 2011, orphan designation (EU/3/10/842) was granted by the European Commission to Azanta A/S, Denmark, for nimorazole for the treatment of squamous cell carcinoma of the head and neck in patients undergoing radiotherapy.

#### **What is squamous cell carcinoma of the head and neck?**

Squamous cell carcinoma of the head and neck is a type of cancer that starts in the 'squamous cells' lining the mouth, nose, throat or larynx (voice box). This type of cancer is often associated with alcohol and tobacco use, and may also be associated with local viral infection. Radiotherapy, the use of radiation to kill cancer cells, is used in about two thirds of patients with this cancer.

Squamous cell carcinoma of the head and neck is a long-lasting, debilitating and life-threatening disease that is associated with poor overall survival.

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

At the time of designation, squamous cell carcinoma of the head and neck in patients undergoing radiotherapy affected less than 4 in 10,000 people in the European Union (EU)\*. This is equivalent to a total of fewer than 202,000 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, treatment of patients with squamous cell carcinoma of the head and neck depended on the site and stage of development of their cancer. Surgery was widely used where possible to remove the tumour, followed by radiotherapy and chemotherapy (medicines to treat cancer), while a number of other medicines were authorised for use in this disease.

---

\*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. This represents a population of 506,500,000 (Eurostat 2010).



The sponsor has provided sufficient information to show that nimorazole might be of significant benefit for patients with squamous cell carcinoma of the head and neck undergoing radiotherapy, because it works in a different way to existing treatments and may enhance the effectiveness of radiotherapy, thus improving 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?**

Nimorazole belongs to a group called 'nitroimidazoles' that are widely used as anti-infective agents and is already authorised in some EU countries as an antimicrobial agent.

In squamous cell carcinoma of the head and neck, nimorazole is expected to work by acting as a 'radiotherapy sensitiser'. This means that it is expected to make the cancer cells more sensitive to the effect of radiation, thus enhancing the effect of radiotherapy.

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

The effects of nimorazole have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with nimorazole in patients with squamous cell carcinoma of the head and neck undergoing radiotherapy were ongoing.

At the time of submission, nimorazole was not authorised anywhere in the EU for squamous cell carcinoma of the head and neck in patients undergoing radiotherapy 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 10 November 2010 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:

Azanta A/S  
Tranegaardsvej 20  
2900 Hellerup  
Denmark  
Telephone: +45 70 25 95 45  
Telefax: +45 70 25 95 46  
E-mail: [info@azanta.com](mailto:info@azanta.com)

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	Nimorazole	Treatment of squamous cell carcinoma of the head and neck in patients undergoing radiotherapy
Bulgarian	Ниморазол	лечение на плоскоклетъчен карцином на главата и врата при пациенти, които са подложени на радиотерапия
Czech	Nimorazol	Léčba spinocelulárního karcinomu hlavy a krku u pacientů podstupujících radioterapii
Danish	Nimorazol	Behandling af pladeepitelcarcinom i hoved- og halsregion i patienter som får strålebehandling
Dutch	Nimorazol	Behandeling van plaveiselcelcarcinoom in hoofd en nek bij patiënten die radiotherapie ondergaan
Estonian	Nimorazool	Pea ja kaela lamerakulise vähiga patsientide ravi radioteraapia korral
Finnish	Nimoratsoli	Pään ja kaulan levyepiteelikarsinooman hoiton potilailla, jotka saavat sädehoitoa
French	Nimorazole	Traitement du carcinome épidermoïde de la tête et du cou chez les patients subissant une radiothérapie
German	Nimorazol	Behandlung von Plattenepithelkarzinomen im Kopf- und Halsbereich bei Patienten, die sich einer Strahlentherapie unterziehen
Greek	Νιμοραζόλη	θεραπεία του πλακώδους καρκινώματος της κεφαλής και του αυχένα σε ασθενείς που υποβάλλονται σε ακτινοθεραπεία
Hungarian	Nimorazol	feji és nyaki pikkelyes rákban szenvedő, sugárkezelésben részesülő betegek kezeléséhez
Italian	Nimorazolo	Trattamento del carcinoma spinocellulare della testa e del collo in pazienti sottoposti a radioterapia
Latvian	Nimorazolas	Galvas un kakla plakanšūnu vēža slimnieku ārstēšanai, kuri saņem staru terapiju.
Lithuanian	Nimorazolas	Plokščialąstelinio galvos ir kaklo vėžio gydymas, radioterapija gydomiems pacientams
Maltese	Nimorazole	Kura ta' kanċer taċ-ċelluli skwamużi tar-ras u tal-għonq f'pazjenti għaddejjin bir-radjuterapija
Polish	Nimorazol	Leczenie nowotworu kolczystokomórkowego głowy i szyi u pacjentów przechodzących radioterapię
Portuguese	Nimorazol	Tratamento do carcinoma de células escamosas da cabeça e do pescoço, em doentes submetidos a radioterapia
Romanian	Nimorazol	Tratamentul carcinomului scuamos al capului și gâtului la pacienții supuși radioterapiei
Slovak	Nimorazol	Liečba karcinómu z dlaždicových buniek na hlave a krku u pacientov podstupujúcich rádioterapiu.
Slovenian	Nimorazol	Zdravljene raka skvamoznih celic glave in vratu, ob potekajoči radioterapiji bolnika

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

Spanish	nimorazol	Tratamiento de carcinoma de células escamosas de cabeza y cuello en pacientes recibiendo radioterapia
Swedish	Nimorazol	Behandling av skivepitelkarcinom i huvud- och halsområdet hos patienter som får strålbehandling
Norwegian	Nimorazol	Behandling av plateepitelkarsinom i hode- og halsregionen hos pasienter som får strålebehandling
Icelandic	Nímórazól	Meðferð á flöguþekjukrabbameini á höfuð- og hálssvæði hjá sjúklingum sem eru í geislameðferð.