



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Soluble yeast beta-1,3/1,6- glucan for the prevention of oral mucositis in head and neck cancer patients undergoing radiation therapy

On 16 June 2005, orphan designation (EU/3/05/294) was granted by the European Commission to Biotec Pharmacon ASA, Norway, for soluble yeast beta-1,3/1,6- glucan for the prevention of oral mucositis in head and neck cancer patients undergoing radiation therapy.

#### **What is oral mucositis?**

Radiation therapy is often used as a treatment option in selected tumours. The technique employs high-dose x-rays or other high-energy rays to kill cancer cells. Radiation therapy will not only kill the tumour cells but will also cause some degree of injury to the surrounding tissue. Radiation therapy given to tumours located in the head and neck region might thus harm the rapidly dividing inner lining (mucosa) of the mouth. This complication of radiation therapy is called oral mucositis.

The condition is chronically debilitating, resulting in severe pain and dryness of the mouth, which leads to difficulties in swallowing.

#### **What is the estimated number of patients at risk of developing the condition?**

At the time of designation, the number of patients at risk of oral mucositis in head and neck cancer patients undergoing radiation therapy was estimated to be not more than 2 people in 10,000 in the European Union (EU)\*. This is equivalent to a total of not more than 92,000 people, which 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 methods of prevention are available?**

No satisfactory methods exist that were authorised at the time of application.

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\*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 25), Norway, Iceland and Lichtenstein. This represents a population of 459,700,000 (Eurostat 2004).



## **How is this medicine expected to work?**

It is not yet fully understood how soluble yeast beta-1,3/1,6- glucan exerts a direct or indirect protection against the negative effects induced by the radiation therapy. It seems that it might act at different levels, by enhancing, for example, the wound healing after the oral lesion, and by acting locally on certain cells within the mucosa, belonging to the body's defence system (immune competent cells). This might stimulate the immune system against possible infections while reducing the inflammation reaction.

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

The evaluation of the effects of soluble yeast beta-1,3/1,6- glucan in experimental models is ongoing.

At the time of submission of the application for orphan designation, clinical trials in head and neck cancer patients receiving radiotherapy were ongoing.

The medicinal product was not marketed anywhere worldwide as a medicinal product for prevention of oral mucositis in head and neck cancer patients undergoing radiation therapy or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 May 2005 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:

Biotec Pharmacon ASA  
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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	Soluble yeast beta-1,3/1,6-glucan	Prevention of oral mucositis in head and neck cancer patients undergoing radiation therapy
Czech	Rozpustný kvasinkový beta-1,3/1,6-glukan	Prevence orální mukositivity u pacientů s nádorovým onemocněním v oblasti hlavy a krku podstupujících radioterapii
Danish	Opløselig β-1,3/1,6-glukan fra gær	Forebyggelse af <i>oral mucositis</i> i patienter med kræft i hoved- og halsregionen som får strålebehandling
Dutch	Oplosbaar β-1,3/1,6-glucan uit gist	Preventie van orale mucositis in hoofd- en nekkankerpatienten die stralingstherapie ondergaan
Estonian	Lahustuv pärimi beeta-1,3/1,6-glükaan	Suuõõne mukosiidi profülaktika patsientidel, kes saavad kiiritusravi pea- ja kaelapiirkonna vähi vastu
Finnish	Liukeneva β -1,3/1,6-glukaani hiivasta	<i>Oraalisen mucositis</i> :in ennaltaehkäisy pään- ja kaulan alueen syöpää sairastavilla potilailla, jotka saavat sädehoitoa
French	β-1,3/1,6-glucan soluble de levure	Prévention de la <i>mucite bucco-pharyngée</i> chez les patients atteints de cancer de la tête et du cou subissant une radiothérapie
German	Lösliches β-1,3/1,6-glucan von Hefe	Prävention der <i>oralen Mucositis</i> (Schleimhautentzündung im Mund) nach Radiotherapie im Kopf- und Halsbereich gegen Krebs
Greek	Διαλυτή βήτα-1,3/1,6-γλυκάνη ζύμης	Πρόληψη της στοματίτιδας σε ασθενείς με καρκίνο της κεφαλής και του λαιμού που υποβάλλονται σε ακτινοθεραπεία
Hungarian	Oldható béta -1,3/1,6)-glukán élesztő	Fej- és nyakrák sugárkezelését követő orális mucositis megelőzése
Italian	Beta-1,3/1,6-glucano solubile da lievito	Prevenzione delle <i>mucositi orali</i> in pazienti con cancro nella regione della testa e del collo sottoposti a radioterapia
Latvian	Šķīstošs rauga beta-1,3/1,6-glukāns	Mutes gļotādas iekaisuma novēršana galvas un kakla vēža pacientiem, kas saņem staru terapiju
Lithuanian	Tirpusis mielių beta-1,3/1,6-gliukanas	Burnos gleivinės uždegimo (mukozito) prevencija pacientams, kuriems taikomas radioterapinis gydymas dėl galvos ir kaklo srities vėžio
Polish	Rozpuszczalny drożdżowy β-1,3/1,6-glukan	Zapobieganie zapaleniu śluzówki jamy ustnej u pacjentów poddawanych radioterapii w przebiegu raka głowy i szyi

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

Language	Active Ingredient	Indication
Portuguese	Glucano $\beta$ -1,3/1,6- de levedura solúvel	Prevenção da mucosite oral induzida pela radioterapia em doentes com neoplasia de cabeça e pescoço.
Slovak	Rozpustný fungálny beta-1,3/1,6-glukán	Prevenia orálnej mukositídy u pacientov podstupujúcich rádioterapiu pri liečbe rakoviny hlavy a krku
Slovenian	Topen beta-1,3/1,6-glukan iz kvasa	Preprečevanje oralnega mukozitisa pri pacientih, ki so izpostavljeni radioterapiji v območju glave in vratu
Spanish	Soluble $\beta$ -1,3/1,6-glucan de la levadura	Prevención de la mucositis oral en pacientes tratados con radioterapia por cánceres de la región de la cabeza y el cuello
Swedish	Lösligt $\beta$ -1,3/1,6-glukan från jäst	Profylax av <i>oral mucositis</i> hos huvud-hals cancer patienter som får strålbehandling
Norwegian	Oppløselig $\beta$ -1,3/1,6-glukan fra gjær	Forebygging av <i>oral mucositis</i> hos pasienter med kreft i hode- og halsregionen som får strålebehandling
Icelandic	Leysanlegt $\beta$ -1,3/1,6-glúkan úr gersveppum	Vörn gegn <i>slímubólgu í munni</i> hjá sjúklingum í geislamedferð vegna krabbameins á höfuð-og hálssvæði