



COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF sodium butyrate (rectal use) for the prevention of radiation proctitis

On 27 May 2005, orphan designation (EU/3/05/284) was granted by the European Commission to Promefarm srl, Italy, for sodium butyrate (rectal use) for the prevention of radiation proctitis.

What is radiation proctitis?

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 also the adjacent normal cells or will cause some degree of injury to the surrounding tissue. Radiation therapy given to tumours located in the pelvis might thus harm the rapidly dividing inner lining (mucosa) of the intestines. Therefore, it may cause proctitis, which is an inflammation of the lowest part of the intestine leading to the anus, the so-called rectum. Patients may experience rectal pain, increase in bowel frequency, diarrhoea, tenesmus (painful and often ineffectual straining), urgency, fecal incontinence, mucous discharge and bleeding. Proctitis can last for weeks or months.

Radiation proctitis is a chronically debilitating condition and may be life-threatening due to complications in some patients.

What are the methods of prevention available?

No satisfactory methods exist for the prevention of radiation proctitis that were authorised at the time of application.

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

According to the information provided by the sponsor, the estimated number of patients at risk of developing radiation proctitis was considered to be about 161,000 persons in the European Union.

How is this medicinal product expected to act?

Sodium butyrate, if applied as a rectal solution, might protect the mucosa of the rectum from the injuries caused by the radiation therapy. Sodium butyrate could stimulate the repair and healing of the rectal mucosa.

What is the stage of development of this medicinal product?

The effects of sodium butyrate (rectal use) were evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients eligible for prevention of radiation proctitis were initiated.

The medicinal product was not marketed anywhere worldwide for prevention of radiation proctitis or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 7 April 2005 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

For more information:

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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). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

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**Translations of the active ingredient and indication in all EU languages
and Norwegian and Icelandic**

Language	Active Ingredient	Indication
English	Sodium butyrate (rectal use)	Prevention of radiation proctitis
Czech	Butyrát sodný (rektální aplikace)	Prevence radiační proktitidy
Danish	Natrumbutyrat (rektal anvendelse)	Forebyggelse af stråleinduceret proctitis
Dutch	Natrium butyraat (rectaal gebruik)	Preventie van stralingsproctitis
Estonian	Naatriumbutüraat (rektaalseks kasutamiseks)	Radiatsioonist tingitud proktiidi ennetamine
Finnish	Natriumbutyaatti (rektaalinen käyttö)	Säteilyn aiheuttaman proktiitin estoon
French	Butyrate de sodium (usage rectal)	Prévention de la proctite de radiation
German	Natriumbutytrat (rektale Anwendung)	Prophylaxe der radiogenen Proktitis
Greek	Βουτυρικό νάτριο (ορθική χρήση)	Πρόληψη πρωκτίτιδας από ακτινοβολία
Hungarian	Nátrium butirát (rektális alkalmazásra)	Sugárkezelés okozta proctitis megelőzése
Italian	sodio butirato (uso rettale)	Prevenzione della proctite da radiazione
Latvian	Nātrija butirāts (rektālai lietošanai)	Apstarošanas proktīta profilakse
Lithuanian	Natrio butyratas (vartojamas į tiesiąją žarną)	Radiacinio proktito prevencija
Polish	Sodu maślan (podanie doodbytnicze)	Zapobieganie popromiennemu zapaleniu odbytnicy
Portuguese	Butirato de sódio (uso rectal)	Prevenção da proctite de radiação
Slovak	Natrium butyrát (rektálne použitie)	Prevenca radiačnej proktitídy
Slovenian	Natrijev butirat (rektalna uporaba)	Preprečevanje radiacijskega proktitisa
Spanish	butirato sódico (uso rectal)	Prevenición de la proctitis inducida por radioterapia
Swedish	Natriumbutytrat (rektal användning)	Förebyggande behandling av proktit orsakad av strålning
Norwegian	Natriumbutytrat (rektal bruk)	Forebygging av stråleindusert proktitt
Icelandic	Natríum butyrate (Til notkunar í endaparm)	Forvörn gegn endaparmsbólgu vegna geislameðferðar