



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

23 March 2011  
EMA/COMP/152488/2008 Rev.2  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

Tegafur, gimeracil, oteracil potassium for the treatment of gastric cancer

***Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in January 2011 on request of the Sponsor.***

On 20 December 2007, orphan designation (EU/3/07/515) was granted by the European Commission to Sanofi Aventis, France, for tegafur, gimeracil, oteracil potassium for the treatment of gastric cancer.

The sponsorship was transferred to Quintiles Ireland Ltd in February 2009 and subsequently to Taiho Pharma Europe Limited, United Kingdom, in May 2009.

### What is gastric cancer?

Cancer that originates in the stomach is called gastric cancer. These tumours are usually first discovered and diagnosed in the affected patients at an advanced stage of the disease when the tumours have spread to surrounding organs in the abdomen or other parts of the body. Symptoms of gastric cancer include loss of appetite, vomiting, weight loss, bleeding in the abdomen and subsequent anaemia. Most gastric cancers are adenocarcinomas, so called because they start in the cells that line the inside of the stomach (the mucosa). Gastric cancer is chronically debilitating and life threatening.

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

At the time of designation, gastric cancer affected approximately 3 in 10,000 people in the European Union (EU)\*. This is equivalent to a total of around 150,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

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\* 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 498,000,000 (Eurostat 2006).



## **What treatments are available?**

If the tumour is diagnosed at an early stage, the cancer might be treated with surgery. Otherwise, the most common way of treating gastric cancer is through chemotherapy (using drugs to kill cancer cells). There were several medicinal products authorised for the treatment of gastric cancer in the Community at the time of orphan status designation.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that tegafur, gimeracil, oteracil potassium might be of potential significant benefit for the treatment of gastric cancer, mainly because of its new mechanism of action and because it may provide a major contribution to patient care. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

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

Tegafur, gimeracil, oteracil potassium is a product that combines a chemotherapeutic substance (tegafur) with two modulators; gimeracil and oteracil potassium. Modulators are drugs that are designed to enhance the activity of another drug, in this case, the activity of tegafur. Tegafur is similar to a naturally occurring molecule that is a precursor to one of the building blocks of DNA (the genetic material found in every cell in the body). Without all these building blocks, cells cannot grow, divide and subsequently survive. This precursor, deoxy-uridylate, is usually modified by an enzyme (protein with catalytic activity) called thymidylate synthase, into thymidine monophosphate. According to the sponsor, tegafur acts by binding to thymidylate synthase and by inhibiting its activity, it hinders the growth of cancer cells and contributes to the destruction of the gastric tumour.

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

The effects of tegafur, gimeracil, oteracil potassium were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with gastric cancer were ongoing.

Tegafur, gimeracil, oteracil potassium was authorised for the treatment of gastric cancer in Japan and South Korea, at the time of submission. Orphan designation of tegafur, gimeracil, oteracil potassium was granted in the United States for the treatment of gastric cancer.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 November 2007 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:

Taiho Pharma Europe Limited  
c/o Wragge & Co LLP  
3 Waterhouse Square  
142 Holborn  
London EC1 2SW  
United Kingdom  
Telephone: +44 207 664 0314  
Telefax: +44 207 664 0315

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	Tegafur, gimeracil, oteracil potassium	Treatment of gastric cancer
Bulgarian	Тегатур, гимерацил, калиев отерацил	Лечение на карцином на стомаха
Czech	Tegafur, gimeracil, oteracil draselný	Léčba karcinomu žaludku
Danish	Tegafur, gimeracil, oteracil kalium	Behandlingen af cancer ventriculi
Dutch	Tegafur, gimeracil, oteracil kalium	Behandeling van maagkanker
Estonian	Tegafur, gimeratsiil, kaaliumoteratsiil	Maovähi ravi
Finnish	Tegafuuri, gimerasiili, kaliumoterasiili	Mahasyövän hoito
French	Tégafur, gimeracil, otéracil potassium	Traitement du cancer gastrique
German	Tegafur, Gimeracil, Oteracil-Kalium	Behandlung von Magenkrebs
Greek	Tegafur, gimeracil, oteracil potassium	Θεραπεία του γαστρικού καρκίνου
Hungarian	tegafur, gimeracil, oteracil kálium	Gyomorrák kezelése
Italian	Tegafur, gimeracile, oteracile potassio	Trattamento del cancro gastrico
Latvian	Tegafūrs, gimeracils, oteracila kālijs	Kuņģa vēža ārstēšana
Lithuanian	Tegafuras, gimeracilas, kalio oteracilas	Skrandžio vėžio gydymas
Maltese	Tegafur, gimeracil, oteracil potassium	Kura tal-kanċer gastriku
Polish	Tegafur, gimeracyl, oteracyl potasu	Leczenie raka żołądka
Portuguese	Tegafur, gimeracilo, oteracilo potássico	Tratamento do cancro gástrico
Romanian	Tegafur, gimeracil, oteracil potasic	Tratamentul cancerului gastric
Slovak	Tegafur, gimeracil, draselná soľ oteracilu	Liečba rakoviny žalúdka
Slovenian	Tegafur, gimeracil, oteracil kalij	Zdravljenje karcinoma želodca
Spanish	Tegafur, gimeracilo, oteracilo potásico	Tratamiento del cáncer de estómago
Swedish	Tegafur, gimeracil, oteracilkalium	Behandling av magcancer
Norwegian	Tegafur, gimeracil, oteracilkalium	Behandling av magekreft
Icelandic	Tegafúr, gimeracíl, óteracíl kalíum	Meðferð við magakrabbameini

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