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Public summary of opinion on orphan designation

Gefinitib for the treatment of Fanconi anaemia

On 26 October 2018, orphan designation (EU/3/18/2075) was granted by the European Commission to Consorcio Centro de Investigación Biomédica en Red, M.P., Spain, for gefinitib for the treatment of Fanconi anaemia.

What is Fanconi anaemia?

Fanconi anaemia is an inherited disorder that mainly affects the bone marrow and results in reduced production of blood cells. Patients with Fanconi anaemia are generally born with physical malformations, and over the years, they develop a wide range of complications such as bone marrow failure (inability to produce new blood cells) and cancers such as squamous cell cancer of the head and neck and acute myeloid leukaemia (a cancer of the white blood cells).

Fanconi anaemia is a severely debilitating and life-threatening condition because of the bone marrow failure and the susceptibility to cancers, which lead to shortened life expectancy.

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

At the time of designation, Fanconi anaemia affected less than 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 5,000 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?

The medicine is intended for the treatment of head and neck squamous cell carcinoma in patients with Fanconi anaemia. At the time of application for orphan designation, treatments for head and neck squamous cell carcinoma included chemotherapy and radiotherapy.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with Fanconi anaemia because preliminary studies suggest that gefinitib may cause fewer side effects in patients with Fanconi anaemia than currently available treatments for head and

*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 517,400,000 (Eurostat 2018).

neck squamous cell carcinoma. 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?

Gefitinib is a tyrosine-kinase inhibitor. This means that it blocks specific enzymes known as tyrosine kinases. In particular, gefitinib blocks the tyrosine kinase in receptors (targets) called EGFRs which are found on the surface of cancer cells and are involved in their growth and spread. By blocking the tyrosine kinase in EGFR, gefitinib is expected to help slow down the growth and spread of head and neck squamous cell carcinoma in patients with Fanconi anaemia.

What is the stage of development of this medicine?

The effects of gefitinib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with gefitinib in patients with Fanconi anaemia had been started.

Gefitinib is authorised in the EU for the treatment of non-small cell lung cancer (NSCLC).

At the time of submission, gefitinib was not authorised anywhere in the EU for Fanconi anaemia 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 13 September 2018 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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Gefitinib	Treatment of Fanconi anaemia
Bulgarian	Гeфитиниб	Лечение на анемия на Фанкони
Croatian	Gefitinib	Liječenje Fanconijeve anemije
Czech	Gefitinib	Léčba Fanconiho anémie
Danish	Gefitinib	Behandling af Fanconis anæmi
Dutch	Gefitinib	Behandeling van Fanconi-anemie
Estonian	Gefitiniib	Fanconi aneemia ravi
Finnish	Gefitinibi	Fanconin anemian hoito
French	Gefitinib	Traitement de l'anémie de Fanconi
German	Gefitinib	Behandlung der Fanconi-Anämie
Greek	Γεφτινίμπη	Θεραπεία της αναιμίας Fanconi
Hungarian	Gefitinib	Fanconi-anémia kezelése
Italian	Gefitinib	Trattamento dell'anemia di Fanconi
Latvian	Gefitinibs	Fankoni (<i>Fanconi</i>) anēmijas ārstēšana
Lithuanian	Gefitinibas	<i>Fanconi</i> anemijos gydymas
Maltese	Gefitinib	Kura tal-anemija ta' Fanconi
Polish	Gefitynib	Leczenie niedokrwistości Fanconiego
Portuguese	Gefitinib	Tratamento da anemia de Fanconi
Romanian	Gefitinib	Tratamentul anemiei Fanconi
Slovak	Gefitinib	Liečba Fanconiho anémie
Slovenian	Gefitinib	Zdravljenje Fanconijeve anemije
Spanish	Gefitinib	Tratamiento de la anemia de Fanconi
Swedish	Gefitinib	Behandling av Fanconis anemi
Norwegian	Gefitinib	Behandling av Fanconis anemi
Icelandic	Gefitiníð	Meðferð á Fanconi blóðleysi

¹ At the time of designation