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EMA/COMP/90982/2010
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Raloxifene hydrochloride for the treatment of hereditary haemorrhagic telangiectasia

On 10 June 2010, orphan designation (EU/3/10/730) was granted by the European Commission to Consejo Superior de Investigaciones Cientificas (CSIC), Spain, for raloxifene hydrochloride for the treatment of hereditary haemorrhagic telangiectasia.

What is hereditary haemorrhagic telangiectasia?

Hereditary haemorrhagic telangiectasia (HHT, also known as Rendu-Osler-Weber syndrome) is a genetic disease that causes abnormalities in the capillaries (small blood vessels that connect arteries with veins). This results in direct connections between arteries and veins, which are fragile, increasing the risk of bleeding. The most common symptoms of the disease are spontaneous and frequent nosebleeds, and red spots on the skin, particularly on the face and hands and in the mouth. Bleeding can also occur in the stomach, gut, brain, liver and lungs, and often leads to anaemia (low red blood cell counts).

HHT is a long-term debilitating disease that may be life threatening because of its complications, such as stroke and liver problems.

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

At the time of designation, HHT affected approximately 2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 101,000 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 the knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of designation, no satisfactory methods were authorised in the EU for the treatment of HHT. Different methods were used to control bleeding, which depended mainly on where in the body it

*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).

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occurred. For nosebleeds, patients used nasal humidifiers and lubricants. Laser treatment and surgery were used to stop internal bleeding. In patients with severe liver problems, liver transplantation was performed. When bleeding caused anaemia, patients were given iron supplements and blood transfusions.

How is this medicine expected to work?

Patients with HHT have a genetic defect that affects the production of two proteins known as ALK1 and ENG. These proteins are involved in angiogenesis (formation of new blood vessels) and wound healing. Raloxifene is expected to increase the production of these proteins, and to relieve the symptoms of bleeding in HHT.

What is the stage of development of this medicine?

The effects of raloxifene hydrochloride have been evaluated in experimental models.

At the time of submission of the application for orphan designation, two observational studies with raloxifene hydrochloride in HHT postmenopausal women had been conducted.

At the time of submission, raloxifene hydrochloride was not authorised anywhere in the EU for HHT 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 3 February 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 Community) 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:

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Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Raloxifene hydrochloride	Treatment of hereditary haemorrhagic telangiectasia
Bulgarian	Ралоксифен хидрохлорид	Лечение на наследствена хеморагична телангиектазия
Czech	Raloxifen hydrochlorid	Léčba hereditární hemoragické telangiektázie
Danish	Raloxifenhydrochlorid	Behandling af hereditær hæmoragisk telangiektasi
Dutch	Raloxifenhydrochloride	Behandeling van hereditaire hemorrhagische telangiëctasie
Estonian	Raloksifeenvesinikkloriidi	Päriliku hemorraagilise teleangiektaasia ravi
Finnish	Raloksifeenihydrokloridi	Perinnöllisen hemorragisen telangiektasian hoito
French	Chlorhydrate de raloxifène	Traitement de la télangiectasie hémorragique héréditaire (Rendu-Osler)
German	Raloxifenhydrochlorid	Behandlung der hereditären hämorrhagischen Teleangiektasie
Greek	Υδροχλωρική ραλοξιφαίνη	Θεραπεία της κληρονομικής αιμορραγικής τηλαγγειεκτασίας
Hungarian	Raloxifen-hidroklorid	Örökletes vérzéses hajszálértágulat kezelése
Italian	Raloxifene cloridrato	Trattamento della telangiectasia emorragica ereditaria
Latvian	Raloksifēna hidrohlorīds	Iedzimtas hemorāģiskas teleangiektāzijas ārstēšana
Lithuanian	Raloksifeno hidrochloridas	Paveldimos hemoraginės telangiektazijos gydymas
Maltese	Raloxifene hydrochloride	Kura tat-telanġektasija ereditarja emorraġika
Polish	Chlorowodorek raloksyfenu	Leczenie wrodzonej naczyniakowatości krwotocznej
Portuguese	Cloridrato de raloxifeno	Tratamento das telangiectasias hemorrágicas hereditárias
Romanian	Clorhidrat de raloxifen	Tratamentul teleangiectaziei hemoragice ereditare
Slovak	Raloxifén hydrochlorid	Liečba hereditárnej hemoragickej teleangiektázie
Slovenian	Raloksifenijev hidroklorid	Zdravljenje dedne hemoragične teleangiektazije
Spanish	Clorhidrato de raloxifeno	Tratamiento de la telangiectasia hemorrágica hereditaria
Swedish	Raloxifenhydroklorid	Behandling av ärftlig hemoragisk telangiektasia
Norwegian	Raloksifenhydroklorid	Behandling av Hereditær hemoragisk telangiektasi
Icelandic	Raloxífen hýdróklóríð	Meðhöndlun á arfgengri blæðinga-háræðavíkkun

[1] At the time of designation