15 January 2019
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Public summary of opinion on orphan designation

Etamsylate for the treatment of hereditary haemorrhagic telangiectasia

On 19 November 2018, orphan designation (EU/3/18/2087) was granted by the European Commission to Consejo Superior de Investigaciones Cientificas, Spain, for etamsylate 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 in which the capillaries (tiny blood vessels that connect arteries with veins) do not develop properly. This results in abnormal direct connections between arteries and veins, which are fragile, and can increase the risk of bleeding. The most common symptoms of the disease are 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 internal bleeding and effects on organs such as the gut, brain, liver and lungs.

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 was equivalent to a total of around 103,000 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?

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 depend mainly on where in the body it 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

*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).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

performed. When bleeding caused anaemia, patients were given iron supplements and blood transfusions.

How is this medicine expected to work?

Etamsylate acts in several ways to help stop bleeding. It increases the formation of proteins involved in blood clotting and decreases formation of prostacyclin I2, a type of fat that blocks clotting. Etamsylate also increases the activity of platelets (cell fragments in blood that help the blood to clot). The medicine is expected to be used as a nasal spray, which would reduce nosebleeds in patients with HHT.

What is the stage of development of this medicine?

The effects of etamsylate have been evaluated in experimental models.

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

At the time of submission, etamsylate was authorised in several European countries as tablets or solution for injection for prevention of bleeding.

At the time of submission, etamsylate 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 18 October 2018 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:

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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Etamsylate	Treatment of hereditary haemorrhagic telangiectasia
Bulgarian	Етамзилат	Лечение на наследствена хеморагична телангиектазия
Croatian	Etamzilat	Liječenje hereditarne hemoragijske teleangiektazije
Czech	Etamsylát	Léčba hereditární hemoragické telangiektázie
Danish	Etamsylat	Behandling af hereditær hæmoragisk telangiektasi
Dutch	Etamsylaat	Behandeling van hereditaire hemorrhagische telangiëctasie
Estonian	Etamsülaat	Päriliku hemorraagilise teleangiektaasia ravi
Finnish	Etamsylaatti	Perinnöllisen hemorragisen telangiektasian hoito
French	Etamsylate	Traitement de la télangiectasie hémorragique héréditaire
German	Etamsylat	Behandlung der hereditären hämorrhagischen Teleangiektasie
Greek	Εταμσυλάτη	Θεραπεία της κληρονομικής αιμορραγικής τηλαγγειεκτασίας
Hungarian	Etamszilát	Örökletes vérzéses hajszálértágulat kezelése
Italian	Etamsilato	Trattamento della telangiectasia emorragica ereditaria
Latvian	Etamsilāts	Iedzimtas hemorāģiskas teleangiektāzijas ārstēšana
Lithuanian	Etamsilatas	Paveldimos hemoraginės telangiektazijos gydymas
Maltese	Etamsilat	Kura tat-telanġektasija ereditarja emorraġika
Polish	Etamsylan	Leczenie wrodzonej naczyniakowatości krwotocznej
Portuguese	Etamsilato	Tratamento das telangiectasias hemorrágicas hereditárias
Romanian	Etamsilat	Tratamentul teleangiectaziei hemoragice ereditare
Slovak	Etamsylát	Liečba hereditárnej hemoragickej teleangiektázie
Slovenian	Etamsilat	Zdravljenje dedne hemoragične teleangiektazije
Spanish	Etamsilato	Tratamiento de la telangiectasia hemorrágica hereditaria
Swedish	Etamsylat	Behandling av ärftlig hemoragisk telangiektasia
Norwegian	Etamsylat	Behandling av hereditær hemoragisk telangiektasi
Icelandic	Etamsýlat	Meðhöndlun á arfgengri blæðinga-háræðavíkkun

[1] At the time of designation