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Public summary of opinion on orphan designation

Thalidomide for the treatment of hereditary haemorrhagic telangiectasia

On 27 February 2017, orphan designation (EU/3/17/1845) was granted by the European Commission to PlumeStars s.r.l., Italy, for thalidomide 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 direct connections between arteries and veins, which are fragile, increasing 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 not more than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 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 performed. When bleeding caused anaemia, patients were given iron supplements and blood transfusions.

*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 515,700,000 (Eurostat 2017).

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How is this medicine expected to work?

In HHT, thalidomide is thought to work on mural cells (cells in the blood vessel wall) by increasing their number and their ability to support blood vessels. This leads to blood vessels becoming stronger. These effects of thalidomide are expected to reduce bleeding, especially nose bleeds, in patients with HHT.

What is the stage of development of this medicine?

The company provided data from the published literature on the effects of thalidomide in experimental models.

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

At the time of submission, thalidomide was authorised in the EU for the treatment of multiple myeloma.

At the time of submission, the medicine 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 19 January 2017 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	Thalidomide	Treatment of hereditary haemorrhagic telangiectasia
Bulgarian	Талидомид	Лечение на наследствена хеморагична телангиектазия
Croatian	Talidomid	Liječenje hereditarne hemoragijske teleangiektazije
Czech	Tahlidomid	Léčba hereditární hemoragické telangiektázie
Danish	Thalidomid	Behandling af hereditær hæmoragisk telangiektasi
Dutch	Thalidomide	Behandeling van hereditaire hemorrhagische telangiëctasie
Estonian	Talidomiid	Päriliku hemorraagilise teleangiektaasia ravi
Finnish	Talidomidi	Perinnöllisen hemorragisen telangiektasian hoito
French	Thalidomide	Traitement de la télangiectasie hémorragique héréditaire
German	Thalidomid	Behandlung der hereditären hämorrhagischen Teleangiektasie
Greek	Θαλιδομιδη	Θεραπεία της κληρονομικής αιμορραγικής τηλαγγειεκτασίας
Hungarian	Thalidomid	Örökletes vérzéses hajszálértágulat kezelése
Italian	Talidomide	Trattamento della telangiectasia emorragica ereditaria
Latvian	Talidomīds	Iedzimtas hemorāģiskas teleangiektāzijas ārstēšana
Lithuanian	Talidomidas	Paveldimos hemoraginės telangiektazijos gydymas
Maltese	Thalidomide	Kura tat-telanġektasija ereditarja emorraġika
Polish	Talidomid	Leczenie wrodzonej naczyniakowatości krwotocznej
Portuguese	Talidomida	Tratamento das telangiectasias hemorrágicas hereditárias
Romanian	Talidomidă	Tratamentul teleangiectaziei hemoragice ereditare
Slovak	Talidomid	Liečba hereditárnej hemoragickej teleangiektázie
Slovenian	Talidomid	Zdravljenje dedne hemoragične teleangiektazije
Spanish	Talidomida	Tratamiento de la telangiectasia hemorrágica hereditaria
Swedish	Thalidomid	Behandling av ärftlig hemoragisk telangiektasia
Norwegian	Talidomid	Behandling av Hereditær hemoragisk telangiektasi
Icelandic	Talídómíð	Meðhöndlun á arfgengri blæðinga-háræðavíkkun

[1] At the time of designation