



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Gusperimus trihydrochloride for the treatment of Wegener's granulomatosis

On, 29 March 2001 orphan designation (EU/3/01/034) was granted by the European Commission to Euro Nippon Kajaku GmbH, Germany, for gusperimus trihydrochloride for the treatment of Wegener's granulomatosis.

The sponsorship was transferred to Nordic Group B.V., The Netherlands, in September 2011.

What is Wegener's granulomatosis?

Wegener's granulomatosis is a disease characterised by chronic inflammation of the blood vessels affecting the respiratory tract (sinuses, nose, trachea (windpipe) and lungs (swelling and redness) and kidneys. This inflammation can damage the organs (partial or complete obstruction of the lung) by limiting the blood flow to them and destroying normal tissue. Wegener's granulomatosis is considered as a life threatening and chronically debilitating condition.

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

At the time of designation, approximately 0.4 to 0.6 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 15,000 to 23,000 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 submission of the application for the orphan drug designation there were some medicinal products authorised for the condition in some member states of the Community.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that gusperimus trihydrochloride might be of potential significant benefit for the treatment of Wegener's granulomatosis. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

*Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition.



How is this medicine expected to work?

The exact way gusperimus trihydrochloride works in the body is not fully understood. Gusperimus trihydrochloride is a chemically synthesised immunosuppressive agent (a compound that reduces the activity of the immune system). The medicinal product interferes with an intracellular signalling event, thus inhibiting growth and differentiation of certain cell of the immune system and, in this way, a special pathway is expected to reduce the symptoms of Wegener's granulomatosis.

What is the stage of development of this medicine?

The effects of gusperimus trihydrochloride were evaluated in experimental models.

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

At the time of submission, gusperimus trihydrochloride was not marketed anywhere worldwide for the treatment of Wegener's granulomatosis or designated as 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 9 February 2001 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:

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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 | Gusperimus trihydrochloride | Treatment of Wegener's granulomatosis |
| Bulgarian | Гусперимус трихидрохлорид | Лечение на грануломатоза на Вегенер |
| Czech | Gusperimus trihydrochlorid | Léčba Wegenerovy granulomatózy |
| Danish | Gusperimus trihydrochlorid | Behandling af Wegeners granulomatose |
| Dutch | Gusperimus-trihydrochloride | Behandeling van Wegeners granulomatosis |
| Estonian | Gusperimus trihüdrokloriid | Wegeneri granulomatoosi ravi |
| Finnish | Gusperimus trihydrokloridi | Wegenerin granulomatoosin hoito |
| French | Gusperimus trichlorhydrique | Traitement de la granulomatose de Wegener |
| German | Gusperimus trihydrochlorid | Behandlung von Wegener-Granulomatose |
| Greek | Γκασπέριμους τριυδροχλωρικό | Θεραπεία για κοκκιωμάτωση Wegener's |
| Hungarian | Guszperimusz trihidroklorid | Wegener granulomatózis kezelése |
| Italian | Gusperimus triclorigrato | Trattamento della granulomatosi di Wegener |
| Latvian | Gusperīma trihidrohlorīds | Vēgenera granulomatozes ārstēšana |
| Lithuanian | Gusperimuso trihidrochloridas | Vegenerio granulomatozės gydymas |
| Maltese | Gusperimus trihydrochloride | Kura tal-granulomatoži ta' Wegener |
| Polish | Trójchlorowodorek gusperimusu | Leczenie ziarniniakowatości Wegenera |
| Portuguese | Gusperimus trihidrocloridrato | Tratamento da granulomatose de Wegener |
| Romanian | Gusperimus trihidroclorid | Tratamentul granulomatozei Wegener |
| Slovak | Gusperimus trihydrochlorid | Liečba Wegenerovej granulomatózy |
| Slovenian | Gusperimijev trihidroklorid | Zdravljenje Wegenerjeve granulomatoze |
| Spanish | Gusperimus trihidroclorhidrato | Tratamiento de la granulomatosis de Wegener |
| Swedish | Gusperimus trihydroklorid | Behandling av Wegeners granulomatos |

¹ At the time of transfer of sponsorship