



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Vorinostat for the treatment of multiple myeloma

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2013 on request of the Sponsor.

On 15 April 2011, orphan designation (EU/3/11/854) was granted by the European Commission to Merck Sharp & Dohme Limited, United Kingdom, for vorinostat for the treatment of multiple myeloma.

What is multiple myeloma?

Multiple myeloma is a cancer of a type of white blood cell called plasma cells. Plasma cells are found in the bone marrow, the spongy tissue inside the large bones in the body. In multiple myeloma, the division of plasma cells becomes out of control, resulting in abnormal, immature plasma cells multiplying and filling up the bone marrow. This interferes with production of normal white blood cells, red blood cells and platelets (components that help the blood to clot), leading to complications such as anaemia (low red blood cell counts), bone pain and fractures, raised blood calcium levels and kidney disease.

Multiple myeloma is a debilitating and life-threatening disease that is associated with poor long-term survival.

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

At the time of designation, multiple myeloma affected less than 2.6 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 132,000 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).

^{*}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. At the time of designation, this represented a population of 507,700,000 (Eurostat 2011).



What treatments are available?

At the time of designation, several medicines were authorised for multiple myeloma in the EU. The main treatment for multiple myeloma was chemotherapy (medicines to treat cancer) usually combined with steroids to reduce the activity of the immune system, the body's natural defences. Radiotherapy (treatment with radiation) was used in treating pain and weakened bones. Interferon alfa, a protein normally produced by the body during viral infections, was sometimes used in combination with chemotherapy.

The sponsor has provided sufficient information to show that vorinostat might be of significant benefit for patients with multiple myeloma because early studies show that it might improve the treatment of patients with this condition, when used in combination with existing treatments. 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?

Vorinostat blocks the activity of enzymes called histone deacetylases, which are involved in turning genes 'on' and 'off' within cells. In multiple myeloma, vorinostat is expected to switch 'on' the genes that suppress the division and growth of the tumour cells. This is expected to lead to a reduction in the growth and division of the cancer cells.

What is the stage of development of this medicine?

The effects of vorinostat have been evaluated in experimental models.

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

At the time of submission, vorinostat was authorised in several countries outside the European Union for the treatment of cutaneous T-cell lymphoma.

At the time of submission, vorinostat was not authorised anywhere in the EU for multiple myeloma. Orphan designation of vorinostat had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 January 2011 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	Vorinostat	Treatment of multiple myeloma
Bulgarian	Вориностат	Лечение на мултиплен миелом
Czech	Vorinostat	Léčba mnohočetného myelomu
Danish	Vorinostat	Behandling af multipelt myelom
Dutch	Vorinostat	Behandeling van multipel myeloom
Estonian	Vorinostat	Multiibelse müeloomi ravi
Finnish	Vorinostaatti	Multippeli myelooman hoito
French	Vorinostat	Traitement du myélome multiple
German	Vorinostat	Behandlung des multiplen Myeloms
Greek	Βορινοστάτη	Θεραπευτική αγωγή πολλαπλού μυελώματος
Hungarian	Vorinostat	Myeloma multiplex kezelése
Italian	Vorinostat	Trattamento del mieloma multiplo
Latvian	Vorinostat	Multiplās mielomas ārstēšana
Lithuanian	Vorinostatas	Dauginės mielomos gydymas
Maltese	Vorinostat	Kura tal-mjeloma multipla
Polish	Worinostat	Leczenie szpiczaka mnogiego
Portuguese	Vorinostato	Tratamento do mieloma múltiplo
Romanian	Vorinostat	Tratamentul mielomului multiplu
Slovak	Vorinostat	Liečba mnohopočetného myelómu
Slovenian	Vorinostat	Zdravljenje multiplega mieloma
Spanish	Vorinostat	Tratamiento del mieloma múltiple
Swedish	Vorinostat	Behandling av multipelt myelom
Norwegian	Vorinostat	Behandling av myelomatose
Icelandic	Vorínóstat	Meðferð við mergfrumuæxli

¹ At the time of designation