



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

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Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in December 2008 on request of the Sponsor.

Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of thalidomide for the treatment of multiple myeloma

On 19 December 2001, orphan designation (EU/3/01/068) was granted by the European Commission to Kendle International Limited, United Kingdom, for thalidomide for the treatment of multiple myeloma.

What is multiple myeloma?

Multiple myeloma is a cancer of a type of white blood cell called a plasma cell. Plasma cells are found in the bone marrow. The bone marrow is the spongy tissue inside the large bones in the body. Normally, the bone marrow makes cells called “blasts” that mature into several different types of blood cells that have specific functions in the body. These include red cells, white cells and platelets. Red blood cells carry oxygen and other materials to all tissues of the body. Platelets make the blood clot, and white blood cells fight infection. In multiple myeloma an excessive number of plasma cells are produced. Normally, the division of cells takes place in a controlled manner but with multiple myeloma, the process gets out of control and abnormal plasma cells multiply, producing many myeloma cells. These fill up the bone marrow and interfere with production of the normal white cells, red cells and platelets. This leads to a number of possible complications, which include anaemia, bone pain and fractures, raised levels of calcium in the blood and kidney disease. Multiple myeloma is life-threatening.

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

At the time of designation, multiple myeloma affected approximately 1.2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 45,000 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 knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

The main treatment of multiple myeloma is chemotherapy (using drugs to kill cancer cells) usually combined with steroids (a group of chemical substances, belonging to a larger class of molecules, the so-called hormones, which have an effect on the activity of certain organs). Several products were authorised for the condition in the Community at the time of submission of the application for orphan drug designation. Radiotherapy (using high-dose x-rays or other high-energy rays to kill cancer cells) can be very useful to treat painful areas and weakened bones. Interferon alpha is a protein normally

*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.

produced by the body during viral infections, such as flu. Sometimes a combination of interferon and chemotherapy may be used.

Thalidomide could be of potential significant benefit for the treatment of multiple myeloma. The main reason is that it may offer a new way of killing cancer cells and stopping tumour growth in these patients. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Cancers need to produce a network of new blood vessels in order to grow. Without forming these blood vessels cancers cannot grow. The theory is that thalidomide will prevent the myeloma from growing by preventing the development of new blood vessels and possibly also reducing the supply of oxygen and nutrients to the cancer cells.

What is the stage of development of this medicine?

The evaluation of the effects of thalidomide in experimental models is ongoing.

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

Thalidomide was not marketed anywhere worldwide for multiple myeloma, at the time of submission. Orphan designation of thalidomide was granted in the United States for the treatment of multiple myeloma.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 7 September 2001 a positive opinion recommending the grant of the above-mentioned 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.

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Translations of the active ingredient and indication in all EU languages

Language	Active Ingredient	Indication
English	Thalidomide	Treatment of multiple myeloma
Danish	Thalidomid	Behandling af multipelt myelom
Dutch	Thalidomide	Behandeling van multipel myeloom
Finnish	Talidomidi	Multippelin myelooman hoito
French	Thalidomide	Traitement du myélome multiple
German	Thalidomid	Behandlung des multiplen Myeloms
Greek	Θαλιδομιδη	θεραπεία του πολλαπλού μυελώματος
Italian	Talidomide	Trattamento del mieloma multiplo
Portuguese	Talidomida	Tratamento do mieloma múltiplo
Spanish	Talidomida	Tratamiento del mieloma múltiple
Swedish	Thalidomid	Behandling av multipelt myelom