29 January 2014
EMA/COMP/90859/2010 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Perifosine for the treatment of multiple myeloma

First publication	22 June 2010
Rev.1: withdrawal from the Community Register	29 January 2014

Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in January 2014 on request of the Sponsor.

On 10 June 2010, orphan designation (EU/3/10/740) was granted by the European Commission to Æterna Zentaris GmbH, Germany, for perifosine 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.

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

At the time of designation, multiple myeloma affected less than 2.2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 111,000 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 the knowledge of the Committee for Orphan Medicinal Products (COMP).

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 considered to be very useful 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 perifosine might be of significant benefit for patients with multiple myeloma because it works in a different way to existing treatments, and early studies show that it might be used in patients who do not respond to existing treatments. In addition, perifosine will be available as tablets, whereas most of the existing treatments need to be given by injection. These assumptions 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?

Perifosine is expected to work by blocking some enzymes, known as protein kinases B (or Akt). These enzymes are involved in stimulating cells to grow and divide. Cancerous cells grow and divide uncontrollably. By blocking these enzymes, the medicine is expected to help control the growth and division of the cancerous cells and slow down the development of multiple myeloma.

What is the stage of development of this medicine?

The effects of perifosine have been evaluated in experimental models.

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

At the time of submission, perifosine was not authorised anywhere in the EU for multiple myeloma. Orphan designation of perifosine 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 3 February 2010 recommending the granting of this designation.

*Disclaimer: 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 506,300,000 (Eurostat 2010).

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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Perifosine	Treatment of multiple myeloma
Bulgarian	Перифозин	Лечение на мултиплен миелом
Czech	Perifosin	Léčba mnohočetného myelomu
Danish	Perifosin	Behandling af multipelt myelom
Dutch	Perifosine	Behandeling van multipel myeloom
Estonian	Perifosiin	Multiibelse müeloomi ravi
Finnish	Perifosiini	Multippeli myelooman hoito
French	Perifosine	Traitement du myélome multiple
German	Perifosin	Behandlung des multiplen Myeloms
Greek	Περιφοσίνη	Θεραπευτική αγωγή πολλαπλού μυελώματος
Hungarian	Perifosin	Myeloma multiplex kezelése
Italian	Perifosina	Trattamento del mieloma multiplo
Latvian	Perifozīns	Multiplās mielomas ārstēšana
Lithuanian	Perifosinas	Dauginės mielomos gydymas
Maltese	Perifosine	Kura tal-mjeloma multipla
Polish	Perifozyna	Leczenie szpiczaka mnogiego
Portuguese	Perifosina	Tratamento do mieloma múltiplo
Romanian	Perifosină	Tratamentul mielomului multiplu
Slovak	Perifosin	Liečba mnohopočetného myelómu
Slovenian	Perifosin	Zdravljenje multiplega mieloma
Spanish	Perifosina	Tratamiento del mieloma múltiple
Swedish	Perifosin	Behandling av multipelt myelom
Norwegian	Perifosină	Tratamentul mielomului multiplu
Icelandic	Perifosin	Liečba mnohopočetného myelómu
Norwegian	Perifosin	Behandling av myelomatose
Icelandic	Perifósín	Meðferð við mergfrumuæxli

[1] At the time of designation