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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Venetoclax for the treatment of multiple myeloma

On 14 October 2016, orphan designation (EU/3/16/1767) was granted by the European Commission to Abbvie Ltd, United Kingdom, for venetoclax for the treatment of multiple myeloma.

What is multiple myeloma?

Multiple myeloma (also called plasma cell myeloma) is a cancer of a type of white blood cell called plasma cells. Plasma cells originate 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 particularly because it disrupts the normal functioning of the bone marrow, damages the bones and causes kidney failure.

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

At the time of designation, multiple myeloma affected approximately 3.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 164,000 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, several medicines were already authorised for multiple myeloma in the EU. The main treatment for multiple myeloma was chemotherapy (medicines to treat cancer) usually combined with corticosteroids to reduce the activity of the immune system, the body's natural defences. Where chemotherapy did not work, some patients received a stem-cell transplant (a procedure where the patient's bone marrow is replaced with stem cells to form new bone marrow that

*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 513,700,000 (Eurostat 2016).

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produces healthy blood cells). Radiotherapy (using radiation to kill cancer cells) was used to treat pain due to bone damage and prevent further damage.

The sponsor has provided sufficient information to show that venetoclax might be of significant benefit for patients with multiple myeloma because early studies in patients show that venetoclax may be effective when other treatment has not worked or when the disease has come back. 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?

Venetoclax is expected to work by blocking proteins called Bcl-2. These proteins allow cells to stay alive by preventing the natural process that leads to cell death (apoptosis). Bcl-2 proteins can be found in high levels in cancer cells. By blocking the action of these proteins, the medicine is expected to make cancer cells more responsive to this natural process, causing their death and slowing the growth of the cancer.

What is the stage of development of this medicine?

The effects of venetoclax have been evaluated in experimental models.

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

At the time of submission, venetoclax was not authorised anywhere in the EU for multiple myeloma. Orphan designation of the medicine had been granted in the EU and the United States for chronic lymphocytic leukaemia and for acute myeloid leukaemia, and in the United States for diffuse large B-cell lymphoma.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 September 2016 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	Venetoclax	Treatment of multiple myeloma
Bulgarian	Венетоклакс	Лечение на мултиплен миелом
Croatian	Venetoklaks	Liječenje multiplog mijeloma
Czech	Venetoclax	Léčba mnohočetného myelomu
Danish	Venetoclax	Behandling af multipelt myelom
Dutch	Venetoclax	Behandeling van multipel myeloom
Estonian	Venetoklaks	Multiibelse müeloomi ravi
Finnish	Venetoklaksi	Multippeli myelooman hoito
French	Venetoclax	Traitement du myélome multiple
German	Venetoclax	Behandlung des multiplen Myeloms
Greek	Βενετοκλάξη	Θεραπεία πολλαπλού μυελώματος
Hungarian	Venetoklax	Myeloma multiplex kezelése
Italian	Venetoclax	Trattamento del mieloma multiplo
Latvian	Venetoklaks	Multiplās mielomas ārstēšana
Lithuanian	Venetoklaksas	Dauginės mielomos gydymas
Maltese	Venetoclax	Kura tal-mjeloma multipla
Polish	Wenetoklaks	Leczenie szpiczaka mnogiego
Portuguese	Venetoclax	Tratamento do mieloma múltiplo
Romanian	Venetoclax	Tratamentul mielomului multiplu
Slovak	Venetoklax	Liečba mnohopočetného myelómu
Slovenian	Venetoklaks	Zdravljenje multiplega mieloma
Spanish	Venetoclax	Tratamiento del mieloma múltiple
Swedish	Venetoclax	Behandling av multipelt myelom
Norwegian	Venetoclax	Behandling av myelomatose
Icelandic	Venetóklax	Meðferð við mergfrumuæxli

[1] At the time of designation