17 June 2011
EMA/COMP/89/2002 Rev.3
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Azacitidine for the treatment of myelodysplastic syndromes

On 6 February 2002, orphan designation (EU/3/01/084) was granted by the European Commission to Pharmion Limited, United Kingdom, for azacitidine for the treatment of myelodysplastic syndromes.

The sponsorship was transferred to Celgene Europe Limited, United Kingdom, in October 2008.

What are myelodysplastic syndromes?

The myelodysplastic syndromes (MDS) are a group of disorders of the blood. The MDS are characterised by ineffective formation of blood and bone marrow failure. MDS are life-threatening.

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

At the time of designation, MDS affected between 1.1 and 3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of between 41,000 and 113,000 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?

There was no satisfactory treatment authorised in the European Union for this medical condition at the time of submission of the application for orphan drug designation. Available therapeutic options for MDS included supportive care, the use of growth factors such as erythropoietin, chemotherapy, differentiating agents, and bone-marrow transplantation.

How is this medicine expected to work?

Azacitidine is an analogue of cytidine which is part of in the fundamental genetic material of cells (DNA and RNA). Azacitidine inhibits the synthesis of DNA and RNA and thus inhibits the growth of tumour cells.

*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).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

What is the stage of development of this medicine?

The effects of azacitidine have been evaluated in experimental models and the sponsor had completed a number of clinical trials in patients with MDS at the time of orphan designation.

According to the sponsor azacitidine was not authorised in any country in the designated indication, and had not been designated as an orphan medicinal product elsewhere, at the time of submission of the designation application.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 21 November 2001 recommending the granting of this designation.

Update: Azacitidine (Vidaza) has been authorised in the EU since 17 December 2008 for the treatment of adult patients who are not eligible for haematopoietic stem cell transplantation with:

- intermediate-2 and high-risk myelodysplastic syndromes (MDS) according to the International Prognostic Scoring System (IPSS),
- chronic myelomonocytic leukaemia (CMML) with 10-29 % marrow blasts without myeloproliferative disorder,
- acute myeloid leukaemia (AML) with 20-30 % blasts and multi-lineage dysplasia, according to World Health Organisation (WHO) classification.

More information on Vidaza can be found in the European public assessment report (EPAR) on the Agency's website: ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports

_____

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:

Celgene Europe Limited
1 Longwalk Road
Stockley Park
Uxbridge
Middlesex UB11 1DB
United Kingdom
Telephone: +44 208 831 83 00
Telefax: +44 208 831 83 01
E-mail: medinfo.uk.ire@celgene.com

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	Azacitidine	Treatment of myelodysplastic syndromes
Bulgarian	Азацитидин	Лечение на миелодиспластичен синдром
Czech	Azacitidin	Léčba myelodysplastického syndrome
Danish	Azacitidine	Behandling af myelodysplastisk syndrom
Dutch	Azacitidine	Behandeling van myelodysplasie syndroom
Estonian	Asatsitidiin	Müelodüsplastiliste sündroomide ravi
Finnish	Atsasitidiini	Myelodysplastisten oireyhtymien hoito
French	Azacitidine	Traitement des syndrômes myélodysplasiques
German	Azacitidin-	Behandlung des myelodysplastischen Syndroms
Greek	Azacitidine	Θεραπεία Μυελοδυσπλαστικού Συνδρόμου
Hungarian	Azacitidin	Myelodysplasias syndroma kezelése
Italian	Azacitidina	Trattamento delle Sindromi mielodisplastiche
Latvian	Azacitidīns	Mielodisplastisko sindromu ārstēšana
Lithuanian	Azacitidinas	Mielodisplastinių sindromų gydymas
Maltese	Azacitidine	Kura tas-sindromi mjelodisplastiċi
Polish	Azacytydyna	Leczenie zespołów mielodysplastycznych
Portuguese	Azacitidina	Tratamento do Síndrome Mielodisplásico
Romanian	Azacitidină	Tratamentul sindromului mielodisplazic
Slovak	Azacitidín	Liečba myelodysplastického syndrómu
Slovenian	Azacitidin	Zdravljenje mielodisplastičnega sindroma
Spanish	Azacitidina	Tratamiento del síndrome Mielodisplásico
Swedish	Azacitidin	Behandling av myeloplastiskt syndrom

[1] At the time of transfer of sponsorship