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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Tosedostat for the treatment of acute myeloid leukaemia

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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.	

On 24 July 2009, orphan designation (EU/3/09/659) was granted by the European Commission to Chroma Therapeutics Ltd, United Kingdom, for tosedostat for the treatment of acute myeloid leukaemia.

The sponsorship was transferred to Voisin Consulting S.A.R.L., France, in May 2015.

What is acute myeloid leukaemia?

Acute myeloid leukaemia (AML) is a cancer of the white blood cells (cells that fight against infections). In patients with AML, the bone marrow (the spongy tissue inside the large bones) produces large numbers of abnormal, immature white blood cells called 'blasts'. These abnormal cells quickly build up in large numbers in the bone marrow and are found in the blood.

AML is a life-threatening disease because these immature cells take the place of the normal white blood cells, reducing the patient's ability to fight infections.

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

At the time of designation, AML affected less than 2 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 101,000 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 504,800,000 (Eurostat 2009).



What treatments are available?

Treatment for AML is complex and depends on a number of factors including the extent of the disease, whether it has been treated before, and the patient's age, symptoms and general state of health. At the time of designation, the main treatments for AML were chemotherapy (medicines to kill cancer cells) and bone marrow transplantation (a complex procedure where the bone marrow of the patient is destroyed and replaced with healthy bone marrow from a matched donor).

The sponsor has provided sufficient information to show that tosedostat might be of significant benefit for patients with AML because it works in a different way to existing treatments and because early studies indicate that it may be an alternative treatment for some patients, particularly elderly patients. 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?

Tosedostat is expected to work as an 'aminopeptidase inhibitor'. This means that it is expected to block the activity of aminopeptidase, an enzyme that is involved in the breakdown of proteins in the body. By blocking this enzyme, tosedostat is expected to decrease the level of 'amino acids', the building blocks of proteins, in the body's cells. With fewer amino acids available for the production of new proteins, cells are expected to find it harder to grow and multiply. Since cancer cells are more dependent on the production of new proteins than normal cells, tosedostat is expected to slow down the growth and spread of AML.

What is the stage of development of this medicine?

The effects of tosedostat have been evaluated in experimental models.

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

At the time of submission, tosedostat was not authorised anywhere in the EU for AML. Orphan designation of tosedostat had been granted in the United States of America for AML.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 June 2009 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	Tosedostat	Treatment of acute myeloid leukaemia
Bulgarian	Тоседостат	Лечение на остра миелоидна левкемия
Croatian	Tosedostat	Liječenje akutne mijeloične leukemije
Czech	Tosedostat	Léčba akutní myeloidní leukémie
Danish	Tosedostat	Behandling af akut myeloid leukæmi
Dutch	Tosedostat	Behandeling van acute myeloïde leukemie
Estonian	Tosedostat	Akuutse müeloidse leukeemia ravi
Finnish	Tosedostaatti	Akuutin myelooisen leukemian hoito
French	Tosédostat	Traitement de la leucémie aiguë myéloïde
German	Tosedostat	Behandlung der akuten myeloischen Leukämie
Greek	Τοσεδοστάτ	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Tosedostat	Akut myeloid leukaemia kezelése
Italian	Tosedostat	Trattamento della leucemia mieloide acuta
Latvian	Tosedostats	Akūtas mieloleikozes ārstēšana
Lithuanian	Tosedostatas	Ūmios mieloleukozės gydymas
Maltese	Tosedostat	Kura tal-lewkimja mjelojda akuta
Polish	Tosedostat	Leczenie ostrej białaczki szpikowej
Portuguese	Tosedostat	Tratamento da leucémia mielóide aguda
Romanian	Tosedostat	Tratamentul leucemiei mieloide acute
Slovak	Tosedostat	Liečba akútnej myeloidkej leukémie
Slovenian	Tosedostat	Zdravljenje akutne mieloične levkemije
Spanish	Tosedostat	Tratamiento de la leucemia mieloide aguda
Swedish	Tosedostat	Behandling av akut myeloisk leukemi
Norwegian	Tosedostat	Behandling av akutt myelogen leukemi
Icelandic	Tósedóstat	Meðferð við bráðu kyrningahvítblæði

¹ At the time of transfer of sponsorship