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EMA/COMP/72409/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Arsenic trioxide for the treatment of acute myeloid leukaemia

On 17 February 2016, orphan designation (EU/3/16/1610) was granted by the European Commission to Orsenix Holdings BV, the Netherlands, for arsenic trioxide for the treatment of acute myeloid leukaemia.

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, where blood cells are produced) produces large numbers of abnormal, immature white blood cells. These abnormal cells quickly build up in large numbers in the bone marrow and are found in the blood.

AML is a long-term debilitating and life-threatening disease because these abnormal immature cells take the place of the normal blood cells, causing bleeding episodes, blood clots and 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 approximately 1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 51,000 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?

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 treat cancer) and haematopoietic (blood) stem-cell transplantation (a complex procedure where the patient receives stem cells from a matched donor to help restore the bone marrow).

*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).

The sponsor has provided sufficient information to show that arsenic trioxide might be of significant benefit for patients with AML because data from the published literature show that it has anti-cancer effects in some patients who have not been treated before. In addition, this medicine can be taken by mouth and so may be easier to take than the currently authorised medicine containing arsenic trioxide (Trisenox) which has to be given into a vein. 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?

Arsenic trioxide is a chemical that has been used in medicines for many years, including for the treatment of leukaemia. The way it works in this disease is not completely understood. It is thought to prevent the production of DNA, which is necessary for leukaemia cells to grow.

What is the stage of development of this medicine?

The effects of this medicine have been evaluated in experimental models.

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

At the time of submission, the medicine was not authorised anywhere in the EU for AML or designated as an orphan medicinal product elsewhere for this condition.

At the time of submission, the medicine Trisenox (arsenic trioxide given into a vein) was authorised in the EU for the treatment of acute promyelocytic leukaemia (APL), a rare form of AML. Trisenox is for use in patients whose disease had not responded to or had come back after previous treatment.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 21 January 2016 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:

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Arsenic trioxide	Treatment of acute myeloid leukaemia
Bulgarian	Арсенов триоксид	Лечение на остра миелоидна левкемия
Croatian	Arsenov trioksid	Liječenje akutne mijeloične leukemije
Czech	Trioxid arsenitý	Léčba akutní myeloidní leukémie
Danish	Arsenik trioxid	Behandling af akut myeloid leukæmi
Dutch	Arseentrioxide	Behandeling van acute myeloïde leukemie
Estonian	Arseentrioksiid	Akuutse müeloidse leukeemia ravi
Finnish	Arseenitrioksiidi	Akuutin myelooisen leukemian hoito
French	Trioxyde d'arsenic	Traitement de la leucémie aiguë myéloïde
German	Arsentrioxid	Behandlung der akuten myeloischen Leukämie
Greek	Τριοξειδιο αρσενικού	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Arsenum trioxydatum	Akut myeloid leukaemia kezelése
Italian	Triossido di arsenico	Trattamento della leucemia mieloide acuta
Latvian	Arsēna trioksīds	Akūtas mieloleikozes ārstēšana
Lithuanian	Arseno trioksidas	Ūmios mieloleukozės gydymas
Maltese	Arsenic trioxide	Kura tal-lewkimja mjelojda akuta
Polish	Arsenu tritlenek	Leczenie ostrej białaczki szpikowej
Portuguese	Trióxido de arsénico	Tratamento da leucemia mielóide aguda
Romanian	Arsenic trioxide	Tratamentul leucemiei mieloide acute
Slovak	Oxid arzenitý	Liečba akútnej myeloickej leukémie
Slovenian	Arzenov trioksid	Zdravljenje akutne mieloične levkemije
Spanish	Trióxido de arsénico	Tratamiento de la leucemia mieloide aguda
Swedish	Arseniktrioxid	Behandling av akut myeloisk leukemi
Norwegian	Arsentrioksid	Behandling av akutt myelogen leukemi
Icelandic	Arsenik þríoxíð	Meðferð við bráðu kyrningahvítblæði

¹ At the time of designation