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Public summary of opinion on orphan designation

6,8-bis(benzylthio)octanoic acid for the treatment of acute myeloid leukaemia

On 14 December 2018, orphan designation (EU/3/18/2123) was granted by the European Commission to IQVIA RDS Ireland Limited, Ireland, for 6,8-bis(benzylthio)octanoic acid (also known as CPI-613) 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 infection). In patients with AML, the bone marrow (the spongy tissue inside the large bones, where blood cells are produced) produces 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 the abnormal immune cells take the place of the normal blood cells, causing bleeding episodes, blood clots and reduced ability to fight infections.

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

At the time of designation, AML affected approximately 1.4 in 10,000 in the European Union (EU). This was equivalent to a total of around 72,000 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 depends on several 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 procedure where the patient's bone marrow is

*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 517,400,000 (Eurostat 2018).

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cleared of cells and replaced by stem cells to form new bone marrow that produces healthy blood cells).

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with AML. Early studies in older patients whose disease had spread or had not improved with treatment suggest that they may live longer when the medicine is added to standard treatment. 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?

The medicine, which is taken up in large amounts by cancer cells, blocks two enzymes that are needed for mitochondria (the energy-producing components within cells) to work properly. As a result, the cells cannot produce the energy needed to survive and grow. This is expected to lead to the death of cancer cells in patients with AML and thereby slow down the growth of the cancer.

What is the stage of development of this medicine?

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

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

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

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 November 2018 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 the EMA website.

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	6,8-bis(benzylthio)octanoic acid	Treatment of acute myeloid leukaemia
Bulgarian	6,8-бис(бензилтио)октанова киселина	Лечение на остра миелоидна левкемия
Croatian	6,8-bis(benziltio)oktanska kiselina	Liječenje akutne mijeloične leukemije
Czech	6,8-bis(benziltio)oktanová kyselina	Léčba akutní myeloidní leukémie
Danish	6,8-bis(benzyl thiol)octansyre	Behandling af akut myeloid leukæmi
Dutch	6,8-bis(benzylthio)octaanzuur	Behandeling van acute myeloïde leukemie
Estonian	6,8-bis-(bensüültio)-oktaanhape	Akuutse müeloidse leukeemia ravi
Finnish	6,8-bis(bentsyylitio)oktaanihappo	Akuutin myelooisen leukemian hoito
French	Acide 6,8-bis(benzylthio)octanoïque	Traitement de la leucémie aiguë myéloïde
German	6,8-Bis(benzylthio)octansäure	Behandlung der akuten myeloischen Leukämie
Greek	6,8-δι(βενζυλοθειο)οκτανοϊκό οξύ	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	6,8-bisz-(benzil-tio)-oktánsav	Akut myeloid leukaemia kezelése
Italian	Acido 6,8-bis(benziltio)ottanoico	Trattamento della leucemia mieloide acuta
Latvian	6,8-bis(benziltio)kaprilskābe	Akūtas mieloleikozes ārstēšana
Lithuanian	6,8-bis(benziltio)oktano rūgštis	Ūmios mieloleukozės gydymas
Maltese	6,8-bis(benzylthio)octanoic acid	Kura tal-lewkimja mjelojda akuta
Polish	Kwas 6,8-bis(benzylotio) oktanowy	Leczenie ostrej białaczki szpikowej
Portuguese	Ácido 6,8-bis(benziltio)octanóico	Tratamento da leucémia mielóide aguda
Romanian	Acid 6,8-bis(benziltio)octanoic	Tratamentul leucemiei mieloide acute
Slovak	Kyselina 6,8-bis(benzylthio)oktánová	Liečba akútnej myeloickej leukémie
Slovenian	6,8-bis(benziltio)oktanojska kislina	Zdravljenje akutne mieloične levkemije
Spanish	Ácido 6,8-bis(benciltio)octanoico	Tratamiento de la leucemia mieloide aguda
Swedish	6,8-bis(bensyltio)oktansyra	Behandling av akut myeloisk leukemi
Norwegian	6,8-bis(benzyltio)oktansyre	Behandling av akutt myelogen leukemi
Icelandic	6,8-bis(bensýlþió)oktansýra	Meðferð við bráðu kyrningahvítblæði

[1] At the time of designation