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EMA/COMP/445179/2009 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

26 base single stranded phosphodiester DNA oligonucleotide for the treatment of acute myeloid leukaemia

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in March 2011 on request of the sponsor.

On 8 October 2009, orphan designation (EU/3/09/662) was granted by the European Commission to Antisoma Research Limited, United Kingdom, for 26 base single stranded phosphodiester DNA oligonucleotide 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) 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 is equivalent to a total of fewer than 101,000 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).

*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. This represents a population of 504,800,000 (Eurostat 2009).

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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 bone marrow transplantation (a complex procedure where the bone marrow of the patient is destroyed and replaced with bone marrow from a matched donor).

The sponsor has provided sufficient information to show that this medicine 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 improve the treatment of this condition when used in combination with existing treatments. 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?

26 base single stranded phosphodiester DNA oligonucleotide is made of multiple strands of nucleotides, the building blocks of DNA, that form a three-dimensional structure that attaches to a specific target. It is expected to work by attaching to nucleolin, a protein found in the nucleus of all cells that are reproducing, but also on the surface of cancer cells. Once attached to the surface of AML cells, this medicine is expected to go inside the cell and cause cell death.

What is the stage of development of this medicine?

The effects of 26 base single stranded phosphodiester DNA oligonucleotide 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, this medicine was not authorised anywhere in the EU for AML or designated as orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 July 2009 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:

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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	26 base single stranded phosphodiester DNA oligonucleotide	Treatment of acute myeloid leukaemia
Bulgarian	26 основен,единично усукан, фосфодиестерен ДНК олигонуклеотид	Лечение на остра миелоидна левкемия
Czech	Jednořetězcový fosfodiesterový DNA oligonukleotid o 26 bazích	Léčba akutní myeloidní leukémie
Danish	Enkeltstrenget phosphodiester-DNA-oligonukleotid med 26 baser	Behandling af akut myeloid leukæmi
Dutch	26 base enkelstrandig fosfodiëster DNA oligonucleotide	Behandeling van acute myeloïde leukemie
Estonian	26 alusega üheahelalise fosfodiester-DNA oligonukleotiid	Akuutse müeloidse leukeemia ravi
Finnish	26-emäksinen yksijuosteinen fosfodiesteri-DNA-oligonukleotidi	Akuutin myelooisen leukemian hoito
French	Oligonucléotide ADN à monobrin phosphodiester de 26 bases	Traitement de la leucémie aiguë myéloïde
German	26-Basen-, Einzelstrang-, Phosphodiester-, DNA-Oligonukleotid	Behandlung der akuten myeloischen Leukämie
Greek	Φωσφοδιεστεράση μονής ολιγονουκλεοτιδικής DNA αλύσου 26 βάσεων	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	26 bázisból álló, egyes szálú foszfodiészter DNS oligonukleotid	Akut myeloid leukaemia kezelése
Italian	Oligonucleotide fosfodiestere del DNA a catena singola a 26 basi	Trattamento della leucemia mieloide acuta
Latvian	26 bāzu vienpavediena fosfodiestera DNS oligonukleotīds	Akūtas mieloleikozes ārstēšana
Lithuanian	26 bazių DNR oligonukleotidas su viena fosfodiesterio jungtimi	Ūmios mieloleukozės gydymas
Maltese	Oligonukleotide phosphodiester tad-DNA b'katina waħda ta' 26 bażi	Kura tal-lewkimja mjelojda akuta
Polish	26 zasadowy oligonukleotyd fosfodiestrowy pojedynczego łańcucha DNA	Leczenie ostrej białaczki szpikowej
Portuguese	Oligonucleótido de DNA fosfodiéster monocatenado com 26 bases	Tratamento da leucémia mielóide aguda
Romanian	Oligonucleotid ADN fosfodiesteric monocatenar cu 26 de baze	Tratamentul leucemiei mieloide acute
Slovak	Jednovláknový fosfodiester- oligonukleotid DNA zložený z 26 báz	Liečba akútnej myeloickej leukémie
Slovenian	26 bazni enoverižni DNK olugonukleotid	Zdravljenje akutne mieloične levkemije
Spanish	Oligonucleótido ADN fosfodiéster monocatenario compuesto por 26 bases	Tratamiento de la leucemia mieloide aguda

[1] At the time of designation

Language	Active ingredient	Indication
Swedish	26-bas enkelsträngad fosfordiester-DNA-oligonukleotid	Behandling av akut myeloisk leukemi
Norwegian	26-base enkelttrådig fosfodiester DNA-oligonukleotid	Behandling av akutt myelogen leukemi
Icelandic	26 basa einþátta fosfótvíester DNA-ólígónúkleótíð	Meðferð við bráðu kyrningahvítblæði