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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Human monoclonal antibody against inhibitory killer cell lg-like receptors for the treatment of acute myeloid leukaemia

First publication	7 April 2009
Rev.1: transfer of sponsorship	23 April 2009
Rev.2: withdrawal from the Community Register	29 October 2012
Rev.3: administrative update	4 December 2013
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.	

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in October 2012 on request of the sponsor.

On 28 August 2006, orphan designation (EU/3/06/392) was granted by the European Commission to Novo Nordisk A/S, Denmark, for human monoclonal antibody against inhibitory killer cell lg-like receptors for the treatment of acute myeloid leukaemia.

The sponsorship was transferred to Innate Pharma S.A., France, in February 2009.

What is acute myeloid leukaemia?

Acute myeloid leukaemia (AML) is a cancer of the white blood cells. In this disease, the bone marrow 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 life-threatening because these immature cells take the place of the normal white blood cells. As a result, the patient's ability to fight diseases is reduced.

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

At the time of designation acute myeloid leukaemia affected approximately 1 in 10,000 people in the European Union (EU)[*]. This was below the threshold for orphan designation, which is 5 people in 10,000, and is equivalent to a total of around 56,000 people. This is based on the information provided by the sponsor and 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. The primary treatment for AML is chemotherapy (using medicines to kill cancer cells).

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that human monoclonal antibody against inhibitory killer cell Ig-like receptors might be of potential significant benefit for the treatment of acute myeloid leukaemia, mainly because it has a new mechanism of action and may be used in combination with other treatments. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

A monoclonal antibody is a protein that recognises and binds to a specific structure, either on a molecule or on a cell. In this case, human monoclonal antibody against inhibitory killer cell Ig-like receptors binds to a structure found on the surface of a type of cells that are part of the body's natural defence system (the immune system), called NK cells. According to the sponsor, by binding to these cells, the antibody will inhibit signals that prevent the NK cells from attacking cancer cells, thus subsequently contributing to the destruction of the cancer cells.

What is the stage of development of this medicine?

The effects of human monoclonal antibody against inhibitory killer cell Ig-like receptors were evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with acute myeloid leukaemia were initiated.

Human monoclonal antibody against inhibitory killer cell Ig-like receptors was not authorised anywhere worldwide for the treatment of acute myeloid leukaemia nor designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 July 2006 recommending the granting of this designation.

[*]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 25), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 468,900,000 (Eurostat 2006).

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;
- and either the rarity of the condition (affecting not more than five in 10,000 people in the Community) or the 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 the 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	Human monoclonal antibody against inhibitory killer cell Ig-like receptors	Treatment of acute myeloid leukaemia
Bulgarian	Човешко моноклонално антитяло срещу инхибиторните клетки убийци Ig-подобни рецептори	Лечение на остра миелоидна левкемия
Czech	Humánní monoklonální protilátky proti imunoglobulinům podobným receptorům inhibujícícím zabíječské buňky	Léčba akutní myeloidní leukémie
Danish	Humant, monoklonalt antistof mod inhibitorisk "killer-cell Ig-like receptor"	Behandling af akut myeloid leukæmi
Dutch	Humaan monoclonaal antilichaam gericht tegen “inhibitory killer cell Ig-like receptor”	Behandeling van acute myeloïde leukemie
Estonian	Inhibeeriva tappurraku Ig-tüüpi retseptori vastane inimese monoklonaalne antikeha	Akuutse müeloidse leukeemia ravi
Finnish	Ihmisen monoklonaalinen vasta-aine inhibitorista Ig-tyyppistä tappajasolureseptoria vastaan	Akuutin myelooisen leukemian hoito
French	Anticorps monoclonal humain dirigé contre le récepteur inhibiteur de type Ig des cellules tueuses	Traitement de la leucémie aiguë myéloïde
German	Humaner monoklonaler Antikörper gegen inhibitorische Ig-ähnliche Killerzellrezeptoren	Behandlung der akuten myeloischen Leukämie
Greek	ανθρώπινα μονοκλωνικά αντισώματα κατά του ανασταλτικού υποδοχέα τύπου I1g των φυσικών φονικών κυττάρων	Θεραπεία της οξείας μυελοειδούς λευχαιμίας
Hungarian	Humán monoklonális antitest gátló killer sejt Ig-szerű receptorok ellen	Akut myeloid leukaemia kezelése
Italian	Anticorpo monoclonale umano per il recettore inibitore immunoglobulino-simile (Ig-like) della cellula killer	Trattamento della leucemia mieloide acuta
Latvian	Cilvēka monoklonāla antiviela pret inhibitoro slepkavšūnu Ig-veidīgo receptoru	Akūtas mieloleikozes ārstēšana
Lithuanian	Monokloniniai žmogaus antikūnai prieš kilerių ląsteles, inhibuojantys Ig-panašius receptorius	Ūmios mieloleukozės gydymas
Maltese	Antikorp monoklonali uman kontra riċetturi inibituri tat-tip Ig taċ-ċelloli qattielin	Kura tal-lewkimja mjelojda akuta

[1] At the time of transfer of sponsorship

Language	Active Ingredient	Indication
Polish	Ludzkie przeciwciało monoklonalne przeciw Ig-podobnym receptorom hamującym komórki typu killer	Leczenie ostrej białaczki szpikowej
Portuguese	Anticorpo monoclonal humano contra os receptores Ig inibidores das células "killer"	Tratamento da leucemia mielóide aguda
Romanian	Anticorp uman monoclonal anti receptori inhibitori ai celulelor killer tip Ig	Tratamentul leucemiei mieloide acute
Slovak	Ľudská monoklonálna protilátka proti inhibujúcim Ig podobným receptorom buniek-zabijakov	Liečba akútnej myeloickej leukémie
Slovenian	Humano monoklonsko protitelo proti inhibicijskemu Ig-podobnemu receptorju celic ubijalk	Zdravljenje akutne mieloične levkemije