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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human monoclonal antibody to human IL-1beta of the IgG1/K class for the treatment of systemic-onset juvenile idiopathic arthritis

First publication	29 July 2008
Rev.1: withdrawal from the Community Register	5 March 2012
Rev.2: administrative update	30 April 2012
Rev.3: administrative update	16 December 2013
Rev.4: sponsor's change of address	5 February 2015

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Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in February 2012 on request of the sponsor.

On 4 February 2008, orphan designation (EU/3/08/527) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for recombinant human monoclonal antibody to human IL-1beta of the IgG1/K class for the treatment of systemic-onset juvenile idiopathic arthritis.

What is systemic-onset juvenile idiopathic arthritis?

Arthritis is defined as the inflammation of the joints; it results in pain and limitation of the normal function of the affected joints. Sometimes arthritis is a chronic condition where inflammation is of unknown origin, probably related to the damage caused by the immune system against the body tissues of the joints. There are many forms of arthritis that differ in terms of number and type of joints affected and the age of onset. Systemic onset juvenile idiopathic arthritis is a type of arthritis characterised by arthritis that starts before the age of 16 and that is associated with other manifestations not exclusive to the joints, such as rash and fever.

Systemic-onset juvenile arthritis is a chronically debilitating condition because its symptoms are recurrent and cause disability and can have long term consequences.

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

At the time of designation, systemic-onset juvenile idiopathic arthritis affected approximately 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 25,000 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?

Several products with anti-inflammatory activity were authorised for idiopathic juvenile arthritis in some Member States in the Community at the time of submission of the application for orphan drug designation. In addition, there are other products not specifically authorised but used, mainly those aimed at suppressing mechanisms involved in the body's immune response.

Recombinant human monoclonal antibody to human IL-1beta of the IgG1/K class may be of potential significant benefit because it acts via a new mechanism of action compared to authorised products and it could possibly improve the results of the treatment by improving the symptoms of the disease. The assumption will have to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Interleukin 1 (IL-1) has been identified as one of the substances responsible for rise in body temperature and systemic inflammation. There is scientific data that links IL-1 and systemic onset juvenile idiopathic arthritis, particularly with regards to the presence of fever and inflammation.

Antibodies are proteins in the body that target and bind specific structures that can be either circulating in the blood stream or on the surface of cells. The product is an antibody able to bind to IL-1 and to block its activity in the body.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, no clinical trials in patients with systemic-onset juvenile idiopathic arthritis were initiated.

Recombinant human monoclonal antibody to human IL-1beta of the IgG1/K class was not authorised anywhere worldwide for systemic-onset juvenile idiopathic arthritis or 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 10 January 2008 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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 502,800,000 (Eurostat 2008).

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	Recombinant human monoclonal antibody to human IL-1beta of the IgG1/K class	Treatment of systemic-onset juvenile idiopathic arthritis
Bulgarian	Рекомбинантно човешко моноклонално антитяло от клас lgG1/K срещу човешки IL-1бета	Лечение на ювенилен идиопатичен артрит със системно начало
Czech	Rekombinantní lidská monoklonální protilátka třídy IgG1/K proti humánnímu IL-1 beta	Léčba systémové získané formy idiopatické juvenilní artritidy
Danish	Recombinant humant monoklonalt antistof mod human IL-1beta af IgG1/K klasse	Behandling af idiopatisk artritis med systemisk debut hos unge
Dutch	Recombinant humaan monoclonaal antilichaam tegen humaan IL-1 beta van de klasse IgG1/K	Behandeling van juveniele idiopathische arthritis met systemische aanvang
Estonian	Inimese IgG1/K klassi IL-1 beeta vastane rekombinantne inimese monoklonaalne antikeha	Süsteemse algusega juveniilse idiopaatilise artriidi ravi
Finnish	DNA-yhdistelmätekniikalla tuotettu humaani monoklonaalinen IgG1/K luokan IL1- beeta vasta-aine	Nuorten systeemisesti puhjenneen idiopaattisen artriitin hoito
French	Anticorps monoclonal recombinant humain anti-IL1 bêta de la classe des IgG1/K	Traitement de l'arthrite chronique à début juvénile
German	Rekombinanter humaner monoklonaler Antikörper gegen humanes IL-1 beta der IgG1/K Klasse	Behandlung der juvenilen idiopathischen Arthritis mit systemischem Beginn
Greek	Ανασυνδυασμένο ανθρώπινο μονοκλωνικό αντίσωμα τύπου IgG1/K κατά της ανθρώπειας IL-1 β	Θεραπεία της νεανικής ιδιοπαθούς αρθρίτιδας που παρουσιάζει συστηματικές εκδηλώσεις
Hungarian	Az IgG1/K osztályba tartozó humán IL 1béta elleni rekombináns humán monoklonális antitest	Szisztémás juvenilis idiopathiás arthritis kezelése
Italian	Anticorpo monoclonale umano ricombinante della classe IgG1/K, anti Interleuchina-1beta	Trattamento dell'artrite giovanile idiopatica ad insorgenza sistemica
Latvian	Rekombinanta lgG1/K klases monoklonāla antiviela pret cilvēka lL-1 beta	Sistēmiska juvenila idiopātiskā artrīta ārstēšana
Lithuanian	Rekombinantinis žmogaus monokloninis antikūnis prieš žmogaus IL – 1 beta, IgG1/K klasės	Sisteminio jaunatvinio idiopatinio artrito gydymas
Maltese	Anti-korp monoklonali uman rikombinanti għall-IL-1beta uman tal-klassi IgG1/K	Kura ta' l-artrite idjopatika taż-żogħżija b'bidu sistemiku

[1] At the time of designation

Language	Active ingredient	Indication
Polish	Ludzkie rekombinowane przeciwciało monoklonalne klasy IgG1/K przeciwko ludzkiej interleukinie 1 beta (IL-1beta)	Leczenie pacjentów z młodzieńczym idiopatycznym zapaleniem stawów o początku uogólnionym
Portuguese	Anticorpo monoclonal humano recombinante da classe IgG1/K para a IL-1beta humana	Tratamento da artrite idiopática juvenil forma sistémica
Romanian	Anticorp monoclonal uman recombinant anti-interleukină-1beta umană din clasa IgG1/K	Tratamentul artritei idiopatice juvenile,cu debut sistemic
Slovak	Rekombinantná humánna monoklonálna protilátka triedy IgG1/K proti humánnemu IL-1beta	Liečba juvenilnej idiopatickej artritídy so systémovým začiatkom
Slovenian	rekombinantno humano monoklonsko protitelo proti humanemu IL-1beta iz podrazreda IgG1/K	Zdravljenje sistemske oblike juvenilnega idiopatskega artritisa
Spanish	Anticuerpo monoclonal recombinante humano anti IL-1beta humana de la clase IgG1/K	Tratamiento de la artritis idiopática juvenil de comienzo sistémico
Swedish	Human rekombinant monoklonal antikropp mot humant IL-1 beta i IgG1/K klassen	Behandling av idiopatisk artrit med systemisk-debut hos unga
Norwegian	Rekombinant humant monoklonalt antistoff av type IgG1/K mot human IL-1beta	Behandling av juvenil idiopatisk artritt med systemisk opprinnelse
Icelandic	Raðbrigða manna einstofna mótefni gegn manna IL-1 beta í IgG1/K flokkinum	Til meðferðar við barnaliðagigt með fjölkerfa upphafi