



**COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS**

**PUBLIC SUMMARY OF  
POSITIVE OPINION FOR ORPHAN DESIGNATION  
OF**

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

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.

**What are the methods of treatment 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. This will be necessary to maintain the orphan status.

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

Based on the information provided by the sponsor and previous knowledge of the Committee, systemic-onset juvenile idiopathic arthritis was considered to affect approximately 0.5 in 10,000 persons in the European Union, which, at the time of designation, corresponded to about 25,000 persons.

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\* Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union (EU 27), Norway, Iceland and Lichtenstein. This represents a population of 498,000,000 (Eurostat 2006). This estimate is based on available information and calculations presented by the sponsor at the time of the application.

**How is this medicinal product expected to act?**

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 medicinal product?**

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.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 10 January 2008 a positive opinion recommending the grant of the above-mentioned designation.

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Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products which were considered for 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 will be necessary before this product can be granted a marketing authorisation.

**For more information:**

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**Translations of the active ingredient and indication in all EU languages  
and Norwegian and Icelandic**

<b>Language</b>	<b>Active Ingredient</b>	<b>Indication</b>
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 IgG1/K срещу човешки IL-1βета	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 arthritis 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 ανθρώπινης IL-1 β	Θεραπεία της νεανικής ιδιοπαθούς αρθρίτιδας που παρουσιάζει συστηματικές εκδηλώσεις
Hungarian	Az IgG1/K osztályba tartozó humán IL 1βéta 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 IgG1/K klases monoklonāla antivielā pret cilvēka IL-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-żoghżija b'bidu sistemiku
Polish	Ludzkie rekombinowane przeciwcało 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-1 beta 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-1 beta	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