30 January 2015
EMA/COMP/663189/2012 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Canakinumab for the treatment of tumour necrosis factor receptor-associated periodic syndrome

First publication	13 December 2012
Rev.1: sponsor's change of address	30 January 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.	

On 8 November 2012, orphan designation (EU/3/12/1071) was granted by the European Commission to Novartis Europharm Limited, United Kingdom, for canakinumab for the treatment of tumour necrosis factor receptor-associated periodic syndrome.

What is tumour necrosis factor receptor-associated periodic syndrome?

Tumour necrosis factor receptor-associated periodic syndrome (TRAPS) is an inflammatory disease in which patients have recurrent episodes of fever, rash and pain in various parts of the body, including the eyes, joints, muscles and the abdomen. These symptoms are caused by excessive inflammation as a result of a mutation in the gene for proteins called 'tumour necrosis factor (TNF) receptors', which play an important role in the inflammatory process.

TRAPS is a long-term disease that is debilitating due to its recurrent symptoms and the development of kidney complications.

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

At the time of designation, TRAPS affected approximately 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of around 500 people*, and is below the ceiling for orphan

*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 509,000,000 (Eurostat 2012).

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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?

At the time of the orphan designation, no satisfactory treatments for TRAPS were authorised in the EU. Anti-inflammatory medicines such as NSAIDs (non-selective non-steroidal anti-inflammatory drugs) and glucocorticosteroids were used to manage the symptoms of the disease.

How is this medicine expected to work?

Patients with TRAPS have excessive activity of inflammatory proteins, including a protein called 'interleukin-1 beta', which is involved in causing the inflammation in these patients. Canakinumab is a monoclonal antibody (a type of protein) designed to attach to interleukin-1 beta and block its activity, thereby reducing the inflammation associated with the disease.

Canakinumab is authorised in the EU for the treatment of another inflammatory disease cryopyrin-associated periodic syndrome (CAPS), in which it also targets interleukin-1 beta.

What is the stage of development of this medicine?

The effects of canakinumab have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with canakinumab in patients with TRAPS were ongoing.

At the time of submission, canakinumab was not authorised anywhere in the EU for TRAPS. Orphan designation has been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 October 2012 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 substance	Indication
English	Canakinumab	Treatment of tumour necrosis factor receptor-associated periodic syndrome
Bulgarian	Канакинумаб	Лечение на TNF-рецептор асоцииран периодичен синдром (TRAPS)
Czech	Canakinumabum	Léčba TNF-receptor asociovaného periodického syndromu (TRAPS)
Danish	Canakinumab	Behandling af TNF-receptor-associeret periodisk syndrom (TRAPS)
Dutch	Canakinumab	Behandeling van TNF- receptor geassocieerd periodiek syndroom (TRAPS)
Estonian	Kanakinumab	TNF retseptoriga seotud perioodilise sündroomi (TRAPS) ravi
Finnish	Kanakinumabi	TNF-reseptoriin liittyvän jaksottaisen oireyhtymän (TRAPS) hoito
French	Canakinumab	Syndrome périodique associé aux mutations du récepteur du TNF (TRAPS)
German	Canakinumab	Behandlung des TNF-Rezeptor-assoziierten periodischen Syndroms (TRAPS)
Greek	Καναkινουμάμπη	Θεραπεία του σχετιζόμενου με τον υποδοχέα του παράγοντα νέκρωσης των όγκων περιοδικού συνδρόμου (TRAPS)
Hungarian	Kanakinumab	TNF-receptor-asszociált periodikus szindróma (TNF-Receptor-Associated Periodic Syndrome) (TRAPS) kezelésé
Italian	Canakinumab	Trattamento della sindrome periodica associata al recettore del TNF (TRAPS)
Latvian	Kanakinumabs	Ar TNF receptoriem saistītā periodiskā sindroma (TRAPS) ārstēšanai
Lithuanian	Kanakinumabas	Su naviko nekrozės faktoriaus receptoriumi susijusiu periodinio sindromo gydymas
Maltese	Canakinumab	Kura tas-sindrome perjodiku assoċjat mar-riċettur għall-fattur tan-nekrosi tat-tumur
Polish	Kanakinumab	Leczenie okresowych zespołów związanych z receptorem dla czynnika martwicy nowotworów (z ang. tumour necrosis factor – TNF)
Portuguese	Canacinumab	Tratamento da síndrome periódica associada ao receptor do TNF (TRAPS)
Romanian	Canakinumab	Tratamentul sindromului periodic asociat receptorului TNF (TRAPS)
Slovak	Kanakinumab	Liečba periodického syndrómu spojeného s receptorom TNF (TRAPS)
Slovenian	Kanakinumab	zdravljenje periodičnega vročinskega sindroma povezanega z receptorjem za TNF (TNF-receptor-associated periodic syndrome, TRAPS)
Spanish	Canakinumab	Tratamiento del síndrome periódico asociado al receptor de TNF (TRAPS)

[1] At the time of designation

Language	Active substance	Indication
Swedish	Kanakinumab	Behandling av TNF-receptor-associerat periodiskt syndrom (TRAPS)
Norwegian	Kanakinumab	Behandling av TNF reseptor-assosiert periodisk syndrom (TRAPS)
Icelandic	Canakinumabi	Meðferð við heilkenni, sem tengist TNF-viðtökum, með lotubundnum einkennum