EMA/644005/2011

European Medicines Agency decision

P/208/2011

of 2 September 2011

on the acceptance of a modification of an agreed paediatric investigation plan for canakinumab (EMEA-000060-PIP01-07-M03) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

Disclaimer

This Decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

Only the English text is authentic.

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on the acceptance of a modification of an agreed paediatric investigation plan for canakinumab (EMEA-000060-PIP01-07-M03) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/27/2008 issued on 23 May 2008, the decision P/96/2010 issued on 4 June 2010,

Having regard to the application submitted by Novartis Europharm Limited on 2 May 2011 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 15 July 2011, in accordance with Article 22 of Regulation (EC) No 1901/2006,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the acceptance of changes to the agreed paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[1] OJ L 378, 27.12.2006, p.1.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for canakinumab , powder for solution for injection, powder and solvent for solution for injection, solution for injection, subcutaneous use, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2,

This decision is addressed to Novartis Europharm Limited, Wimblehurst Road, Horshham, West Sussex RH12 5AB, United Kingdom.

Done at London, 2 September 2011

For the European Medicines Agency
Andreas Pott
Acting Executive Director
(Signature on file)

EMA/PDCO/386730/2011

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-000060-PIP01-07-M03

Scope of the application

Active substance(s):

Canakinumab

Condition(s):

Cryopyrin Associated Periodic Syndromes (CAPS) including:

Familial Cold Autoinflammatory Syndrome (FCAS) / Familial Cold Urticaria (FCU),

Muckle-Wells Syndrome (MWS),

Neonatal-Onset Multisystem Inflammatory Disease (NOMID) / Chronic Infantile Neurological, Cutaneous, Articular Syndrome (CINCA)

Pharmaceutical form(s):

Powder for solution for injection

Powder and solvent for solution for injection

Solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Novartis Europharm Limited

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Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Novartis Europharm Limited submitted to the European Medicines Agency on 2 May 2011 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/27/2008 issued on 23 May 2008, and the decision P/96/2010 issued on 4 June 2010.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 18 May 2011.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

   The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annex(es) and appendix.

London, 15 July 2011

On behalf of the Paediatric Committee
Dr Daniel Brasseur, Chairman
(Signature on file)

Annex I

The measures and timelines of the agreed paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver

1. Condition(s)

Cryopyrin Associated Periodic Syndromes (CAPS) including:

- Familial Cold Autoinflammatory Syndrome (FCAS) / Familial Cold Urticaria (FCU),
- Muckle-Wells Syndrome (MWS),
- Neonatal-Onset Multisystem Inflammatory Disease (NOMID) / Chronic Infantile Neurological, Cutaneous, Articular Syndrome (CINCA).

2. Waiver

2.1. Condition

Cryopyrin Associated Periodic Syndromes (CAPS) including:

- Familial Cold Autoinflammatory Syndrome (FCAS) / Familial Cold Urticaria (FCU),
- Muckle-Wells Syndrome (MWS),
- Neonatal-Onset Multisystem Inflammatory Disease (NOMID) / Chronic Infantile Neurological, Cutaneous, Articular Syndrome (CINCA).

The waiver applies to:

- Newborn infants (from birth to less than 28 days),
- for powder for solution for injection, powder and solvent for solution for injection, and solution for injection, for subcutaneous use,
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

3. Paediatric Investigation Plan

3.1. Condition to be investigated

Cryopyrin Associated Periodic Syndromes (CAPS) including:

- Familial Cold Autoinflammatory Syndrome (FCAS) / Familial Cold Urticaria (FCU),
- Muckle-Wells Syndrome (MWS),
- Neonatal-Onset Multisystem Inflammatory Disease (NOMID) / Chronic Infantile Neurological, Cutaneous, Articular Syndrome (CINCA).

3.1.1. Indication targeted by the PIP

Treatment of Cryopyrin-Associated Periodic Syndromes (CAPS) in paediatric patients of infant age and older, including:

- Familial Cold Autoinflammatory Syndrome (FCAS) / Familial Cold Urticaria (FCU),
- Muckle-Wells Syndrome (MWS),
- Neonatal-Onset Multisystem Inflammatory Disease (NOMID) / Chronic Infantile Neurological, Cutaneous, Articular Syndrome (CINCA).

3.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From one month to less than 18 years of age.

3.1.3. Pharmaceutical form(s)

Powder for solution for injection

Powder and solvent for solution for injection

Solution for injection

3.1.4. Studies

Area	Number of studies	Description
Quality	0	Not applicable.
Non-clinical	1	1. Juvenile toxicity study in mice.
Clinical	5	2. Open-label, dose titration study to assess the clinical efficacy, safety, pharmacokinetics and pharmacodynamics in paediatric and adult patients > 4 years with NALP-3 mutations. 3. Three-part, multicentre study of 48 weeks duration, with a randomised, double-blind, placebo controlled, withdrawal design in Part II to assess efficacy, safety and tolerability of canakinumab administered as a subcutaneous (S.C.) injection in paediatric and adult patients with Muckle-Wells Syndrome (MWS). 4. Multi-centre, open-label, 24-month study to establish the safety, tolerability, efficacy, pharmacokinetics and pharmacodynamics of canakinimab in patients with Neonatal-Onset Multisystem Inflammatory Disease (NOMID)/Chronic Infantile, Neurologic, Cutaneous, Articular Syndrome (CINCA). 5. Open-label, long-term safety and efficacy study of canakinumab administered for at least 6 months in patients with the following cryopyrin-associated periodic syndromes (CAPS): Familial Cold Autoinflammatory Syndrome (FCAS), Muckle-Wells Syndrome (MWS), or Neonatal Onset Multisystem Inflammatory Disease (NOMID). 6. A 52-week, open label study to collect efficacy, safety and tolerability data in infants with CAPS.

4. Follow-up, completion and deferral of PIP

Concerns on potential long term safety issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By December 2014
Deferral for one or more studies contained in the paediatric investigation plan:	Yes