



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/153837/2015

European Medicines Agency decision

P/0057/2015

of 1 April 2015

on the agreement of a paediatric investigation plan and on the granting of a waiver for canakinumab (Ilaris), (EMA-000060-PIP04-14) 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 agreement of a paediatric investigation plan and on the granting of a waiver for canakinumab (Ilaris), (EMA-000060-PIP04-14) 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 726/2004¹,

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 Agency²,

Having regard to the application submitted by Novartis Europharm Ltd on 11 April 2014 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 13 February 2015, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a waiver.
- (2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.
- (3) It is therefore appropriate to adopt a decision granting a waiver.

¹ OJ L 378, 27.12.2006, p.1.

² OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for canakinumab (Ilaris), solution for injection, powder for solution for injection, powder and solvent for solution for injection, subcutaneous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A waiver for canakinumab (Ilaris), solution for injection, powder for solution for injection, powder and solvent for solution for injection, subcutaneous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This agreed PIP covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decisions P/27/2008, EMEA-000060-PIP01-07 issued on 23 May 2008 including subsequent modifications thereof; P/131/2009, EMEA-000060-PIP02-08 issued on 15 July 2009, including subsequent modifications thereof.

Article 4

This decision is addressed to Novartis Europharm Ltd, Frimley Business Park, GU16 7SR – Camberley, United Kingdom.

Done at London, 1 April 2015

For the European Medicines Agency
Jordi Llinares Garcia
Head of Division (ad interim)
Human Medicines Research and Development Support
(Signature on file)



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/757761/2014

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a waiver

EMA-000060-PIP04-14

Scope of the application

Active substance(s):

Canakinumab

Invented name:

Ilaris

Condition(s):

Treatment of familial Mediterranean fever

Treatment of hyperimmunoglobulin D syndrome

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Solution for injection

Powder for solution for injection

Powder and solvent for solution for injection

Route(s) of administration:

Subcutaneous use

Name / corporate name of the PIP applicant:

Novartis Europharm Ltd

Information about the authorised medicinal product:

See Annex II



Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Novartis Europharm Ltd submitted for agreement to the European Medicines Agency on 11 April 2014 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 16 July 2014.

Supplementary information was provided by the applicant on 21 November 2014. The applicant proposed modifications to the paediatric investigation plan.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

- to agree the paediatric investigation plan in accordance with Article 18 of said Regulation;
- to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset(s) of the paediatric population, and with Article 11 (1) (c) of said Regulation, on the grounds that that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

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

2. 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 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 annexes and appendix.

London, 13 February 2015

On behalf of the Paediatric Committee
Dr Dirk Mentzer, Chairman
(Signature on file)

Annex I

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

1. Waiver

1.1. Condition:

Treatment of familial Mediterranean fever

The waiver applies to:

- preterm newborn infants and term newborn infants from birth to less than 28 days;
- solution for injection, powder for solution for injection, powder and solvent for solution for injection, subcutaneous use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subsets.

1.2. Condition:

Treatment of hyperimmunoglobulin D syndrome

The waiver applies to:

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

2. Paediatric investigation plan

2.1. Condition:

Treatment of familial Mediterranean fever

2.1.1. Indication(s) targeted by the PIP

Treatment of familial Mediterranean fever, in paediatric patients from 28 days to less than 18 years of age who are non-responders or intolerant to effective therapeutic doses of colchicine or for whom treatment with colchicine is contraindicated.

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

From 28 days to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Solution for injection.

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	Study 1 Development of 1:1 dilution procedure with commercially available diluents
Non-clinical studies	0	Not applicable.
Clinical studies	1	Study 2 Randomized, multicentre, double-blind, placebo controlled study to evaluate efficacy and safety of canakinumab compared to placebo in children from 2 years to less than 18 years of age (and adults), and to collect efficacy and safety data in children from 28 days to less than 2 years of age with Hereditary Periodic Fevers (familial Mediterranean fever, hyperimmunoglobulin D syndrome and TNF receptor associated periodic syndrome) (CACZ885N2301).
Extrapolation, modelling and simulation studies	0	Not applicable.
Other studies	0	Not applicable
Other measures	0	Not applicable.

2.2. Condition:

Treatment of Hyperimmunoglobulin D syndrome

2.2.1. Indication(s) targeted by the PIP

Treatment of Hyperimmunoglobulin D syndrome in patients from 28 days to less than 18 years of age.

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

From 28 days to less than 18 years of age.

2.2.3. Pharmaceutical form(s)

Solution for injection.

2.2.4. Measures

Area	Number of measures	Description
Quality-related studies	1	Study 1 Development of 1:1 dilution procedure with commercially available diluents
Non-clinical studies	0	Not applicable.
Clinical studies	1	Study 2 Randomized, multicentre, double-blind, placebo controlled study to evaluate efficacy and safety of canakinumab compared to placebo in children from 2 years to less than 18 years of age (and adults), and to collect efficacy and safety data in children from 28 days to less than 2 years of age with Hereditary Periodic Fevers (familial Mediterranean fever, hyperimmunoglobulin D syndrome and TNF receptor associated periodic syndrome) (CACZ885N2301).
Extrapolation, modelling and simulation studies	0	Not applicable.
Other studies	0	Not applicable.
Other measures	0	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By March 2016
Deferral for one or more measures contained in the paediatric investigation plan:	No

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of 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)

Authorised indication(s):

- Ilaris is indicated for the treatment of Cryopyrin-Associated Periodic Syndromes (CAPS) in adults, adolescents and children aged 2 years and older with body weight of 7.5 kg or above, including:
 - Muckle-Wells Syndrome (MWS),
 - Neonatal-Onset Multisystem Inflammatory Disease (NOMID) / Chronic Infantile Neurological, Cutaneous, Articular Syndrome (CINCA),
 - Severe forms of Familial Cold Autoinflammatory Syndrome (FCAS) / Familial Cold Urticaria (FCU) presenting with signs and symptoms beyond cold-induced urticarial skin rash.

2. Treatment of juvenile idiopathic arthritis

Authorised indication(s):

- Ilaris is indicated for the treatment of active Systemic Juvenile Idiopathic Arthritis (SJIA) in patients aged 2 years and older who have responded inadequately to previous therapy with non-steroidal anti-inflammatory drugs (NSAIDs) and systemic corticosteroids. Ilaris can be given as monotherapy or in combination with methotrexate.

3. Treatment of primary gout (excluding Lesh-Nyhan syndrome and other secondary forms of gout)

Authorised indication(s):

- Ilaris is indicated for the symptomatic treatment of adult patients with frequent gouty arthritis attacks (at least 3 attacks in the previous 12 months) in whom non-steroidal anti-inflammatory drugs (NSAIDs) and colchicine are contraindicated, are not tolerated, or do not provide an adequate response, and in whom repeated courses of corticosteroids are not appropriate.

Authorised pharmaceutical form(s):

Powder for solution for injection

Powder and solvent for solution for injection

Authorised route(s) of administration:

Subcutaneous use.