



European Medicines Agency

Doc. Ref. EMA/129196/2010  
P/28/2010

**EUROPEAN MEDICINES AGENCY DECISION**

**of 5 March 2010**

**on the agreement of a Paediatric Investigation Plan and on the granting of a deferral and on the granting of a waiver for dalcetrapib (EMEA-000580-PIP01-09) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council as amended**

(ONLY THE ENGLISH TEXT IS AUTHENTIC)

*DISCLAIMER: This Decision does not entitle to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006, as amended.*

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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 as amended and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004<sup>1</sup>,

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<sup>2</sup>,

Having regard to the application submitted by Roche Registration Limited on 24 April 2009 under Article 16(1) of Regulation (EC) No 1901/2006 as amended also requesting a waiver under Article 13 of said Regulation and a deferral under Article 20 of said Regulation,

Having regard to the Opinion of the Paediatric Committee of the European Medicines Agency, issued on 15 January 2010, in accordance with Article 18 of Regulation (EC) No 1901/2006 as amended, and Article 13 of said Regulation and Article 21 of said Regulation,

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

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 deferral 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 deferral.
- (4) It is therefore appropriate to adopt a Decision granting a waiver.

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<sup>1</sup> OJ L 378, 27.12.2006, p.1

<sup>2</sup> OJ L 136, 30.4.2004, p. 1

HAS ADOPTED THIS DECISION:

*Article 1*

A Paediatric Investigation Plan for dalcetrapib, film-coated tablet, oral 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 deferral for dalcetrapib, film-coated tablet, oral 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*

A waiver for dalcetrapib, film-coated tablet, oral 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 4*

This decision is addressed to Roche Registration Limited, 6 Falcon Way, Shire Park, AL7 1TW - Welwyn Garden City, United Kingdom.

Done at London, 5 March 2010

For the European Medicines Agency  
Thomas Lönngren  
Executive Director

(Signature on file)



European Medicines Agency  
*Pre-authorisation Evaluation of Medicines for Human Use*

Doc. Ref. EMA/PDCO/17698/2010  
EMA-000580-PIP01-09

**OPINION OF THE PAEDIATRIC COMMITTEE ON THE AGREEMENT OF  
A PAEDIATRIC INVESTIGATION PLAN AND A DEFERRAL AND A WAIVER**

**Scope of the application**

Active substance(s):

Dalcetrapib

Condition(s):

Lipoprotein deficiency

Pharmaceutical form(s):

Film-coated tablet

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Roche Registration Limited

**Basis for opinion**

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Roche Registration Limited submitted for agreement to the European Medicines Agency on 24 April 2009 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 28 May 2009.

Supplementary information was provided by the applicant on 09 November 2009.

## 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 deferral in accordance with Article 21 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)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population.

The Icelandic and the Norwegian Paediatric Committee members agree 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 Agency, together with its annex(es) and appendix.

London, 15 January 2010

On behalf of the Paediatric Committee  
Dr Daniel Basseur, 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**

## **A. CONDITION(S)**

Lipoprotein deficiency

## **B. WAIVER**

- **Condition**

Lipoprotein deficiency

The waiver applies to:

- Preterm newborn infants, Term newborn infants (from birth to less than 28 days), Infants and toddlers (from 28 days to less than 24 months), Prepubertal Children (from 2 years to Tanner Stage I)
- for film-coated tablet, oral use
- on the grounds that the specific medicinal product is likely to be ineffective.

## **C. PAEDIATRIC INVESTIGATION PLAN**

- **Condition to be investigated**

Lipoprotein deficiency

- **Proposed PIP indication**

Treatment of low high density lipoprotein-cholesterol (HDL-C) in children with Familial Hypercholesterolaemia (FH) and low HDL-C or Familial HDL-C deficiency

- **Subset(s) of the paediatric population concerned by the paediatric development**

Pubertal and postpubertal children (Tanner stage II-V) less than 18 years of age.

- **Formulation(s)**

Film-coated tablet, oral use.

- **Studies**

<b>Area</b>	<b>Number of studies</b>	<b>Description</b>
Quality	1	Development of a film-coated tablet for oral use with lower age-appropriate strength(s).
Non-clinical	0	Not applicable.
Clinical	3	<p>Open label, 3-treatment, ascending dose trial to evaluate the pharmacodynamic effects of short-term dalcetrapib treatment in patients with Familial HDL-C deficiency or Familial Hypercholesterolaemia (FH) and low HDL-C in pubertal and postpubertal children (Tanner stage II-V) less than 18 years of age.</p> <p>Multi-center, double-blind, randomized, placebo-controlled trial to evaluate the safety and efficacy of dalcetrapib by measuring vascular function and levels of HDL-C in adolescents with FH and low HDL-C or Familial HDL-C deficiency and high global risk of cardiovascular disease in pubertal and postpubertal children (Tanner stage II-V) less than 18 years of age.</p> <p>Multi-center, double-blind, randomized, placebo-controlled 2-year extension trial to evaluate the safety and efficacy of dalcetrapib by measuring vascular function and levels of HDL-C in adolescents with FH and low HDL-C or Familial HDL-C deficiency and high global risk of cardiovascular disease in pubertal and postpubertal children (Tanner stage II-V) less than 18 years of age.</p>

<b>Measures to address long term follow-up of potential safety issues in relation to paediatric use:</b>	<b>Yes</b>
<b>Date of completion of the paediatric investigation plan:</b>	<b>By October 2020</b>
<b>Deferral for some or all studies contained in the paediatric investigation plan:</b>	<b>Yes</b>