EMA/887311/2018

# European Medicines Agency decision

P/0050/2019

of 29 January 2019

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for ozanimod (EMEA-001710-PIP04-17) 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.**

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
**Telephone** +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
**Contact EMA** www.ema.europa.eu/contact

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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 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 application submitted by Celgene Europe Limited on 14 December 2017 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 14 December 2018, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation 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 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.

[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 ozanimod, capsule, hard, age-appropriate oral liquid dosage form, 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 ozanimod, capsule, hard, age-appropriate oral liquid dosage form, 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 ozanimod, capsule, hard, age-appropriate oral liquid dosage form, 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 Celgene Europe Limited, 1 Longwalk Road, Stockley Park, UB11 1DB - Uxbridge, United Kingdom.

EMA/PDCO/659051/2018
London, 14 December 2018

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

EMEA-001710-PIP04-17

## Scope of the application

**Active substance(s):**

Ozanimod

**Condition(s):**

Treatment of Crohn's disease

**Pharmaceutical form(s):**

Capsule, hard

Age-appropriate oral liquid dosage form

**Route(s) of administration:**

Oral use

**Name/corporate name of the PIP applicant:**

Celgene Europe Limited

## Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Celgene Europe Limited submitted for agreement to the European Medicines Agency on 14 December 2017 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 23 January 2018.

Supplementary information was provided by the applicant on 7 September 2018. 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 17(1) 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)(c) of said Regulation, on the grounds 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 annex and appendix.

# 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 Crohn's disease

The waiver applies to:

- the paediatric population from birth to less than 2 years;
- capsule, hard, age-appropriate oral liquid dosage form, oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

# 2. Paediatric investigation plan

## *2.1. Condition:*

Treatment of Crohn's disease

### 2.1.1. Indication(s) targeted by the PIP

Treatment of moderate to severe active Crohn's disease

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

From 2 years to less than 18 years of age.

### 2.1.3. Pharmaceutical form(s)

Capsule, hard

Age-appropriate oral liquid dosage form

### 2.1.4. Measures

| Area | Number of measures | Description |
|---|---|---|
| Quality-related studies | 1 | **Study 1**<br>Development of an age-appropriate oral liquid dosage form. |
| Non-clinical studies | 1 | **Study 2**<br>33-Day oral immunotoxicity study in juvenile Sprague-Dawley rats. |

| | | |
|---|---|---|
| Clinical studies | 6 | **Study 3 (RPC01-3201)**<br><br>Multicentre, randomized, double-blind, placebo-controlled study of ozanimod as induction therapy for adolescents from 12 years to less than 18 years (and adults) with moderately to severely active Crohn's disease (CD).<br><br>**Study 4 (RPC01-3202)**<br><br>Multicentre, randomized, double-blind, placebo-controlled study of ozanimod as induction therapy for adolescents from 12 years to less than 18 years (and adults) with moderately to severely active Crohn's disease (CD).<br><br>**Study 5 (RPC01-3203)**<br><br>Multicentre, randomized, double-blind, placebo-controlled study of ozanimod as maintenance therapy for adolescents from 12 years to less than 18 years (and adults) with moderately to severely active Crohn's disease (CD).<br><br>**Study 6 (RPC01-3204)**<br><br>Open-label, multicentre extension study to evaluate safety and efficacy of ozanimod in adolescents from 12 years to less than 18 years (and adults) with moderately to severely active Crohn's disease (CD).<br><br>**Study 7 (CD-PED-PK)**<br><br>Open-label pharmacokinetic study in children from 2 years to less than 18 years with moderately to severely active Crohn's disease (CD).<br><br>**Study 8 (CD-PED-PH3)**<br><br>Safety and efficacy study with an open-label induction phase and a multicentre, randomised, double-blind, placebo controlled withdrawal period in children and adolescents from 2 years to less than 18 years with moderately to severely active Crohn’s disease (CD). |
| Extrapolation, modelling and simulation studies | 1 | **Study 9**<br><br>Modelling and simulation study, to evaluate use of ozanimod in children from 2 years to less than 18 years of age with moderately to severely active Crohn’s disease (CD). |
| 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 September 2026 |
| Deferral for one or more measures contained in the paediatric investigation plan: | Yes |