EMA/424132/2015

European Medicines Agency decision

P/0157/2015

of 10 July 2015

on the agreement of a paediatric investigation plan and on the granting of a waiver for momelotinib (EMEA-001656-PIP01-14) 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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An agency of the European Union

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on the agreement of a paediatric investigation plan and on the granting of a waiver for momelotinib (EMEA-001656-PIP01-14) 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 726/2004[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 Gilead Sciences International Ltd on 6 August 2014 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 22 May 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 momelotinib, film-coated tablet, age-appropriate oral 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 waiver for momelotinib, film-coated tablet, age-appropriate oral 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

This decision is addressed to Gilead Sciences International Ltd, Flowers Building, Granta Park, Abington, CB21 6GT – Cambridge, United Kingdom.

Done at London, 10 July 2015

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

EMA/PDCO/159625/2015 Corr
London, 22 May 2015

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

EMEA-001656-PIP01-14

Scope of the application

Active substance(s):

Momelotinib

Condition(s):

Treatment of acute lymphoblastic leukaemia

Treatment of essential thrombocythaemia

Treatment of post-essential thrombocythaemia myelofibrosis

Treatment of polycythaemia vera

Treatment of post-polycythaemia vera myelofibrosis

Pharmaceutical form(s):

Film-coated tablet

Age-appropriate oral dosage form

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Gilead Sciences International Ltd

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Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Gilead Sciences International Ltd submitted for agreement to the European Medicines Agency on 6 August 2014 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 16 September 2014.

Supplementary information was provided by the applicant on 27 February 2015. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a deferral.

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 occurs only in adult populations.

   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. Conditions:

- Treatment of essential thrombocythaemia
- Treatment of post-essential thrombocythaemia myelofibrosis
- Treatment of polycythaemia vera
- Treatment of post-polycythaemia vera myelofibrosis

The waiver applies to:

- all subsets of the paediatric population from birth to less than 18 years of age;
- for film-coated tablet and age-appropriate oral liquid dosage form for oral use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of acute lymphoblastic leukaemia

2.1.1. Indication(s) targeted by the PIP

Treatment of paediatric patients with newly diagnosed acute lymphoblastic leukaemia with a Janus kinase (JAK)-activating mutation in combination with chemotherapy.

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

From birth to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Film-coated tablet

Age-appropriate oral dosage form

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	2	**Study 1**: Adaptation of film-coated tablet for paediatric use **Study 2**: Development of an age-appropriate oral dosage form
Non-clinical studies	2	**Study 3**: Juvenile toxicity study **Study 4**: *In vitro* and *in vivo* efficacy testing

Clinical studies	3	**Study 5**: Cohort-sequential, open label, non-controlled, single-agent dose-escalation trial to evaluate pharmacokinetics, safety and anti-leukaemia activity of momelotinib in children from birth to less than 18 years of age (and young adults) with a relapsed or refractory acute lymphoblastic leukaemia with a Janus kinase-activating mutation or with a CRLF2 overexpression for whom no effective treatment is available **Study 6**: Cohort-sequential, open label, non-controlled dose-escalation trial to evaluate pharmacokinetics, safety and anti-leukaemia activity of momelotinib in combination with a chemotherapy regimen in children from birth to less than 18 years of age (and young adults) with newly-diagnosed acute lymphoblastic leukaemia with a JAK-activating mutation **Study 7**: Open-label, controlled trial to evaluate safety and efficacy of momelotinib as add-on to a chemotherapy regimen in children from birth to less than 18 years of age (and young adults) with newly-diagnosed acute lymphoblastic leukaemia with a JAK-activating mutation
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 July 2027
Deferral for one or more measures contained in the paediatric investigation plan:	No