EMA/281879/2015

European Medicines Agency decision

P/0097/2015

of 8 May 2015

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for human recombinant interleukin-2 (EMEA-001556-PIP01-13) 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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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 Iltoo Pharma on 11 October 2013 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 20 March 2015, in accordance with Article 18 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 human recombinant interleukin-2, powder 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 deferral for human recombinant interleukin-2, powder 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

A waiver for human recombinant interleukin-2, powder 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 4

This decision is addressed to Iltoo Pharma, 47/83, Bd de l'hôpital, 75013 – Paris, France.

Done at London, 8 May 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/28114/2015
London, 20 March 2015

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

EMEA-001556-PIP01-13

Scope of the application

Active substance(s):

Human recombinant interleukin-2

Condition(s):

Treatment of type 1 diabetes mellitus

Pharmaceutical form(s):

Powder for solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Iltoo Pharma

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Iltoo Pharma submitted for agreement to the European Medicines Agency on 11 October 2013 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 July 2014.

Supplementary information was provided by the applicant on 18 December 2014. The applicant proposed modifications to the paediatric investigation plan.

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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)(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 the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

   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 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 type 1 diabetes mellitus

The waiver applies to:

- the paediatric population from birth to less than 6 months;
- for powder for solution for injection, for 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 subset(s).

The waiver applies to:

- the paediatric population from 6 months to less than 12 months;
- for powder for solution for injection, for subcutaneous 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 type 1 diabetes mellitus

2.1.1. Indication(s) targeted by the PIP

Treatment of recently diagnosed type 1 diabetes with residual beta cell function

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

From 1 year to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Powder for solution for injection

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable

Area	Number of measures	Description
Non-clinical studies	1	**Study 1** Evaluation of systemic immune changes in juvenile CD-1 mice after chronic low-dose IL-2 administration
Clinical studies	5	**Study 2** Double-blind, randomised, multiple-dose, placebo-controlled trial to evaluate activity and safety of human recombinant interleukin-2 as add-on to insulin in children from 6 to less than 12 years of age with type 1 diabetes diagnosed within the last 3 months **Study 3** Double-blind, randomised, placebo-controlled exploratory trial to evaluate efficacy, safety and pharmacokinetics of human recombinant interleukin-2 as add-on to insulin in children from 6 to less than 18 years of age (and in adults) with type 1 diabetes diagnosed within the last 2 months **Study 4** Double-blind, randomised, single-dose, placebo-controlled trial to evaluate efficacy and safety of human recombinant interleukin-2 as add-on to insulin in children from 6 to less than 18 years of age (and in adults) with type 1 diabetes diagnosed within the last 2 months **Study 5** Double-blind, randomised, single-dose, placebo-controlled exploratory trial to evaluate efficacy and safety of human recombinant interleukin-2 as add-on to insulin in children from 1 to less than 6 years of age with type 1 diabetes diagnosed within the last 2 months **Study 6** Double-blind, randomised, single-dose, placebo-controlled trial to evaluate efficacy and safety of human recombinant interleukin-2 as add-on to insulin in children from 1 to less than 6 years of age with type 1 diabetes diagnosed within the last 2 months
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 September 2024
Deferral for one or more measures contained in the paediatric investigation plan:	Yes