EMA/171157/2023

European Medicines Agency decision

P/0178/2023

of 15 May 2023

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for itolizumab (EMEA-003208-PIP02-22) 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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Biocon Pharma Malta-I Limited on 25 April 2022 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 31 March 2023, 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for itolizumab, powder for solution for infusion, intravenous 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 itolizumab, powder for solution for infusion, intravenous 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 itolizumab, powder for solution for infusion, intravenous 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 Biocon Pharma Malta-I Limited, Lower Ground Floor Unit 2 Triq Valletta Il-Mosta, MST9012 – Mosta, Malta.

EMA/PDCO/955062/2022
Amsterdam, 31 March 2023

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

EMEA-003208-PIP02-22

Scope of the application

Active substance(s):

Itolizumab

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of acute graft versus host disease

Pharmaceutical form(s):

Powder for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Biocon Pharma Malta-I Limited

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Biocon Pharma Malta-I Limited submitted for agreement to the European Medicines Agency on 25 April 2022 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 May 2022.

Supplementary information was provided by the applicant on 1 December 2022. 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)(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.

   The Paediatric Committee member Norway 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.

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 acute graft versus host disease

The waiver applies to:

- the paediatric population from birth to less than 28 days of age;
- powder for solution for infusion, intravenous 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 graft versus host disease

2.1.1. Indication(s) targeted by the PIP

Treatment of acute graft versus host disease

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

From 28 days of age to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Powder for solution for infusion

2.1.4. Measures

Area	**Description**
Quality-related studies	**Study 1** Development of an age appropriate presentation.
Non-clinical studies	**Study 2** (EQ001) Pivotal toxicity study in marmoset monkeys.
Clinical studies	**Study 3** (EQ-100-02) Double blind, randomised, placebo-controlled trial to evaluate pharmacokinetics, pharmacodynamics, safety and efficacy of itolizumab in combination with standard steroid therapy compared to placebo plus standard steroid therapy in children from 12 years to less than 18 years of age (and adults) with acute graft versus host disease (aGvHD).

	Study 4 (EQ-100-03) Double blind, randomised, placebo-controlled trial to evaluate pharmacokinetics, pharmacodynamics, safety and efficacy of itolizumab in combination with standard steroid therapy compared to placebo plus standard steroid therapy in children from 28 days of age to less than 12 years of age with acute graft versus host disease (aGvHD).
Modelling and simulation studies	**Study 5** Modelling and Simulation study to support the use of itolizumab in patients from 28 days of age to less than 18 years of age with acute graft versus host disease (aGvHD).
Other studies	Not applicable
Extrapolation plan	Studies 3, 4 and 5 are part of an extrapolation plan covering the paediatric population from 28 days to less than 12 years of age, as agreed by the PDCO.

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 August 2029
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.