EMA/68911/2022

European Medicines Agency decision

P/0068/2022

of 11 March 2022

on the acceptance of a modification of an agreed paediatric investigation plan for glutamine (EMEA-001996-PIP02-16-M01) 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.

European Medicines Agency decision

P/0068/2022

of 11 March 2022

on the acceptance of a modification of an agreed paediatric investigation plan for glutamine (EMEA-001996-PIP02-16-M01) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0003/2018 issued on 4 January 2018,

Having regard to the application submitted by Emmaus Medical Europe Ltd. on 18 October 2021 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 21 January 2022, in accordance with Article 22 of Regulation (EC) No 1901/2006,

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 acceptance of changes to the agreed paediatric investigation plan and to the deferral.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral.

[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

Changes to the agreed paediatric investigation plan for glutamine, oral powder, oral use, including changes to the deferral, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision is addressed to Emmaus Medical Europe Ltd., 1 Anglesea Lane, Corrig Avenue, A96 VK65 – Dublin, Ireland.

EMA/PDCO/603088/2021
Amsterdam, 21 January 2022

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-001996-PIP02-16-M01

Scope of the application

Active substance(s):

Glutamine

Condition(s):

Treatment of sickle cell disease

Pharmaceutical form(s):

Oral powder

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Emmaus Medical Europe Ltd.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Emmaus Medical Europe Ltd. submitted to the European Medicines Agency on 18 October 2021 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0003/2018 issued on 4 January 2018.

The application for modification proposed changes to the agreed paediatric investigation plan and to the deferral.

The procedure started on 22 November 2021.

Scope of the modification

Some timelines of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan and to the deferral in the scope set out in the Annex I of this opinion.

   The Norwegian Paediatric Committee member agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the 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 sickle cell disease

The waiver applies to:

- the paediatric population from birth to less than 6 months of age;
- oral powder, oral use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of sickle cell disease

2.1.1. Indication(s) targeted by the PIP

Treatment of sickle cell disease

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

From 6 months to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Oral powder

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable
Clinical studies	4	**Study 1** Exploratory randomised, placebo-controlled, double-blind, parallel-group, study to evaluate the safety and activity of l-glutamine therapy for sickle cell anemia and sickle β0-thalassemia in patients from 8 to less than 18 years of age (and adults) (Study 10478). **Study 2** Confirmatory randomised, placebo-controlled, double-blind, parallel-group, study to evaluate the safety and efficacy of

		l-glutamine therapy for sickle cell anemia and sickle β0-thalassemia in patients from 5 to less than 18 years of age (and adults) (GLUSCC09-01). **Study 3** Confirmatory randomised, placebo controlled, study to evaluate the safety and efficacy of l-glutamine therapy for sickle cell anemia and sickle β0-thalassemia in patients from 6 months to less than 5 years of age. **Study 4** Open-label study to investigate the dose, safety and activity of L-glutamine oral powder in adult and paediatric patients with a body weight less than or equal to 65 Kg with sickle cell anemia and sickle β0-thalassemia.
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:	No
Date of completion of the paediatric investigation plan:	By December 2028
Deferral for one or more measures contained in the paediatric investigation plan:	Yes