EMA/339066/2019

European Medicines Agency decision

P/0258/2019

of 16 July 2019

on the agreement of a paediatric investigation plan and on the granting of a waiver for lonafarnib (EMEA-002516-PIP01-18) 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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on the agreement of a paediatric investigation plan and on the granting of a waiver for lonafarnib (EMEA-002516-PIP01-18) 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 Eiger BioPharmaceuticals Europe Limited on 22 February 2019 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 29 May 2019, in accordance with Article 17 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 lonafarnib, capsule, hard, 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 lonafarnib, capsule, hard, 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 Eiger BioPharmaceuticals Europe Limited, 30 Upper High Street, Thame, OX9 3EZ – Oxon, United Kingdom.

EMA/PDCO/254152/2019
Amsterdam, 29 May 2019

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

EMEA-002516-PIP01-18

Scope of the application

Active substance(s):

Lonafarnib

Condition(s):

Treatment of Hutchinson-Gilford Progeria Syndrome

Treatment of progeroid laminopathies

Pharmaceutical form(s):

Capsule, hard

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Eiger BioPharmaceuticals Europe Limited

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Eiger BioPharmaceuticals Europe Limited submitted for agreement to the European Medicines Agency on 22 February 2019 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 1 April 2019.

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 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 Hutchinson-Gilford Progeria Syndrome

The waiver applies to:

- the paediatric population from birth to less than 12 months of age;
- capsule, hard, oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

1.2. Condition:

Treatment of progeroid laminopathies

The waiver applies to:

- the paediatric population from birth to less than 12 months of age;
- capsule, hard, oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible

2. Paediatric investigation plan

2.1. Condition:

Treatment of Hutchinson-Gilford Progeria Syndrome

2.1.1. Indication(s) targeted by the PIP

Treatment of Hutchinson-Gilford Progeria Syndrome

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

From 12 months of age to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Capsule, hard

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1:** Compatibility study of lonafarnib capsule powder with recommended mixing materials.
Non-clinical studies	0	Not applicable.
Clinical studies	2	**Study 2 (ProLon1):** Open-label, single-centre, single-arm, dose-escalation trial to evaluate pharmacokinetics (PK), safety and efficacy of lonafarnib in children with Hutchinson-Gilford Progeria Syndrome or progeroid laminopathies **Study 3:** Observational cohort survival analysis in children with Hutchinson-Gilford Progeria Syndrome or progeroid laminopathies
Extrapolation, modelling and simulation studies	0	Not applicable.
Other studies	0	Not applicable.
Other measures	0	Not applicable.

2.2. Condition:

Treatment of progeroid laminopathies

2.2.1. Indication(s) targeted by the PIP

Treatment of progeroid laminopathies

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

From 12 months of age to less than 18 years of age.

2.2.3. Pharmaceutical form(s)

Capsule, hard

2.2.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1:** The same as for condition Treatment of Hutchinson-Gilford Progeria Syndrome
Non-clinical studies	0	Not applicable.
Clinical studies	2	**Study 2 (ProLon1):** The same as for condition Treatment of Hutchinson-Gilford Progeria Syndrome **Study 3:** The same as for condition Treatment of Hutchinson-Gilford Progeria Syndrome
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 2019
Deferral for one or more measures contained in the paediatric investigation plan:	No