EMA/410892/2021

European Medicines Agency decision

P/0284/2021

of 28 July 2021

on the agreement of a paediatric investigation plan and on the granting of a waiver for human immunoglobulin G1 constant region – human ectodysplasin-A1 receptor-binding domain fusion protein (ER004) (EMEA-002995-PIP01-21) 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 EspeRare Foundation on 19 March 2021 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 25 June 2021, 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 human immunoglobulin G1 constant region – human ectodysplasin-A1 receptor-binding domain fusion protein (ER004), solution for injection, intraamniotic 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 human immunoglobulin G1 constant region – human ectodysplasin-A1 receptor-binding domain fusion protein (ER004), solution for injection, intraamniotic 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 EspeRare Foundation, 15 Avenue Sécheron, 1202 - Geneva, Switzerland.

EMA/PDCO/322416/2021 Corr
Amsterdam, 25 June 2021

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

EMEA-002995-PIP01-21

Scope of the application

Active substance(s):

Human immunoglobulin G1 constant region – human ectodysplasin-A1 receptor-binding domain fusion protein (ER004)

Condition(s):

Treatment of X-linked hypohidrotic ectodermal dysplasia (XLHED)

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Intraamniotic use

Name/corporate name of the PIP applicant:

EspeRare Foundation

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, EspeRare Foundation submitted for agreement to the European Medicines Agency on 19 March 2021 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 27 April 2021.

Supplementary information was provided by the applicant on 4 June 2021.

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)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population.

   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 X-linked hypohidrotic ectodermal dysplasia

The waiver applies to:

- the paediatric population from birth to less than 18 years of age;
- for solution for injection, intraamniotic use;
- on the grounds that the specific medicinal product is likely to be ineffective.

2. Paediatric investigation plan

2.1. Condition:

Treatment of X-linked hypohidrotic ectodermal dysplasia

2.1.1. Indication(s) targeted by the PIP

Treatment of X-linked hypohidrotic ectodermal dysplasia in male foetuses

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

From gestational week 25 to less than gestational week 32.

2.1.3. Pharmaceutical form(s)

Solution for injection

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	Study 1: Development of a pharmaceutical form (solution for injection) suitable for intra-amniotic administration.
Non-clinical studies	N/A	Not applicable.
Clinical studies	1	Study 2: Prospective, open-label, genotype-match controlled, multicentre clinical trial to investigate the efficacy and safety of intra-amniotic administration in male foetuses with X-linked hypohidrotic ectodermal dysplasia (XLHED) between week 25 and week 32 of gestational age of Human Immunoglobulin G1 constant region – human ectodysplasin-A1 receptor-binding domain fusion protein, with a 5-year extension to evaluate long-term safety and efficacy.

Area	Number of measures	Description
Extrapolation, modelling and simulation studies	N/A	Not applicable.
Other studies	N/A	Not applicable.
Other measures	N/A	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 30 June 2029
Deferral for one or more measures contained in the paediatric investigation plan:	No