MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a waiver

MHRA-100100-PIP01-21

Scope of the Application

Active Substance(s)

Human Immunoglobulin G1 Constant Region – Human Ectodysplasin-A1 Receptor-binding Domain Fusion Protein

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 the Decision

Pursuant to the Human Medicines Regulations 2012, EspeRare Foundation submitted to the licensing authority on 04/06/2021 15:41 BST an application for a Paediatric Investigation Plan

The procedure started on 22/04/2022 07:23 BST

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to agree a paediatric investigation plan and grant a waiver

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-100100-PIP01-21

Of 17/06/2022 07:46 BST

On the adopted decision for Human Immunoglobulin G1 Constant Region – Human Ectodysplasin-A1 Receptor-binding Domain Fusion Protein (MHRA-100100-PIP01-21) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for Human Immunoglobulin G1 Constant Region – Human Ectodysplasin-A1 Receptor-binding Domain Fusion Protein, Solution for injection , Intraamniotic use .

This decision is addressed to EspeRare Foundation, 15 Avenue Sécheron, Geneva, Switzerland, 1202

ANNEX I

1. Waiver

1.1 Condition:

Treatment of X-linked hypohidrotic ectodermal dysplasia (XLHED) The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 18 years of age Pharmaceutical form(s): Solution for injection Route(s) of administration: Intraamniotic use Reason for granting waiver: on the grounds that the specific medicinal product is likely to be ineffective.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of X-linked hypohidrotic ectodermal dysplasia (XLHED)

2.2 Indication(s) targeted by the PIP:

Treatment of XLHED in male foetuses

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

Male foetuses from 25 weeks to less than 32 weeks gestational age.

2.4 Pharmaceutical Form(s):

Solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of a pharmaceutical form (solution of injection) suitable for intra-amniotic administration.
Non-Clinical Studies	0	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 administrations 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 (ER004), with a 5 year extension to evaluate long-term safety and efficacy.
Extrapolation, Modeling & Simulation Studies	0	Not applicable
Other Studies	0	Not applicable
Other Measures	0	Not applicable

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	30/06/2029
Deferral of one or more studies contained in the paediatric investigation plan:	No