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

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan

MHRA-100371-PIP01-21

Scope of the Application

Active Substance(s)

Ex vivo expanded autologous human keratinocytes containing epidermal stem cells genetically modified with a gamma-retroviral (RV) vector expressing the full-length LAMB3 cDNA

Condition(s)

Treatment of Junctional Epidermolysis Bullosa (JEB)

Pharmaceutical Form(s)

Living tissue equivalent

Route(s) of Administration

Cutaneous use

Name / Corporate name of the PIP applicant

Holostem Terapie Avanzate s.r.l.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Holostem Terapie Avanzate s.r.l. submitted to the licensing authority on 23/11/2021 10:32 GMT an application for a Paediatric Investigation Plan

The procedure started on 22/07/2022 07:26 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

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-100371-PIP01-21

Of 11/04/2023 07:23 BST

On the adopted decision for Ex vivo expanded autologous human keratinocytes containing epidermal stem cells genetically modified with a gamma-retroviral (RV) vector expressing the full-length LAMB3 cDNA (MHRA-100371-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 Ex vivo expanded autologous human keratinocytes containing epidermal stem cells genetically modified with a gamma-retroviral (RV) vector expressing the full-length LAMB3 cDNA, Living tissue equivalent , Cutaneous use .

This decision is addressed to Holostem Terapie Avanzate s.r.l., via Glauco Gotterdi 11, Modena, Italy, 41125

ANNEX I

1. Waiver

1.1 Condition:

Not applicable.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Junctional Epidermolysis Bullosa (JEB)

2.2 Indication(s) targeted by the PIP:

Treatment of intermediate (previously generalised intermediate) LAMB3-dependent Junctional Epidermolysis Bullosa (JEB)

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

The paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Living tissue equivalent

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 1 (HTA-HG5-02) Open label, single arm study to evaluate the safety and activity of ex vivo expanded autologous human keratinocyte suspension containing epidermal stem cells genetically modified with a gamma-retroviral (RV) vector expressing the full-length LAMB3 cDNA (Hologene 5) in children and adolescents from birth to less than 18 years of age (and adults) with genetically confirmed intermediate LAMB3-dependent Junctional Epidermolysis Bullosa (JEB).
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:	31/10/2024
Deferral of one or more studies contained in the paediatric investigation plan:	No