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

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan and to the deferral

MHRA-101107-PIP01-23-M01

Scope of the Application

Active Substance(s)

BIRCH BARK EXTRACT

Condition(s)

Treatment of epidermolysis bullosa

Pharmaceutical Form(s)

Gel

Route(s) of Administration

Cutaneous Use

Name / Corporate name of the PIP applicant

Amryt Pharmaceuticals DAC

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Amryt Pharmaceuticals DAC submitted to the licensing authority on 09/10/2023 12:59 BST an application for a Modification

The procedure started on 01/04/2024 12:59 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 accept change(s) to the agreed paediatric investigation plan and to the deferral

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-101107-PIP01-23-M01

Of 04/04/2024 09:55 BST

On the adopted decision for BIRCH BARK EXTRACT (MHRA-101107-PIP01-23-M01) 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 modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan)

This decision applies to a Modification for BIRCH BARK EXTRACT, Gel , CUTANEOUS USE .

This decision is addressed to Amryt Pharmaceuticals DAC, 45 Mespil Road, Dublin, IRELAND, D04 W2F1

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of epidermolysis bullosa

2.2 Indication(s) targeted by the PIP:

Treatment of epidermolysis bullosa

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):

Gel

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 (EASE study; BEB-13) Double-blind, randomised, placebo (vehicle) controlled trial to evaluate efficacy and safety of birch bark extract on top of standard care in children from birth to less than 18 years of age (and adults) with epidermolysis bullosa
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:	No
Date of completion of the paediatric investigation plan:	31/12/2022
Deferral of one or more studies contained in the paediatric investigation plan:	Yes