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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

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

MHRA-100328-PIP01-21-M01

Scope of the Application

Active Substance(s)

METRELEPTIN

Condition(s)

Treatment of lipodystrophy

Pharmaceutical Form(s)

Powder for solution for injection

Route(s) of Administration

Subcutaneous 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 03/11/2021 14:31 GMT an application for a Modification

The procedure started on 22/07/2022 07:25 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.

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

gov.uk/mhra

Final Decision Letter

MHRA-100328-PIP01-21-M01

Of 03/08/2022 16:42 BST

On the adopted decision for METRELEPTIN (MHRA-100328-PIP01-21-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 METRELEPTIN, Powder for solution for injection , Subcutaneous use .

This decision is addressed to Amryt Pharmaceuticals DAC, 45 Mespil Road, Dublin 4, , Dublin 4, Ireland, D04 W2F1

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of lipodystrophy

2.2 Indication(s) targeted by the PIP:

Treatment of lipodystrophy

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):

Powder for solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	2	Study 1 Development of a 2.5 mg and of a 5 mg presentation of metreleptin to facilitate dosing of paediatric patients. Study 2 Study to select an appropriate small capacity syringe appropriate to provide accurate dosing of metreleptin to paediatric patients to be available with the appropriate presentations.
Non-Clinical Studies	0	Not applicable
Clinical Studies	3	Study 3 (991265/20010769) Open-label uncontrolled trial to evaluate activity and safety of metreleptin in children from 1 year to less than 18 years of age (and adults) with lipodystrophy. Study 4 (FHA101) Open-label, uncontrolled trial to evaluate activity and safety of metreleptin in children from 9 years to less than 18 years of age (and adults) with lipodystrophy and associated diabetes mellitus and/or hypertriglyceridaemia. Study 5 Open-label, uncontrolled trial to evaluate PK, activity and safety of metreleptin in patients less than 6 years of age with generalised lipodystrophy and associated diabetes mellitus and/or hypertriglyceridaemia.
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:	31/12/2024
Deferral of one or more studies contained in the paediatric investigation plan:	Yes