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-101151-PIP01-23-M01

Scope of the Application

Active Substance(s)

OBETICHOLIC ACID

Condition(s)

Treatment of primary biliary cirrhosis, Treatment of biliary atresia

Pharmaceutical Form(s)

Coated tablet; Tablet; Age-appropriate oral solid dosage form

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Advanz Pharma Europe Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Advanz Pharma Europe Limited submitted to the licensing authority on 14/09/2023 07:51 BST an application for a Modification

The procedure started on 05/07/2024 15:12 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-101151-PIP01-23-M01

Of 12/07/2024 13:47 BST

On the adopted decision for OBETICHOLIC ACID (MHRA-101151-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 OBETICHOLIC ACID, Coated tablet; Tablet; Age-appropriate oral solid dosage form , ORAL USE .

This decision is addressed to Advanz Pharma Europe Limited, Capital House 85 King William Street, London, UNITED KINGDOM, EC4N 7BL

ANNEX I

1. Waiver

1.1 Condition:

Treatment of primary biliary cirrhosis (PBC). The waiver applies / applied to: Paediatric Subset(s): All subsets of the paediatric population from birth to less than 18 years of age. Pharmaceutical form(s): Coated tablet Tablet Age-appropriate oral solid dosage form Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of biliary atresia.

2.2 Indication(s) targeted by the PIP:

Treatment of biliary atresia.

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

All subsets of the paediatric population from birth to less than 18 years of age.

2.4 Pharmaceutical Form(s):

Coated tablet; Tablet; Age-appropriate oral solid dosage form

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	2	Study 1 Development of an age appropriate oral solid dosage form (1.5 mg mini-tablet). Study 2 Development of an age appropriate oral solid dosage form (0.1 mg mini-tablet).
Non-Clinical Studies	2	Study 3 Dose range-finding juvenile toxicity study. Study 4 Definitive juvenile toxicity study.
Clinical Studies	2	Study 5 deleted during procedure MHRA-101151-PIP01-23-M01. Study 6 deleted during adopted UK-PIP procedure EMEA-001304-PIP02-13-M03. Study 7 deleted during adopted UK-PIP procedure EMEA-001304-PIP02-13-M03. Study 8 Natural history data collection study from biliary atresia registries. Study 9 Randomised, placebo-controlled study to evaluate the efficacy, safety, tolerability, pharmacokinetics, and pharmacodynamics of obeticholic acid in children from birth to less than 18 years with biliary atresia, post-hepatoportoenterostomy.
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:	30/06/2028
Deferral of one or more studies contained in the paediatric investigation plan:	Yes