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 deferral and grant a waiver

MHRA-101579-PIP01-24

Scope of the Application

Active Substance(s)

AZD0780

Condition(s)

Treatment of elevated cholesterol, Treatment of mixed dyslipidaemia

Pharmaceutical Form(s)

Film-coated tablet

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

AstraZeneca UK Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, AstraZeneca UK Limited submitted to the licensing authority on 19/12/2024 17:34 GMT an application for a

The procedure started on 13/01/2025 15:05 GMT

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 deferral 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.

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

gov.uk/mhra

Final Decision Letter

MHRA-101579-PIP01-24

Of 22/04/2025 15:11 BST

On the adopted decision for AZD0780 (MHRA-101579-PIP01-24) 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 for AZD0780, Film-coated tablet , ORAL USE .

This decision is addressed to AstraZeneca UK Limited, 2 Pancras Square, London, UNITED KINGDOM, N1C 4AG

ANNEX I

1. Waiver

1.1 Condition:

Condition 1: Treatment of elevated cholesterol The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 years of age Pharmaceutical form(s): Film-coated tablet Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments Condition 2: Treatment of mixed dyslipidaemia The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 18 years of age Pharmaceutical form(s): Film-coated tablet Route(s) of administration: ORAL USE Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of elevated cholesterol

2.2 Indication(s) targeted by the PIP:

Treatment of heterozygous familial hypercholesterolaemia (HeFH)

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

The paediatric population from 6 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Film-coated tablet

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 Double-blind, randomised, placebo-controlled trial on top of standard of care to evaluate the efficacy of AZD0780, safety, tolerability, growth and pubertal development in paediatric patients from 6 to less than 18 years of age with heterozygous familial hypercholesterolaemia (HeFH) and elevated low density lipoprotein cholesterol (LDL-C) $\geq$ 130 mg/dL (3.4 mmol/L).
Extrapolation, Modeling & Simulation Studies	1	Study 2 Development of population PK and population PK/PD models for AZD0780 to support the dose selection in paediatric patients from 6 years to less than 18 years of age.
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:	30/06/2031
Deferral of one or more studies contained in the paediatric investigation plan:	Yes