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 (MHRA-101177-PIP01-23)

MHRA-101177-PIP01-23-M01

Scope of the Application

Active Substance(s)

navenibart

Condition(s)

Treatment of hereditary angioedema

Pharmaceutical Form(s)

Solution for injection

Route(s) of Administration

SUBCUTANEOUS USE

Name / Corporate name of the PIP applicant

Astria Therapeutics Inc

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Astria Therapeutics Inc submitted to the licensing authority on 17/01/2025 14:15 GMT an application for a Modification

The procedure started on 17/02/2025 17:46 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 accept change(s) to the agreed 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-101177-PIP01-23-M01

Of 15/05/2025 18:08 BST

On the adopted decision for navenibart (MHRA-101177-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 navenibart, Solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Astria Therapeutics Inc, 22 Boston Wharf Road, 10th Floor, Boston, UNITED STATES OF AMERICA, 02210

ANNEX I

1. Waiver

1.1 Condition:

Treatment of hereditary angioedema The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Solution for injection Route(s) of administration: SUBCUTANEOUS USE Reason for granting waiver: on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of hereditary angioedema

2.2 Indication(s) targeted by the PIP:

Routine prevention of recurrent attacks of hereditary angioedema in paediatric patients 2 years of age and older

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

The paediatric population from 2 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 (STAR-0215-PIP Quality) Development of a lower strength of solution for injection appropriate for the paediatric population from 2 years to less than 12 years of age.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	2	Study 2 (STAR-0215-301) Randomised, double-blind, placebo-controlled trial to evaluate efficacy and safety of navenibart for prevention of angioedema attacks in adult participants with hereditary angioedema (HAE). Efficacy, safety, and PK will be assessed in adolescent participants treated with open-label navenibart. Study 3 (STAR-0215-303) Open-label trial to evaluate the safety, pharmacokinetics, pharmacodynamics and clinical activity of navenibart in children from 2 years to less than 12 years of age with hereditary angioedema.
Extrapolation, Modeling & Simulation Studies	2	Study 4 (STAR-0215-PIP M&S-1) Population PK (popPK) model to inform dose selection and support extrapolation of efficacy to the target paediatric population. Study 5 (STAR-0215-PIP Extrapolation-1) Analysis of in-house data to support the use of navenibart in the paediatric population from 2 years to less than

		18 years of age with hereditary angioedema.
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:	31/12/2032
Deferral of one or more studies contained in the paediatric investigation plan:	Yes