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

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Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral

MHRA-101705-PIP01-24

Scope of the Application

Active Substance(s)

Elamipretide

Condition(s)

Treatment of Barth syndrome

Pharmaceutical Form(s)

Solution for injection

Route(s) of Administration

SUBCUTANEOUS USE INTRAVENOUS USE

Name / Corporate name of the PIP applicant

Atnahs Pharma UK Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Atnahs Pharma UK Limited submitted to the licensing authority on 19/12/2024 18:30 GMT an application for a Paediatric Investigation Plan

The procedure started on 13/01/2025 18:43 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

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-101705-PIP01-24

Of 12/01/2026 10:19 GMT

On the adopted decision for Elamipretide (MHRA-101705-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 Paediatric Investigation Plan for Elamipretide, Solution for injection , SUBCUTANEOUS USE .

This decision is addressed to Atnahs Pharma UK Limited, Sovereign House, Miles Gray Road, Essex, Basildon , UNITED KINGDOM, SS14 3FR

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of Barth syndrome

2.2 Indication(s) targeted by the PIP:

Treatment of Barth syndrome (BTHS)

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):

Solution for injection

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of a preservative-free buffered solution of elamipretide.
Non-Clinical Studies	1	Study 2 (SPI-CIT-16-04) Definitive juvenile toxicity study in rats.
Clinical Studies	5	Study 3 (SPIBA-201 [TAZPOWER]) Two part study with a randomised, double-blind, placebo-controlled crossover study (Part 1) part followed by an open label extension (Part 2), to evaluate the safety, tolerability and efficacy of elamipretide once daily subcutaneous injection versus placebo, in children from 12 years to less than 18 years of age (and adults) with genetically confirmed Barth syndrome (BTHS). Study 4 (SPIBA-001) Open-label trial to evaluate long-term efficacy of elamipretide in children from 12 years to less than 18 years of age with genetically confirmed Barth syndrome who were enrolled in study SPIBA-201. Study 5 (SPIBA-202) Open-label, adaptive-design trial to evaluate the pharmacokinetics and safety of elamipretide (subcutaneous or intravenous administration) in children from birth to less than 12 years of age with genetically confirmed Barth syndrome. Study 6 (SPIBA-401) Randomised, double-blind, parallel-group, placebo-controlled study to evaluate efficacy and safety of elamipretide in children from 5 years to less than 18 years

		of age (and adults) with genetically confirmed Barth syndrome. Study 9 (SPIBA-402) Prospective, non-interventional clinical registry study to evaluate long-term safety and clinical outcomes in children from birth to less than 18 years of age (and adults).
Extrapolation, Modeling & Simulation Studies	2	Study 7 Physiologically-based pharmacokinetic (PBPK) modelling and simulation study to determine the initial paediatric dose for children from 2 years to less than 18 years of age. Study 8 Physiologically-based pharmacokinetic (PBPK) modelling and simulation study to determine the paediatric dose for children from birth 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:	31/12/2031
Deferral of one or more studies contained in the paediatric investigation plan:	Yes