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-101993-PIP01-25

Scope of the Application

Active Substance(s)

avenciguat

Condition(s)

Treatment of systemic sclerosis

Pharmaceutical Form(s)

Film-coated tablet; Age appropriate dosage form

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

BOEHRINGER INGELHEIM INTERNATIONAL GMBH

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, BOEHRINGER INGELHEIM INTERNATIONAL GMBH submitted to the licensing authority on 18/07/2025 19:56 BST an application for a Paediatric Investigation Plan

The procedure started on 26/08/2025 18:31 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 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-101993-PIP01-25

Of 05/12/2025 07:40 GMT

On the adopted decision for avenciguat (MHRA-101993-PIP01-25) 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 avenciguat, Film-coated tablet; Age appropriate dosage form , ORAL USE .

This decision is addressed to BOEHRINGER INGELHEIM INTERNATIONAL GMBH, Binger Strasse 173, Ingelheim am Rhein, GERMANY, 55216

ANNEX I

1. Waiver

1.1 Condition:

Treatment of systemic sclerosis The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 5 years of age Pharmaceutical form(s): Film-coated tablet Age appropriate dosage form 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 as clinical studies(s) are not feasible

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of systemic sclerosis

2.2 Indication(s) targeted by the PIP:

Treatment of juvenile systemic sclerosis (jSSc)

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

The paediatric population from 5 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Film-coated tablet Age appropriate dosage form

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an age-appropriate oral formulation.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 2 (1366-0056) Open-label single-arm trial to evaluate pharmacokinetics, safety, activity, acceptability/ palatability of avenciguat as add-on to best standard of care in children from 5 years to less than 18 years of age with jSSc.
Extrapolation, Modeling & Simulation Studies	3	Study 3 Modelling and simulation dose-finding study in children from 5 years to less than 18 years of age with jSSc. Study 4 Modelling and simulation pharmacokinetic-pharmacodynamic (PK/PD) analysis to support extrapolation of efficacy from adults to children from 5 years to less than 18 years of age with jSSc. Extrapolation plan Studies 2, 3 and 4 are part of an extrapolation plan covering the paediatric population from 5 years to less than 18 years of age with jSSC.
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:	30/06/2035
Deferral of one or more studies contained in the paediatric investigation plan:	Yes