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 and grant a waiver

MHRA-101400-PIP01-24

Scope of the Application

Active Substance(s)

nirogacestat hydrobromide

Condition(s)

Treatment of soft tissue sarcoma

Pharmaceutical Form(s)

Film-coated tablet

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

SpringWorks Therapeutic Ireland Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, SpringWorks Therapeutic Ireland Limited submitted to the licensing authority on 15/04/2024 17:28 BST an application for a Paediatric Investigation Plan

The procedure started on 21/06/2024 15:14 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.

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Final Decision Letter

MHRA-101400-PIP01-24

Of 19/07/2024 15:45 BST

On the adopted decision for nirogacestat hydrobromide (MHRA-101400-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 nirogacestat hydrobromide, Film-coated tablet , ORAL USE .

This decision is addressed to SpringWorks Therapeutic Ireland Limited, 70 Sir John Regerson's Quay, Co Dublin, Dublin, IRELAND, D02R296

ANNEX I

1. Waiver

1.1 Condition:

Treatment of soft tissue sarcoma The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 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 is likely to be unsafe.

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of soft tissue sarcoma

2.2 Indication(s) targeted by the PIP:

Treatment of desmoid tumours

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):

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 (ARST1921) Open-label, single arm trial to evaluate pharmacokinetics, safety, activity of nirogacestat hydrobromide in children from 2 years to less than 18 years of age with progressing desmoid tumours.
Extrapolation, Modeling & Simulation Studies	1	Study 2 (PopPK Analysis Plan) Modelling and simulation study to evaluate the use of nirogacestat hydrobromide in the proposed paediatric indication in children from 2 years to less than 18 years of age with progressing desmoid tumours.
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:	Yes
Date of completion of the paediatric investigation plan:	31/07/2025
Deferral of one or more studies contained in the paediatric investigation plan:	Yes