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-101923-PIP01-25

Scope of the Application

Active Substance(s)

Pimicotinib hydrochloride hydrate

Condition(s)

Treatment of tenosynovial giant cell tumours

Pharmaceutical Form(s)

Capsule, hard

Route(s) of Administration

ORAL USE

Name / Corporate name of the PIP applicant

Merck Serono Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Merck Serono Limited submitted to the licensing authority on 16/05/2025 14:38 BST an application for a Paediatric Investigation Plan

The procedure started on 09/06/2025 17:42 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-101923-PIP01-25

Of 05/08/2025 13:39 BST

On the adopted decision for Pimicotinib hydrochloride hydrate (MHRA-101923-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 Pimicotinib hydrochloride hydrate, Capsule, hard , ORAL USE .

This decision is addressed to Merck Serono Limited, 5 New Square, Bedfont Lakes Business Park, Feltham, UNITED KINGDOM, TW14 8HA

ANNEX I

1. Waiver

1.1 Condition:

Treatment of tenosynovial giant cell tumours The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 12 years of age Pharmaceutical form(s): Capsule, hard 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 tenosynovial giant cell tumours

2.2 Indication(s) targeted by the PIP:

Treatment of patients from 12 years to less than 18 years of age with tenosynovial giant cell tumours (TGCT) who require systemic therapy.

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

The paediatric population from 12 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Capsule, hard

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Generation of data on the compatibility of mixing the content of the hard capsules of pimicotinib in common food and liquid.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 2 (ABSK021-101) Open-label multicentre study to assess the safety, pharmacokinetics and antitumor activity of pimicotinib and to provide PK/pharmacodynamic data to support the extrapolation of efficacy from adults to adolescents from 12 to less than 18 years of age with tenosynovial giant cell tumours who require systemic therapy.
Extrapolation, Modeling & Simulation Studies	2	Study 3 Modelling and simulation study to confirm and further define the dose and regimen of pimicotinib to be used in adolescents from 12 years to less than 18 years of age with tenosynovial giant cell tumour and to support the extrapolation of efficacy from adults with tenosynovial giant cell tumour. Extrapolation plan Study 2 (ABSK021-101) and study 3 are part of the extrapolation plan of efficacy data from adult patients to the paediatric population from 12 years to less than 18 years of age with tenosynovial giant cell 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:	31/08/2027
Deferral of one or more studies contained in the paediatric investigation plan:	Yes