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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan (MHRA-100492-PIP01-22-M01) and to the deferral

MHRA-100492-PIP01-22-M02

Scope of the Application

Active Substance(s)

MOLGRAMOSTIM

Condition(s)

Treatment of pulmonary alveolar proteinosis

Pharmaceutical Form(s)

Nebuliser solution

Route(s) of Administration

INHALATION USE

Name / Corporate name of the PIP applicant

Kinesys Consulting Limited

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Kinesys Consulting Limited submitted to the licensing authority on 27/06/2024 23:08 BST an application for a Modification

The procedure started on 03/09/2024 12:27 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 accept change(s) to the agreed paediatric investigation plan and to the 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-100492-PIP01-22-M02

Of 08/10/2024 17:43 BST

On the adopted decision for MOLGRAMOSTIM (MHRA-100492-PIP01-22-M02) 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 MOLGRAMOSTIM, Nebuliser solution , INHALATION USE .

This decision is addressed to Kinesys Consulting Limited, 196 Bath Street, Glasgow, UNITED KINGDOM, G2 4HG

ANNEX I

1. Waiver

1.1 Condition:

Treatment of pulmonary alveolar proteinosis The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 years of age Pharmaceutical form(s): Nebuliser solution Route(s) of administration: INHALATION USE Reason for granting waiver: on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric Investigation Plan:

2.1 Condition(s):

Treatment of pulmonary alveolar proteinosis

2.2 Indication(s) targeted by the PIP:

Treatment of autoimmune pulmonary alveolar proteinosis (aPAP)

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

The paediatric population from 6 years to less than 18 years of age

2.4 Pharmaceutical Form(s):

Nebuliser solution

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	2	Study 1 Development of lower strength formulation appropriate to the paediatric population. Study 2 Evaluation of formulations and reduction of fill volume of delivered dose using simulated paediatric breathing pattern.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 3 (SAV006-04) Open-label, un-controlled multicentre study to investigate the activity of inhaled molgramostim in paediatric patients from 6 years to less than 18 years of age with autoimmune pulmonary alveolar proteinosis.
Extrapolation, Modeling & Simulation Studies	1	Study 4 Analysis of adult data (Studies MOL-PAP-002 and SAV006-05) and paediatric data (study SAV006-04) to extrapolate efficacy of molgramostim from adult to paediatric patients from 6 years to less than 18 years of age with autoimmune pulmonary alveolar proteinosis.
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:	31/03/2028
Deferral of one or more studies contained in the paediatric investigation plan:	Yes