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

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan and to the deferral

MHRA-100627-PIP01-22-M01

Scope of the Application

Active Substance(s)

Liposomal ciclosporin A (L-CsA)

Condition(s)

Treatment of bronchiolitis obliterans syndrome (BOS)

Pharmaceutical Form(s)

Powder for nebuliser solution

Route(s) of Administration

INHALATION USE

Name / Corporate name of the PIP applicant

Zambon S.p.A.

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Zambon S.p.A. submitted to the licensing authority on 29/07/2022 09:24 BST an application for a Modification

The procedure started on 25/01/2023 07:01 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 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-100627-PIP01-22-M01

Of 20/02/2023 08:11 GMT

On the adopted decision for Liposomal ciclosporin A (L-CsA) (MHRA-100627-PIP01-22-M01) 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 Liposomal ciclosporin A (L-CsA), Powder for nebuliser solution , INHALATION USE .

This decision is addressed to Zambon S.p.A. , Via Lillo Del Duca 10, Bresso (MI), ITALY, 20091

ANNEX I

1. Waiver

1.1 Condition:

Treatment of bronchiolitis obliterans syndrome (BOS) The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 6 years of age Pharmaceutical form(s): Powder for nebuliser solution Route(s) of administration: INHALATION 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 bronchiolitis obliterans syndrome (BOS)

2.2 Indication(s) targeted by the PIP:

Treatment of bronchiolitis obliterans syndrome (BOS)

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):

Powder for nebuliser solution

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 Open label trial, historical-controlled, to assess the tolerability, safety and pharmacokinetics of aerosolised L-CsA in addition to standard of care therapy for the treatment of BOS of any aetiology in children from 6 years to less than 18 years of age.
Extrapolation, Modeling & Simulation Studies	0	Not applicable.
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:	31/08/2025
Deferral of one or more studies contained in the paediatric investigation plan:	No