EMA/575636/2022

# European Medicines Agency decision

P/0231/2022

of 8 July 2022

on the acceptance of a modification of an agreed paediatric investigation plan for liposomal ciclosporin A (L-CsA) (EMEA-002344-PIP02-18-M01) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

**Disclaimer**

This decision does not constitute entitlement to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006.

**Only the English text is authentic.**

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The European Medicines Agency,

Having regard to the Treaty on the Functioning of the European Union,

Having regard to Regulation (EC) No 1901/2006 of the European Parliament and of the Council of 12 December 2006 on medicinal products for paediatric use and amending Regulation (EEC) No. 1768/92, Directive 2001/20/EC, Directive 2001/83/EC and Regulation (EC) No 726/2004[1],

Having regard to Regulation (EC) No 726/2004 of the European Parliament and of the Council of 31 March 2004 laying down Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0312/2019 issued on 11 September 2019,

Having regard to the application submitted by Zambon S.p.A. on 18 February 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 20 May 2022, in accordance with Article 22 of Regulation (EC) No 1901/2006,

Having regard to Article 25 of Regulation (EC) No 1901/2006,

Whereas:

(1) The Paediatric Committee of the European Medicines Agency has given an opinion on the acceptance of changes to the agreed paediatric investigation plan.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[1] OJ L 378, 27.12.2006, p.1, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

***Article 1***

Changes to the agreed paediatric investigation plan for liposomal ciclosporin A (L-CsA), powder for nebuliser solution, inhalation use, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

***Article 2***

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

EMA/PDCO/141807/2022
Amsterdam, 20 May 2022

# Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-002344-PIP02-18-M01

## Scope of the application

**Active substance(s):**

Liposomal ciclosporin A (L-CsA)

**Condition(s):**

Treatment of bronchiolitis obliterans syndrome

**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 opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Zambon S.p.A. submitted to the European Medicines Agency on 18 February 2022 an application for modification of the agreed paediatric investigation plan with a waiver as set out in the European Medicines Agency's decision P/0312/2019 issued on 11 September 2019.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 21 March 2022.

On 18 March 2021, Breath Therapeutics GmbH requested to transfer the paediatric investigation plan to Zambon S.p.A.

## Scope of the modification

Some timelines of the Paediatric Investigation Plan have been modified.

## Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

   The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan and the subset(s) of the paediatric population and condition(s) covered by the waiver are set out in the Annex I.

This opinion is forwarded to the applicant and the Executive Director of the European Medicines Agency, together with its annex and appendix.

# Annex I

# The subset(s) of the paediatric population and condition(s) covered by the waiver and the measures and timelines of the agreed paediatric investigation plan (PIP)

# 1. Waiver

## *1.1. Condition:*

Treatment of bronchiolitis obliterans syndrome

The waiver applies to:

- the paediatric population from birth to less than 6 years of age;
- powder for nebuliser solution, inhalation use;
- 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:*

Treatment of bronchiolitis obliterans syndrome

### 2.1.1. Indication(s) targeted by the PIP

Treatment of bronchiolitis obliterans syndrome (BOS)

### 2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 6 years to less than 18 years of age

### 2.1.3. Pharmaceutical form(s)

Powder for nebuliser solution

### 2.1.4. Measures

| Area | Description |
|---|---|
| Quality-related studies | Not applicable |
| Non-clinical studies | Not applicable |
| Clinical studies | Study 1 (BOSTON-5)<br>Open-label trial, historical-controlled, to assess the tolerability, safety, and pharmacokinetics of aerosolized 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, modelling and simulation studies | Not applicable |
| Other studies | Not applicable |
| Other measures | Not applicable |

# 3. Follow-up, completion and deferral of PIP

| | |
|---|---|
| Concerns on potential long term safety/efficacy issues in relation to paediatric use: | No |
| Date of completion of the paediatric investigation plan: | By August 2025 |
| Deferral for one or more measures contained in the paediatric investigation plan: | No |