EMA/631870/2020

European Medicines Agency decision

P/0494/2020

of 21 December 2020

on the acceptance of a modification of an agreed paediatric investigation plan for aztreonam (Cayston), (EMEA-000827-PIP01-09-M05) 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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/228/2010 issued on 29 October 2010, the decision P/117/2011 issued on 20 May 2011, the decision P/0124/2013 issued on 28 May 2013 and the decision P/0064/2016 issued on 18 March 2016,

Having regard to the application submitted by Gilead Sciences International Ltd. on 7 August 2020 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 13 November 2020, 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 and to the deferral.

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

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

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for aztreonam (Cayston), powder and solvent for nebuliser solution, inhalation use, including changes to the deferral, 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 Gilead Sciences International Ltd., Flowers Building, Granta Park, Great Abington, CB21 6GT - Cambridge, United Kingdom.

EMA/PDCO/438736/2020
Amsterdam, 13 November 2020

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

EMEA-000827-PIP01-09-M05

Scope of the application

Active substance(s):

Aztreonam

Invented name:

Cayston

Condition(s):

Treatment of *Pseudomonas aeruginosa* pulmonary infection / colonisation in patients with cystic fibrosis

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Powder and solvent for nebuliser solution

Route(s) of administration:

Inhalation use

Name/corporate name of the PIP applicant:

Gilead Sciences International Ltd.

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Gilead Sciences International Ltd. submitted to the European Medicines Agency on 7 August 2020 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/228/2010 issued on 29 October 2010, the decision P/117/2011 issued on 20 May 2011, the decision P/0124/2013 issued on 28 May 2013 and the decision P/0064/2016 issued on 18 March 2016.

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

The procedure started on 15 September 2020.

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 and to the deferral in the scope set out in the Annex I of this opinion.

   The Norwegian Paediatric Committee member 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 annexes 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 Pseudomonas aeruginosa (PA) pulmonary infection/colonisation in patients with cystic fibrosis (CF)

The waiver applies to:

- all subsets of the paediatric population from birth to less than 3 months of age;
- powder and solvent for nebuliser solution, inhalation use;
- on the grounds that clinical studies cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the paediatric population.

2. Paediatric Investigation Plan

2.1. Condition:

Treatment of *Pseudomonas aeruginosa* pulmonary infection/colonisation in patients with cystic fibrosis (CF)

2.1.1. Indication(s) targeted by the PIP

Treatment of initial *Pseudomonas aeruginosa* pulmonary infection/colonisation in patients with cystic fibrosis

Treatment of chronic *Pseudomonas aeruginosa* pulmonary infection/colonisation in patients with cystic fibrosis

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

From 3 months to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Powder and solvent for nebuliser solution

2.1.4. Measures

Area	Number of measures	Description
Quality	0	Not applicable
Non-clinical	0	Not applicable
Clinical	4	**Study 1** Open-label, single arm trial to evaluate the safety and pharmacokinetic data of aztreonam 75 mg powder and solvent for nebulizer solution (AZLI) in children from 3 months to less than 18 years of age with CF and initial pulmonary infection/colonisation with PA with a 6 month follow-up for safety and recurrence of PA.(GS-US-205-0162)

		Study 2 Deleted at procedure EMEA-000827-PIP01-09-M04 **Study 3** Open-label, randomized, multicentre, parallel group trial to evaluate efficacy and safety of aztreonam 75 mg powder and solvent for nebulizer solution (AZLI) versus tobramycin nebuliser solution (TNS) in an intermittent aerosolized antibiotic regimen, in adults and children from 6 years to less than 18 years with CF and chronic PA pulmonary colonisation/infection followed by a 6 months open-label, single-arm extension.(GS-US-205-0110) **Study 4** Open-label single arm trial to evaluate the safety of aztreonam 75 mg powder and solvent for nebulizer solution (AZLI) in children less than 13 years of age with CF and chronic PA pulmonary colonisation/infection. (GS US 205 0160) **Study 5** Double blind, parallel group, active controlled study to evaluate aztreonam 75 mg powder and solvent for nebulizer solution (AZLI) for 14 versus 28 days for eradication of new onset *PA* infection in children from 3 months to less than 18 years with CF with a 24 months follow-up. (GS-US-205-1850)

3. Follow-up, completion and deferral of PIP

Concerns on potential long-term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By April 2022
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of *Pseudomonas aeruginosa* pulmonary infection/colonisation in patients with cystic fibrosis (CF).

Authorised indication(s):

- Cayston is indicated for the suppressive therapy of chronic pulmonary infections due to *Pseudomonas aeruginosa* in patients with cystic fibrosis (CF) aged 6 years and older.

Authorised pharmaceutical form(s):

Powder and solvent for nebuliser solution

Authorised route(s) of administration:

Inhalation use