EMA/663186/2010

European Medicines Agency decision

P/228/2010

of 29 October 2010

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for aztreonam (Cayston), (EMEA-000827-PIP01-09) 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 application submitted by Gilead Sciences International Limited on 8 March 2010 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 8 October 2010, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 21 of said Regulation and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision granting a deferral.

(4) It is therefore appropriate to adopt a decision granting a waiver.

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

Has adopted this decision:

Article 1

A paediatric investigation plan for aztreonam (Cayston), powder and solvent for nebuliser solution, inhalation use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

Article 2

A deferral for aztreonam (Cayston), powder and solvent for nebuliser solution, inhalation use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

A waiver for aztreonam (Cayston), powder and solvent for nebuliser solution, inhalation use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 4

This decision is addressed to Gilead Sciences International Limited, Granta Park, Abington, CB21 6GT – Cambridge, United Kingdom.

Done at London, 29 October 2010

For the European Medicines Agency
Thomas Lönngren
Executive Director

(Signature on file)

EMA/PDCO/592877/2010

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and a waiver

EMEA-000827-PIP01-09

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 Limited

Information about the authorised medicinal product: see Annex II

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Gilead Sciences International Limited submitted for agreement to the European Medicines Agency on 8 March 2010 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 15 April 2010.

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Supplementary information was provided by the applicant on 30 July 2010.

Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary reports:

   - to agree the paediatric investigation plan in accordance with Article 18 of said Regulation,
   - to grant a deferral in accordance with Article 21 of said Regulation,
   - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

   The Icelandic and the Norwegian Paediatric Committee members agree with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the agreed 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(es) and appendix.

London, 8 October 2010

On behalf of the Paediatric Committee
Dr Daniel Brasseur, Chairman

(Signature on file)

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

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;
- for 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. Studies

Area	Number of studies	Description
Quality	-	Not applicable.
Non-clinical	-	Not applicable.
Clinical	2	**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. **Study 2** Open-label, randomized, active-controlled trial to evaluate the safety, efficacy and pharmacokinetics for a single course (28 days) 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 24 months follow-up for safety and efficacy. **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. **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.

3. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety issues and efficacy in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By July 2016
Deferral for one or more studies 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 indications:

- Cayston is indicated for the suppressive therapy of chronic pulmonary infections due to Pseudomonas aeruginosa in patients with cystic fibrosis (CF) aged 18 years and older.

EU Number	Invented name	Strength	Pharmaceutical form	Route of administration	Packaging	Package size
EU/1/09/543/001	Cayston	75 mg	Powder and solvent for nebuliser solution	Inhalation use	Powder: vial (glass)	84 vials + 88 ampoules
EU/1/09/543/002	Cayston	75 mg	Powder and solvent for nebuliser solution	Inhalation use	Powder: vial (glass)	84 vials + 88 ampoules + 1 Altera Nebuliser Handset