EMA/370084/2011

European Medicines Agency decision

P/117/2011

of 20 May 2011

on the acceptance of a modification of an agreed paediatric investigation plan for aztreonam (Cayston) (EMEA-000827-PIP01-09-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 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,

Having regard to the application submitted by Gilead Sciences International Limited on 5 March 2011 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 20 April 2011, 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.
[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 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 Limited, Granta Park, Abington, CB21 6GT Cambridge, United Kingdom.

Done at London, 20 May 2011

For the European Medicines Agency
Andreas Pott
Acting Executive Director
(Signature on file)

EMA/370084/2011

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

EMEA-000827-PIP01-09-M01

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 22 of Regulation (EC) No 1901/2006 as amended, Gilead Sciences International Limited submitted to the European Medicines Agency on 5 March 2011 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 application for modification proposed changes to the agreed paediatric investigation plan.

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The procedure started on 23 March 2011.

Scope of the modification

Some measures and/or timelines of the original opinion 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 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 annex(es) and appendix.

London, 20 April 2011

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 October 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