



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/34144/2011

European Medicines Agency decision P/31/2011

of 28 January 2011

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for amikacin (sulfate) (EMA-000525-PIP01-08) 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 726/2004¹,

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 Agency²,

Having regard to the application submitted by Transave, Inc. on 3 May 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 10 December 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 amikacin (sulfate), nebuliser suspension, 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 amikacin (sulfate), nebuliser suspension, 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 amikacin (sulfate), nebuliser suspension, 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 Transave, Inc., Princeton Corporate Plaza, 11 Deer Park Drive, Suite 117, 08852-1923 Monmouth Junction, New Jersey, the United States.

Done at London, 28 January 2011

For the European Medicines Agency
Andreas Pott
Acting Executive Director

(Signature on file)



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/709177/2010

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

EMA-000525-PIP01-08

Scope of the application

Active substance(s):

Amikacin (sulfate)

Condition(s):

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

Pharmaceutical form(s):

Nebuliser suspension

Route(s) of administration:

Inhalation use

Name/corporate name of the PIP applicant:

Transave, Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Transave, Inc. submitted for agreement to the European Medicines Agency on 3 May 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 10 June 2010.

Supplementary information was provided by the applicant on 4 October 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 report:

- 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 do 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, 10 December 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* lung infection/colonisation in cystic fibrosis patients

The waiver applies to:

- children from birth to less than 3 months of age,
- for nebuliser suspension for inhalation use,
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies are not feasible.

2. Paediatric Investigation Plan

2.1. Condition: Treatment of *Pseudomonas aeruginosa* lung infection/colonisation in cystic fibrosis patients

2.1.1. Indication(s) targeted by the PIP

Treatment of initial (early) *Pseudomonas aeruginosa* lung infection/colonisation in cystic fibrosis patients

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

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)

Nebuliser suspension

2.1.4. Studies

Area	Number of studies	Description
Quality	Total number of quality studies	Not applicable.
Non-clinical	2	Study 1: 2-year inhalation carcinogenicity study in rats Study 2: Juvenile rodent inhalation toxicity and toxicokinetic study
Clinical	8	Study 3: Multidose safety and tolerability study of dose escalation of liposomal amikacin for inhalation in cystic fibrosis patients (children from 6 years to less than 18 years and adults) with chronic lung infections due to <i>Pseudomonas aeruginosa</i>-double blind portion Study 4: Multidose safety and tolerability study of dose escalation of liposomal amikacin for inhalation in cystic fibrosis patients (children from 6 years to less than 18 years and adults)with chronic lung infections due to <i>Pseudomonas aeruginosa</i>-open label portion Study 5: Multidose safety and tolerability study of dose escalation of liposomal amikacin for inhalation in cystic fibrosis patients (children from 6 years to less than 18 years and adults)with chronic lung infections due to <i>Pseudomonas aeruginosa</i> Study 6: Randomised, active controlled, multicentre study to assess the efficacy, safety and tolerability of liposomal amikacin for inhalation in cystic fibrosis patients (children from 6 years to less than 18 years and adults) with chronic lung infection due to <i>Pseudomonas aeruginosa</i>. Study 7: Randomised, placebo controlled, double blind, multicentre study to assess the efficacy, safety and tolerability of liposomal amikacin for inhalation in cystic fibrosis patients (children from 6 years to less than 18 years and adults) with chronic lung infection due to <i>Pseudomonas aeruginosa</i> Study 8: Long term safety and tolerability study of open label liposomal amikacin for inhalation in cystic fibrosis patients (children from 6 years to less than 18 years and adults) with chronic lung infection due to <i>Pseudomonas aeruginosa</i> Study 9: Randomised, active controlled, multi-centre study to assess the efficacy and safety of liposomal amikacin for inhalation for the treatment of first or early infections of <i>P. aeruginosa</i> in children aged from 3 months to less than 18 years with cystic fibrosis Study 10: Open label, multi-cycle study of safety, tolerability and efficacy of liposomal amikacin for inhalation in cystic fibrosis children aged from 3 years to less than 6 years old, with chronic infection due to <i>Pseudomonas aeruginosa</i>

3. Follow-up, completion and deferral of PIP

Measures to address long term follow-up of potential safety issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By January 2017
Deferral for one or more studies contained in the paediatric investigation plan:	Yes