



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/299522/2011

European Medicines Agency decision P/106/2011

of 4 May 2011

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for ataluren (EMA-000115-PIP02-09) 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 726/2004¹,

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 Genzyme Europe B.V. on 9 April 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 18 March 2011, 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 ataluren, granules for oral suspension, oral 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 ataluren, granules for oral suspension, oral 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 ataluren, granules for oral suspension, oral 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 Genzyme Europe B.V., Gooimeer 10, 1411 DD Naarden, The Netherlands.

Done at London, 4 May 2011

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



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/13859/2011

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

EMA-000115-PIP02-09

Scope of the application

Active substance(s):

Ataluren

Condition(s):

Treatment of cystic fibrosis

Pharmaceutical form(s):

Granules for oral suspension

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Genzyme Europe B.V.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Genzyme Europe B.V. submitted for agreement to the European Medicines Agency on 9 April 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 20 May 2010.

Supplementary information was provided by the applicant on 6 January 2011. The applicant proposed modifications to the paediatric investigation plan.



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 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 and appendix.

London, 18 March 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 cystic fibrosis

The waiver applies to:

- neonates from birth to less than 28 days of age;
- granules for oral suspension, oral 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 cystic fibrosis

2.1.1. Indication(s) targeted by the PIP

Treatment cystic fibrosis due to nonsense mutation (nmCF)

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

Children from 28 days to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Granules for oral suspension

2.1.4. Studies

Area	Number of studies	Description
Quality	1	Study 1: Age appropriate formulation and dosing device that allows accurate dosing for children weighing less than 20 kg.
Non-clinical	3	Study 2: 7-day tolerability and pharmacokinetic study of ataluren in neonatal Beagle dogs Study 3: 28-day dose range finding juvenile toxicology and toxicokinetic Study of ataluren in Beagle Dogs Study 4: 3-Month Juvenile Toxicology and Toxicokinetic Study of ataluren in Beagle Dogs with a 3-Month Recovery Period

Clinical	5	Study 5: Open-label, randomized, dose-ranging, challenge-dechallenge-rechallenge evaluation of pharmacodynamic (PD) activity, safety, and pharmacokinetics of ataluren. Study 6: Randomised, double-blind, placebo-controlled study to evaluate efficacy of ataluren adult and paediatric nmCF patients, ≥ 6 years of age Study 7: Open-label extension study to evaluate safety of ataluren in adult and paediatric nmCF patients, ≥ 6 years of age. Study 8: Open label, multiple dose trial to evaluate the pharmacokinetics (PK), safety and pharmacodynamic (PD) effect of ataluren in paediatric patients with nmCF aged from 2 to less than 6 years. Study 9: Open label, multiple dose trial to evaluate the pharmacokinetics (PK) and safety of ataluren in paediatric patients with nmCF aged from 28 days to less than 23 months.
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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 December 2016
Deferral for one or more studies contained in the paediatric investigation plan:	Yes