EMA/133060/2016

European Medicines Agency decision

P/0052/2016

of 18 March 2016

on the acceptance of a modification of an agreed paediatric investigation plan for basmisanil (EMEA-001506-PIP02-14-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/0075/2015 issued on 1 April 2015,

Having regard to the application submitted by Roche Registration Ltd on 5 November 2015 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 29 January 2016, 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 basmisanil, granules, oral 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 Roche Registration Ltd, 6 Falcon Way, Shire Park, AL7 1TW - Welwyn Garden City, United Kingdom.

Done at London, 18 March 2016

For the European Medicines Agency
Zaïde Frias
Head of Division
Human Medicines Research and Development Support
(Signature on file)

EMA/PDCO/750664/2015
London, 29 January 2016

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

EMEA-001506-PIP02-14-M01

Scope of the application

Active substance(s):

Basmisanil

Condition(s):

Treatment of Down syndrome

Pharmaceutical form(s):

Granules

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Roche Registration Ltd

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Roche Registration Ltd submitted to the European Medicines Agency on 5 November 2015 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/0075/2015 issued on 1 April 2015.

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

The procedure started on 30 November 2015.

Scope of the modification

Some measures 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 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 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 Down syndrome

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- granules, oral use;
- on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric investigation plan

2.1. Condition

Treatment of Down syndrome

2.1.1. Indication(s) targeted by the PIP

Treatment of intellectual disability associated with Down syndrome

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

From 2 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Granules

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	4	**Study 1** Technical feasibility validation of the sachet filling step of granules manufacturing. **Study 2** Dose recovery study of the granule formulation. **Study 3** Compatibility study of the fine granule formulation with food. **Study 4** Palatability (taste and acceptability) testing of the granule formulation as part of a clinical pharmacology study in healthy adult volunteers (Study WP28978).

Non-clinical studies	1	**Study 5** Definitive juvenile toxicity study (9000198).
Clinical studies	5	**Study 6** Randomised, double-blind, placebo controlled, multicentre trial, 2 doses design to assess the efficacy, safety and tolerability of basmisanil, and validity of pharmacokinetic model predictions of dose selection in adolescents (BP27832). **Study 7** Randomised, double-blind, placebo-controlled, parallel group study to explore the pharmacokinetics, pharmacodynamic effects, efficacy, safety and tolerability of basmisanil compared with placebo in children with Down syndrome aged from 6 to less than 12 years of age (WP28760). **Study 8** Double-blind, placebo-controlled, randomised, multicentre long-term efficacy and safety study of basmisanil as monotherapy in adolescents with Down syndrome aged from 12 to less than 18 years of age (and adults) (P3STUD1). **Study 9** Double-blind, placebo-controlled, randomised, multicentre long-term efficacy and safety study of basmisanil as monotherapy in children with Down syndrome aged from 6 to less than 12 years of age (P3STUD2). **Study 10** Double-blind, placebo-controlled, randomised, multicentre long-term efficacy and safety study of basmisanil as monotherapy in children with Down syndrome aged from 2 to less than 6 years of age (P3STUD3).
Extrapolation, modelling and simulation studies	3	**Study 11** Physiologically based pharmacokinetic (PBPK) model. **Study 12** Population pharmacokinetic (PopPK) model. **Study 13** Population pharmacokinetic/pharmacodynamic (PopPK/PD) model.
Other studies	0	Not applicable.
Other measures	0	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By December 2024
Deferral for one or more measures contained in the paediatric investigation plan:	Yes