EMA/585770/2016

European Medicines Agency decision

P/0257/2016

of 5 October 2016

on the acceptance of a modification of an agreed paediatric investigation plan for zuretinol (acetate) (EMEA-001453-PIP01-13-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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on the acceptance of a modification of an agreed paediatric investigation plan for zuretinol (acetate) (EMEA-001453-PIP01-13-M01) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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/0023/2014 issued on 22 January 2014,

Having regard to the application submitted by QLT Ophthalmics (UK), Ltd. on 30 May 2016 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 19 August 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 and to the deferral.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the deferral.

[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 zuretinol (acetate), oral solution, oral use, including changes to the deferral, 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 QLT Ophthalmics (UK), Ltd., c/o Hackwood Secretaries, Ltd., One Silk Street, EC2Y 8HQ - London, United Kingdom.

EMA/PDCO/399206/2016
London, 19 August 2016

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

EMEA-001453-PIP01-13-M01

Scope of the application

Active substance(s):

Zuretinol (acetate)

Condition(s):

Treatment of retinitis pigmentosa

Treatment of Leber's congenital amaurosis

Pharmaceutical form(s):

Oral solution

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

QLT Ophthalmics (UK), Ltd.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, QLT Ophthalmics (UK), Ltd. submitted to the European Medicines Agency on 30 May 2016 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0023/2014 issued on 22 January 2014.

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

The procedure started on 21 June 2016.

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Scope of the modification

Some measures and timelines 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 and to the deferral 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 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

Not applicable.

2. Paediatric Investigation Plan

2.1. Condition:

Treatment of retinitis pigmentosa

2.1.1. Indication(s) targeted by the PIP

Treatment of inherited retinal disease caused by Lecithin:retinol Acyltransferase (LRAT) or Retinal Pigment Epithelium Protein 65 (RPE65) Mutations

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Oral solution

2.1.4. Measures

Area	Number of measures	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	4	Measure 1 Open-label, multi-centre, single treatment course trial in children 5 years and older and adults with Leber's congenital amaurosis or retinitis pigmentosa with LRAT or RPE65 mutations (IRD 01). Measure 2 Open-label, multi-centre, repeated treatment course trial in children 5 years and older and adults with Leber's congenital amaurosis and retinitis pigmentosa with LRAT and RPE65 mutations (IRD 02). Measure 3 Multi-centre, randomised, double-masked, placebo-controlled trial to evaluate efficacy, safety and pharmacokinetics in children from 6 years to less than 18 years of age (and adults) with inherited retinal disease due to LRAT or RPE65 mutation with a safety extension (IRD 04). Measure Study 4 (deleted within procedure EMEA-001453-PIP01-13-M01)

		Measure 5 Open label safety study in children from birth to less than 6 years of age with inherited retinal disease due to LRAT and RPE65 mutation (IRD 06).

2.2. Condition

Treatment of Leber's congenital amaurosis

2.2.1. Indication(s) targeted by the PIP

Treatment of inherited retinal disease caused by Lecithin:retinol Acyltransferase (LRAT) or Retinal Pigment Epithelium Protein 65 (RPE65) Mutations

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

From birth to less than 18 years of age.

2.2.3. Pharmaceutical form(s)

Oral solution

2.2.4. Measures

Area	Number of measures	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	4	Measure 1 Open-label, multi-centre, single treatment course trial in children 5 years and older and adults with Leber's congenital amaurosis or retinitis pigmentosa with LRAT or RPE65 mutations (IRD 01). Measure 2 Open-label, multi-centre, repeated treatment course trial in children 5 years and older and adults with Leber's congenital amaurosis and retinitis pigmentosa with LRAT and RPE65 mutations (IRD 02). Measure 3 Multi-centre, randomised, double-masked, placebo-controlled trial to evaluate efficacy, safety and pharmacokinetics in children from 6 years to less than 18 years (and adults) with inherited retinal disease due to LRAT or RPE65 mutation with a safety extension (IRD 04). Measure 4 (deleted within procedure EMEA-001453-PIP01-13-M01)

		Measure 5 Open label safety study in children from birth to less than 6 years of age with inherited retinal disease due to LRAT and RPE65 mutation (IRD 06).

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By May 2022
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

6. Reminder

Interventional studies and trials have to be registered with the EudraCT database.

All investigational medicinal products used in the studies or trials must be registered in the EudraVigilance Medicinal Product Dictionary by the sponsor.

Studies or trials which are inconclusive or not interpretable will be considered non-compliant.

The Clinical trials should be performed in accordance with Good Clinical Practice. Those conducted outside the community should be carried out in accordance with the ethical standards of Directive 2001/20/EC and those conducted within the community should be carried out in accordance with Directive 2001/20/EC.

Applicants are reminded that it is their responsibility to submit a request for modification of an agreed paediatric investigation plan, if they encounter difficulties with its implementation as to render the plan unworkable or no longer appropriate.

Applicants are also reminded that in order to verify compliance with the agreed PIP, the final study report must be submitted (Commission Guideline 2008/C243/01 of 24 September 2008) for any study that had to be completed. This is part of the validation of the application for e.g. marketing authorisation, or line extension). Applicants are therefore advised to consider the time necessary to produce the final report for their planned date of submission. For authorised medicinal products, the report needs to be submitted within 6 months of completion of the study according to article 46 of Regulation (EC) No 1901/2006.