EMA/738903/2013

European Medicines Agency decision

P/0023/2014

of 22 January 2014

on the agreement of a paediatric investigation plan and on the granting of a deferral for 9-cis-retinyl acetate (EMEA-001453-PIP01-13) 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 application submitted by QLT Ophthalmics (UK) Ltd. on 12 April 2013 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 6 December 2013, in accordance with Article 18 of Regulation (EC) No 1901/2006, and Article 21 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.

(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.

[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 9-cis-retinyl acetate, oral solution, 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 9-cis-retinyl acetate, oral solution, 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

This decision is addressed to QLT Ophthalmics (UK) Ltd., c/o Hackwood Secretaries, Ltd., One Silk Street, EC2Y 8HQ – London, United Kingdom,

Done at London, 22 January 2014

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)

EMA/PDCO/577630/2013

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

EMEA-001453-PIP01-13

Scope of the application

Active substance(s):

9-cis-retinyl 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 16(1) of Regulation (EC) No 1901/2006 as amended, QLT Ophthalmics (UK), Ltd. submitted for agreement to the European Medicines Agency on 12 April 2013 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 22 May 2013.

Supplementary information was provided by the applicant on 16 September 2013. The applicant proposed modifications to the paediatric investigation plan and requested a deferral and withdrew its request for a waiver.

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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.

   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 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 annexes and appendix.

London, 6 December 2013

On behalf of the Paediatric Committee
Dr Dirk Mentzer, 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

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	5	Measure 1 Open-label, multi-centre, single treatment course trial in children 5 years and older and adults with Leber congenital amaurosis or retinitis pigmentosa with LRAT or RPE65 mutations. Measure 2 Open-label, multi-centre, repeated treatment course trial in children 5 years and older and adults with Leber congenital amaurosis and retinitis pigmentosa with LRAT and RPE65 mutations. Measure 3 Multi-centre, randomised, double-masked, placebo-controlled trial to evaluate efficacy, safety and pharmacokinetics in children 6 years and older (and adults) with inherited retinal disease due to LRAT or RPE65 mutation with a safety extension. Measure 4 Multi-centre, randomised, double-masked, placebo-controlled trial to evaluate efficacy, safety and pharmacokinetics in children 6 years and older (and adults) with inherited retinal disease due to LRAT or RPE65 mutation with a safety extension.

Area	Number of measures	Description
		Measure 5 Open label safety study in children 5 years and younger with inherited retinal disease due to LRAT and RPE65 mutation.

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	5	Measure 1 Open-label, multi-centre, single treatment course trial in children 5 years and older and adults with Leber congenital amaurosis or retinitis pigmentosa with LRAT or RPE65 mutations. Measure 2 Open-label, multi-centre, repeated treatment course trial in children 5 years and older and adults with Leber congenital amaurosis and retinitis pigmentosa with LRAT and RPE65 mutations. Measure 3 Multi-centre, randomised, double-masked, placebo-controlled trial to evaluate efficacy, safety and pharmacokinetics in children 6 years and older (and adults) with inherited retinal disease due to LRAT or RPE65 mutation with a safety extension.

Area	Number of measures	Description
		Measure 4 Multi-centre, randomised, double-masked, placebo-controlled trial to evaluate efficacy, safety and pharmacokinetics in children 6 years and older (and adults) with inherited retinal disease due to LRAT or RPE65 mutation with a safety extension. Measure 5 Open label safety study in children 5 years and younger with inherited retinal disease due to LRAT and RPE65 mutation.

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 2018
Deferral for one or more measures contained in the paediatric investigation plan:	Yes