EMA/299187/2019

European Medicines Agency decision

P/0213/2019

of 14 June 2019

on the agreement of a paediatric investigation plan and on the granting of a waiver for lentiviral vector containing the human ABCA4 gene (SAR422459) (EMEA-002407-PIP01-18) 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 Sanofi-Aventis Recherche & Développement on 16 July 2018 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 26 April 2019, in accordance with Article 17 of Regulation (EC) No 1901/2006 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 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 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 lentiviral vector containing the human ABCA4 gene (SAR422459), solution for injection, intraocular 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 waiver for lentiviral vector containing the human ABCA4 gene (SAR422459), solution for injection, intraocular 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 Sanofi-Aventis Recherche & Développement, 1, avenue Pierre Brossolette, 91385 – Paris, France.

EMA/PDCO/122624/2019
London, 26 April 2019

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

EMEA-002407-PIP01-18

Scope of the application

Active substance(s):

Lentiviral vector containing the human ABCA4 gene (SAR422459)

Condition(s):

Treatment of inherited retinal disorders

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Intraocular use

Name/corporate name of the PIP applicant:

Sanofi-Aventis Recherche & Développement

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Sanofi-Aventis Recherche & Développement submitted for agreement to the European Medicines Agency on 16 July 2018 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 21 August 2018.

Supplementary information was provided by the applicant on 15 January 2019. 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 17(1) 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)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset(s) of the paediatric population.

   The Norwegian Paediatric Committee member agrees 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.

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 inherited retinal disorders

The waiver applies to:

- the paediatric population from birth to less than 3 years;
- solution for injection, intraocular use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of inherited retinal disorders

2.1.1. Indication(s) targeted by the PIP

Treatment of patients with autosomal-recessive ABCA4 gene mutations, including patients with Stargardt macular degeneration

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

From 3 years to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Solution for injection

2.1.4. Measures

Area	**Number of measures**	**Description**
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable
Clinical studies	2	**Study 1 (TDU13583)** Open label dose escalation study of subretinally injected SAR422459 in children and adolescents from 6 years to less than 18 years of age (and adults) with Stargardt's Macular Degeneration (SMD)

		Study 2 (EFC14740) Multi-centre, multi-national, open-label, no-treatment-controlled, rater-blinded study of the efficacy and safety of subretinally injected SAR422459 administration in children and adolescents from 3 years to less than 18 years of age (and young adults) diagnosed with Stargardt's Macular Degeneration (SMD).
Extrapolation, modelling and simulation studies	0	Not applicable
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 February 2023
Deferral for one or more measures contained in the paediatric investigation plan:	No