EMA/343784/2024

European Medicines Agency decision

P/0291/2024

of 16 August 2024

on the acceptance of a modification of an agreed paediatric investigation plan for dexamethasone (sodium phosphate) encapsulated in human autologous erthrocytes (EMEA-001957-PIP02-19-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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the European Medicines Agency's decision P/0211/2020 issued on 16 June 2020,

Having regard to the application submitted by Quince Therapeutics, S.p.A. on 25 March 2024 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 28 June 2024, 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for dexamethasone (sodium phosphate) encapsulated in human autologous erthrocytes, dispersion for infusion, intravenous 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 Quince Therapeutics, S.p.A., 3 Via A. Meucci, 20091 - Bresso (MI) Italy.

EMA/PDCO/142139/2024
Amsterdam, 28 June 2024

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

EMEA-001957-PIP02-19-M01

Scope of the application

Active substance(s):

Dexamethasone (sodium phosphate) encapsulated in human autologous erthrocytes

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of ataxia telangiectasia

Pharmaceutical form(s):

Dispersion for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Quince Therapeutics, S.p.A.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Quince Therapeutics, S.p.A. submitted to the European Medicines Agency on 25 March 2024 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/0211/2020 issued on 16 June 2020.

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

The procedure started on 29 April 2024.

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 Paediatric Committee member of Norway 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 annexes 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 ataxia telangiectasia

The waiver applies to:

- the paediatric population from birth to less than 9 kg of body weight;
- dispersion for infusion, intravenous use;
- on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric investigation plan

2.1. Condition:

Treatment of ataxia telangiectasia

2.1.1. Indication(s) targeted by the PIP

Treatment of ataxia telangiectasia

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

From 9 kg of body weight to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Dispersion for infusion

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**Study 1:** Open-label, uncontrolled trial to evaluate pharmacokinetics, safety and activity of dexamethasone (sodium phosphate) encapsulated in human autologous erythrocytes in children above 3 to less than 18 years of age (and adults) with ataxia telangiectasia (IEDAT-ERY01-2010). **Study 2:** Double-blind, randomised, placebo-controlled trial to evaluate pharmacokinetics, safety and efficacy of two dose ranges of dexamethasone (sodium phosphate) encapsulated in human autologous erythrocytes compared to placebo in children from 6 to

	less than 18 years of age (and adults) with ataxia telangiectasia (IEDAT-02-2015 / ATTeST). **Study 3:** Open-label, uncontrolled trial to evaluate pharmacokinetics and safety of dexamethasone (sodium phosphate) encapsulated in human autologous erythrocytes in children from 9 to 15 kg of body weight with ataxia telangiectasia (IEDAT-05-2019). **Study 5** (added in procedure EMEA-001957-PIP02-19-M01) Double-blind, randomised, placebo-controlled trial to evaluate the neurological effects of dexamethasone (sodium phosphate) encapsulated in human autologous erythrocytes (EryDex) in children from 6 years to less than 18 years of age with ataxia telangiectasia (NEAT trial/ IEDAT-04-2022)
Extrapolation, modelling and simulation studies	**Study 4:** Modelling and simulation study to support the use of the dexamethasone (sodium phosphate) encapsulated in human autologous erythrocytes for the treatment of ataxia telangiectasia in children from 9 kg to less than 15 kg of body weight.
Other studies	Not applicable
Other measures	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long-term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By May 2027
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.