



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/336054/2023

European Medicines Agency decision P/0314/2023

of 9 August 2023

on the agreement of a paediatric investigation plan and on the granting of a deferral for mRNA encoding modified human ornithine transcarbamylase (ARCT-810), (EMA-003315-PIP01-22) 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 726/2004¹,

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 Agency²,

Having regard to the application submitted by Arcturus Therapeutics Europe B.V. on 8 September 2022 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 23 June 2023, in accordance with Article 17 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, as amended.

² OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for mRNA encoding modified human ornithine transcarbamylase (ARCT-810), solution for infusion, intravenous 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 mRNA encoding modified human ornithine transcarbamylase (ARCT-810), solution for infusion, intravenous 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 Arcturus Therapeutics Europe B.V., Claude Debussylaan 10, 1082MD - Amsterdam, The Netherlands.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/137216/2023
Amsterdam, 23 June 2023

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

EMA-003315-PIP01-22

Scope of the application

Active substance(s):

mRNA encoding modified human ornithine transcarbamylase (ARCT-810)

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of ornithine transcarbamylase deficiency

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Arcturus Therapeutics Europe B.V.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Arcturus Therapeutics Europe B.V. submitted for agreement to the European Medicines Agency on 8 September 2022 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 17 October 2022.

Supplementary information was provided by the applicant on 16 March 2023. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a waiver.



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 deferral in accordance with Article 21 of said Regulation.

The Paediatric Committee member of Norway agrees 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.

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 ornithine transcarbamylase (OTC) deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment of ornithine transcarbamylase deficiency

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. Measures

Area	Description
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	<p>Study 1 (ARCT-810-03)</p> <p>Randomized, double blinded, placebo controlled, nested single and multiple ascending dose study to assess safety, tolerability and pharmacokinetics (PK) of ARCT-810 in adolescent patients from 12 years to less than 18 years of age (and adults) with ornithine transcarbamylase (OTC) deficiency.</p> <p>Study 2</p> <p>Randomized, unblinded, controlled study to assess efficacy, safety, tolerability and pharmacokinetics (PK) of ARCT-810 in adolescent patients from 12 years to less than 18 years of age (and adults) with ornithine transcarbamylase (OTC) deficiency.</p> <p>Study 3</p> <p>Randomized, unblinded, controlled study to assess efficacy, safety, tolerability and pharmacokinetics (PK) of ARCT-810 in children from 5 years to less than 12 years of age with ornithine transcarbamylase (OTC) deficiency.</p> <p>Study 4</p> <p>Randomized, unblinded, controlled study to assess efficacy, safety, tolerability and pharmacokinetics (PK) of ARCT-810 in children from birth to less than 5 years of age) with ornithine transcarbamylase</p>

	<p>(OTC) deficiency.</p> <p>Study 5</p> <p>Open label extension study up to 2 years to assess safety and efficacy of ARCT-810 in children and adolescents from birth to less than 18 years of age (and adults) with ornithine transcarbamylase (OTC) deficiency.</p>
Modelling and simulation studies	<p>Study 6</p> <p>Modelling and simulation population PK study, to evaluate the use of ARCT-810 in children and adolescents from birth to less than 18 years of age with ornithine transcarbamylase (OTC) deficiency.</p>
Other studies	Not applicable
Extrapolation plan	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 January 2032
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.