EMA/443765/2024

European Medicines Agency decision

P/0357/2024

of 25 October 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral for (R)-3-(1-cyclopropyl-3-(2-fluoro-4-(trifluoromethoxy)benzyl)ureido)piperidine-1-carboxamide (JNT-517), (EMEA-003531-PIP01-23) 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 application submitted by Jnana Therapeutics Inc on 8 November 2023 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 6 September 2024, 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 (R)-3-(1-cyclopropyl-3-(2-fluoro-4-(trifluoromethoxy)benzyl)ureido)piperidine-1-carboxamide (JNT-517), oral powder, tablet, granules for oral suspension, 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 (R)-3-(1-cyclopropyl-3-(2-fluoro-4-(trifluoromethoxy)benzyl)ureido)piperidine-1-carboxamide (JNT-517), oral powder, tablet, granules for oral suspension, 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 Jnana Therapeutics Inc., One Design Center Place , Suite 19-400, 02210 – Boston, USA.

EMA/PDCO/270230/2024
Amsterdam, 6 September 2024

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

EMEA-003531-PIP01-23

Scope of the application

Active substance(s):

(R)-3-(1-cyclopropyl-3-(2-fluoro-4-(trifluoromethoxy)benzyl)ureido)piperidine-1-carboxamide (JNT-517)

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of hyperphenylalaninemia

Pharmaceutical form(s):

Oral powder

Tablet

Granules for oral suspension

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Jnana Therapeutics Inc

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Jnana Therapeutics Inc submitted for agreement to the European Medicines Agency on 8 November 2023 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 3 January 2024.

Supplementary information was provided by the applicant on 20 May 2024. 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 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 hyperphenylalaninemia

2.1.1. Indication(s) targeted by the PIP

Reduction of plasma phenylalanine concentrations in patients with phenylketonuria who have uncontrolled plasma phenylalanine concentrations.

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 powder

Tablet

Granules for oral suspension

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Development of age-appropriate formulation (granules for oral suspension) for use in children below 12 years of age or adolescents unable to swallow tablets.
Non-clinical studies	Study 2 Definitive juvenile animal study
Clinical studies	Study 3 (JNT517-201) Randomised, double-blind, placebo-controlled study to evaluate efficacy and safety of JNT-517 in adolescents from 12 to less than 18 years of age with phenylketonuria (PKU). Study 4 (JNT517-301) Randomised, double-blind, placebo-controlled, two-part study to evaluate efficacy and safety of JNT-517 in children from 4 years to less than 18 years of age (and adults) with PKU. Study 5 (JNT517-302) Open-label, single-arm study to evaluate safety and efficacy of JNT-517

	in paediatric participants from birth to less than 4 years of age with PKU.
Modelling and simulation analyses	Study 6 (JNT517-M&S-1) Modelling and simulation analyses to support dosing of JNT517 in children from 4 years to less than 18 years of age with PKU. Study 7 (JNT517-M&S-2) Modelling and simulation analyses to support dosing of JNT517 in children from birth to less than 4 years of age with PKU.
Other studies	Not applicable
Extrapolation plan	Studies 3, 4, 5, 6 and 7 are part of an extrapolation plan covering the paediatric population from birth to less than 18 years of age, as agreed by the PDCO.

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.