EMA/385776/2023

European Medicines Agency decision

P/0369/2023

of 8 September 2023

on the acceptance of a modification of an agreed paediatric investigation plan for recombinant human ectonucleotide pyrophosphatase/phosphodiesterase 1 fused to the Fc fragment of IgG1 (INZ-701) (EMEA-003232-PIP01-22-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/0125/2023 issued on 18 April 2023,

Having regard to the application submitted by Inozyme Pharma Ireland Limited on 24 April 2023 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 21 July 2023, 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.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[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 recombinant human ectonucleotide pyrophosphatase/phosphodiesterase 1 fused to the Fc fragment of IgG1 (INZ-701), powder for solution for injection, subcutaneous use, 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 Inozyme Pharma Ireland Limited, 29 Earlsfort Terrace, 2 – Dublin, Ireland.

EMA/PDCO/203517/2023
Amsterdam, 21 July 2023

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

EMEA-003232-PIP01-22-M01

Scope of the application

Active substance(s):

Recombinant human ectonucleotide pyrophosphatase/phosphodiesterase 1 fused to the Fc fragment of IgG1 (INZ-701)

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of ectonucleotide pyrophosphatase/phosphodiesterase 1 deficiency

Pharmaceutical form(s):

Powder for solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Inozyme Pharma Ireland Limited

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Inozyme Pharma Ireland Limited submitted to the European Medicines Agency on 24 April 2023 an application for modification of the agreed paediatric investigation plan as set out in the European Medicines Agency's decision P/0125/2023 issued on 18 April 2023.

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

The procedure started on 22 May 2023.

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 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 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 ectonucleotide pyrophosphatase/phosphodiesterase 1 deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment of ectonucleotide pyrophosphatase/phosphodiesterase 1 (ENPP1) 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. Pharmaceutical form(s)

Powder for solution for injection

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Development of a lower strength formulation appropriate for the paediatric population
Non-clinical studies	**Study 2 (20254642)** Definitive juvenile toxicity study in mice to evaluate the safety of INZ-701 in paediatric population
Clinical studies	**Study 6 (INZ701-104, ENERGY)** *Study added during procedure EMEA-003232-PIP01-22-M01.* Open-label, multi-centre, non-comparative study to assess pharmacokinetics (PK), and safety and activity of INZ-701 in infants from 1 month to less than 1 year of age with ectonucleotide pyrophosphatase/phosphodiesterase 1 (ENPP1) deficiency and to provide PK data to contribute to modelling of the PK in children from 1 month to less than 1 year of age. **Study 3 (INZ701-106, ENERGY 3)** Two-arm, randomised, controlled, open-label, multi-centre study to assess pharmacokinetics (PK), and safety and efficacy of INZ-701 in children from 1 year to less than 13 years of age with ectonucleotide

	pyrophosphatase/phosphodiesterase 1 (ENPP1) deficiency and to provide PK data to contribute to modelling of the PK in children from 1 year to less than 13 years of age. **Study 4 (INZ701-105, ENERGY 2)** Open-label, multi-centre, non-comparative study to assess pharmacokinetics (PK), and safety and activity of INZ-701 in children from birth to less than 1 year of age with ectonucleotide pyrophosphatase/phosphodiesterase 1 (ENPP1) deficiency and to provide PK data to contribute to modelling of the PK in children from birth to 1 year of age with ENPP1 deficiency. **Study 7 (INZ701-107, ENERGY 4)** *Study added during procedure EMEA-003232-PIP01-22-M01.* Two-arm, randomised, double-blind, placebo-controlled, multi-center study to assess safety and efficacy of INZ-701 compared to the control arm in adolescents from 13 years to less than 18 years of age (and adults) with ectonucleotide pyrophosphatase/phosphodiesterase 1 (ENPP1) deficiency.
Modelling and simulation studies	**Study 5 (INZ0101H)** Modelling and simulation population pharmacokinetics (PK) and pharmacodynamics (PD) study, to evaluate the use of the INZ-701 in children from birth to less than 18 years of age with ectonucleotide pyrophosphatase/phosphodiesterase 1 (ENPP1) deficiency.
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 March 2026
Deferral for one or more measures contained in the paediatric investigation plan:	No

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.