EMA/240867/2020

European Medicines Agency decision

P/0196/2020

of 15 May 2020

on the agreement of a paediatric investigation plan and on the granting of a deferral for idasanutlin (EMEA-001489-PIP02-19) 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.

European Medicines Agency decision

P/0196/2020

of 15 May 2020

on the agreement of a paediatric investigation plan and on the granting of a deferral for idasanutlin (EMEA-001489-PIP02-19) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 Roche Registration GmbH on 20 December 2019 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 27 March 2020, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for idasanutlin, film-coated tablet, film-coated dispersible tablet, 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 idasanutlin, film-coated tablet, film-coated dispersible tablet, 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 Roche Registration GmbH, Emil-Barell-Strasse 1, 79639 - Grenzach-Wyhlen, Germany.

EMA/PDCO/46119/2020
Amsterdam, 27 March 2020

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

EMEA-001489-PIP02-19

Scope of the application

Active substance(s):

Idasanutlin

Condition(s):

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue).

Pharmaceutical form(s):

Film-coated tablet

Film-coated dispersible tablet

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Roche Registration GmbH

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Roche Registration GmbH submitted for agreement to the European Medicines Agency on 20 December 2019 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 28 January 2019.

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 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 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

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Treatment of all conditions included in the category of malignant neoplasms (except haematopoietic and lymphoid tissue)

2.1.1. Indication(s) targeted by the PIP

Treatment of children with a solid malignant tumour which is newly-diagnosed and metastatic, or refractory to first-line treatment

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)

Film-coated tablet

Film-coated dispersible tablet

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1** (same study as Study 1 of PIP EMEA-001489-PIP01-13-M02) Development of age-appropriate, liquid or solid dosage form for oral use
Non-clinical studies	1	**Study 2** (same study as Study 2 of PIP EMEA-001489-PIP01-13-M02) Juvenile toxicity study
Clinical studies	2	**Study 3** (same study as study Study 3 of PIP EMEA-001489-PIP01-13-M02) Open-label, non-controlled, multi-centre trial to evaluate the pharmacokinetics, toxicity, safety and any activity of idasanutlin in children from birth to less than 18 years of age with a solid tumour or acute leukaemia for which no effective treatment is known, with an expansion cohort(s) in defined tumour types using idasanutlin combined with anti-cancer medicine(s)

		Study 4 Open-label, randomised, controlled add-on trial to evaluate the safety and efficacy of idasanutlin in children from birth to less than 18 years of age with a first relapse of, or with frontline-refractory solid tumours.
Modelling and simulation studies	1	**Study 5** (same study as Study 5 of PIP EMEA-001489-PIP01-13-M02) Dose finding through modeling and simulation based on paediatric pharmacokinetic (PK) data from studies 3 & 4

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 2029
Deferral for one or more measures contained in the paediatric investigation plan:	Yes