EMA/74170/2020

European Medicines Agency decision

P/0081/2020

of 18 March 2020

on the acceptance of a modification of an agreed paediatric investigation plan for idasanutlin (EMEA-001489-PIP01-13-M02) 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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on the acceptance of a modification of an agreed paediatric investigation plan for idasanutlin (EMEA-001489-PIP01-13-M02) 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 European Medicines Agency's decision P/0285/2014 issued on 28 October 2014 and the decision P/0366/2018 issued on 7 December 2018,

Having regard to the application submitted by Roche Registration GmbH on 25 October 2019 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 31 January 2020, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for idasanutlin, film-coated tablet, film-coated dispersible tablet, oral 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 Roche Registration GmbH, Emil-Barell-Strasse 1, 79639 - Grenzach-Wyhlen, Germany.

EMA/PDCO/602657/2019
Amsterdam, 31 January 2020

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

EMEA-001489-PIP01-13-M02

Scope of the application

Active substance(s):

Idasanutlin

Condition(s):

Treatment of acute myeloid leukaemia

Treatment of acute lymphoblastic leukaemia

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 22 of Regulation (EC) No 1901/2006 as amended, Roche Registration GmbH submitted to the European Medicines Agency on 25 October 2019 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0285/2014 issued on 28 October 2014 and the decision P/0366/2018 issued on 7 December 2018.

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

The procedure started on 3 December 2019.

Scope of the modification

Some timelines of the Paediatric Investigation Plan have been modified. Amendment of the scope of the Paediatric Investigation Plan to exclude a condition.

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 Norwegian Paediatric Committee member 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 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 acute myeloid leukaemia

2.1.1. Indication(s) targeted by the PIP

Treatment of children with first relapse of, or with frontline-refractory acute myeloid leukaemia

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** Development of age-appropriate, liquid or solid dosage form for oral use
Non-clinical studies	1	**Study 2** Juvenile toxicity study
Clinical studies	2	**Study 3** 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 acute leukaemia (either lymphoblastic or myeloid)

Modelling and simulation studies	1	**Study 6** Dose finding through modeling and simulation based on paediatric pharmacokinetic (PK) data from studies 3 & 4

2.2. Condition

Treatment of acute lymphoblastic leukaemia

2.2.1. Indication(s) targeted by the PIP

Treatment of children with first relapse of, or with frontline-refractory acute lymphoblastic leukaemia

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

From birth to less than 18 years of age

2.2.3. Pharmaceutical form(s)

Film-coated tablet

Film-coated dispersible tablet

2.2.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1** As per condition "treatment of acute myeloid leukaemia".
Non-clinical studies	1	**Study 2** As per condition "treatment of acute myeloid leukaemia".
Clinical studies	2	**Study 3** As per condition "treatment of acute myeloid leukaemia". **Study 4** As per condition "treatment of acute myeloid leukaemia".
Modelling and simulation studies	1	**Study 6** As per condition "treatment of acute myeloid leukaemia".

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