EMA/167090/2020

European Medicines Agency decision

P/0076/2020

of 6 April 2020

on the acceptance of a modification of an agreed paediatric investigation plan for Macimorelin (Macimorelin Aeterna Zentaris) (EMEA-001988-PIP01-16-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 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/0105/2017 issued on 11 April 2017,

Having regard to the application submitted by Aeterna Zentaris GmbH on 28 October 2019 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 27 March 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, following a re-examination procedure of the Paediatric Committee's opinion according to Article 25(3) of Regulation (EC) No 1901/2006, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for macimorelin (Macimorelin Aeterna Zentaris), granules for oral solution, oral 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 Aeterna Zentaris GmbH, Weismuellerstrasse 50, 60314 - Frankfurt am Main, Germany.

EMA/139676/2020
Amsterdam, 27 March 2020

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

EMEA-001988-PIP01-16-M01

Scope of the application

Active substance(s):

Macimorelin

Invented name:

Macimorelin Aeterna Zentaris

Condition(s):

Diagnosis of growth hormone deficiency

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Granules for oral solution

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Aeterna Zentaris GmbH

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Aeterna Zentaris GmbH submitted to the European Medicines Agency on 28 October 2019 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0105/2017 issued on 11 April 2017.

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

An Opinion was adopted by the Paediatric Committee on 31 January 2020 for the above mentioned product. Aeterna Zentaris GmbH received the Paediatric Committee Opinion on 12 February 2020.

On 12 March 2020 Aeterna Zentaris GmbH submitted to the European Medicines Agency a written request including detailed grounds for a re-examination of the Opinion.

The re-examination procedure started on 13 March 2020.

Scope of the modification

Some measures of the Paediatric Investigation Plan have been modified.

Final Opinion

1. The Paediatric Committee, having assessed the detailed grounds for re-examination, in accordance with Article 25(3) of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

1.1. to revise its opinion and

- to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion;

1.2. following re-examination, to amend the scope of the modifications of the paediatric investigation plan.

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 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 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

1.1. Condition

Diagnosis of growth hormone deficiency

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- granules for oral solution, oral use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition

Diagnosis of growth hormone deficiency

2.1.1. Indication(s) targeted by the PIP

Diagnosis of growth hormone deficiency

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

From 2 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Granules for oral solution

2.1.4. Measures

Area	**Number of measures**	**Description**
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable
Clinical studies	2	**Study 1** Open label, single dose trial to investigate the pharmacokinetics, pharmcodynamics, safety and tolerability of macimorelin acetate after ascending single oral doses of macimorelin in paediatric patients from 2 to less than 18 years of age with suspected growth hormone deficiency (AEZS-130-P01)

		Study 2 Open label, single dose trial to determine the diagnostic efficacy and safety of macimorelin acetate in paediatric patients from 2 to less than 18 years of age with suspected growth hormone deficiency (AEZS-130-P02)
Extrapolation, modelling and simulation studies	0	Not applicable
Other studies	0	Not applicable
Other measures	0	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 July 2022
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Diagnosis of growth hormone deficiency

Authorised indication(s):

- Macimorelin Aeterna Zentaris is indicated for the diagnosis of growth hormone deficiency (GHD) in adults

Authorised pharmaceutical form(s):

Granules for oral solution

Authorised route(s) of administration:

Oral use