EMA/886036/2018

European Medicines Agency decision

P/0001/2019

of 4 January 2019

on the acceptance of a modification of an agreed paediatric investigation plan for venetoclax (Venclyxto), (EMEA-002018-PIP02-16-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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on the acceptance of a modification of an agreed paediatric investigation plan for venetoclax (Venclyxto), (EMEA-002018-PIP02-16-M01) 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/0235/2017 issued on 9 August 2017,

Having regard to the application submitted by AbbVie Ltd on 10 September 2018 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 14 December 2018, 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 venetoclax (Venclyxto), film-coated tablet, tablet for oral suspension, 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 AbbVie Ltd, AbbVie House, Vanwall Road, SL6 4UB - Maidenhead, United Kingdom.

EMA/PDCO/651731/2018
London, 14 December 2018

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

EMEA-002018-PIP02-16-M01

Scope of the application

Active substance(s):

Venetoclax

Invented name:

Venclyxto

Condition(s):

Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue

Treatment of solid malignant tumours

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Film-coated tablet

Tablet for oral suspension

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

AbbVie Ltd

Information about the authorised medicinal product:

See Annex II

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Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, AbbVie Ltd submitted to the European Medicines Agency on 10 September 2018 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0235/2017 issued on 9 August 2017.

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

The procedure started on 16 October 2018.

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 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 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 malignant neoplasms of the haematopoietic and lymphoid tissue

2.1.1. Indication(s) targeted by the PIP

Treatment of relapsed or refractory Acute Lymphocytic Leukemia (ALL)

Treatment of relapsed or refractory Acute Myeloid Leukemia (AML)

Treatment of relapsed or refractory Non-Hodgkin lymphoma (NHL)

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

Tablet for oral suspension

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1** Development of an age appropriate tablet for oral suspension.
Non-clinical studies	1	**Study 2** Definitive juvenile toxicity study to determine the potential effects of venetoclax on development.
Clinical studies	2	**Study 3** Open-label dose determination (Part 1) and cohort expansion (Part 2) study in paediatric patients from birth to 18 years old (and young adults) with select relapsed or refractory solid and haematologic malignancies. (M13-833) **Study 4** Evaluation of efficacy of venetoclax in paediatric patients from birth to less than 18 years of age (and young adults) with select paediatric solid or haematologic tumour type prioritized based on anti-tumour activity in Study 3.

Extrapolation, modelling and simulation studies	0	Not applicable.
Other studies	0	Not applicable.
Other measures	0	Not applicable.

2.2. Condition

Treatment of solid malignant tumours

2.2.1. Indication(s) targeted by the PIP

Treatment of patients with relapsed or refractory neuroblastoma in patients from birth to less than 18 years of age

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

Tablet for oral suspension

2.2.4. Measures

Area	**Number of measures**	**Description**
Quality-related studies	1	**Study 1** The same as for condition "Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue".
Non-clinical studies	1	**Study 2** The same as for condition "Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue".
Clinical studies	2	**Study 3** The same as for condition "Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue". (M13-833) **Study 4** The same as for condition "Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue".

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:	Yes
Date of completion of the paediatric investigation plan:	By October 2026
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. Treatment of malignant neoplasms of the haematopoietic tissue

Authorised indication(s):

- Venclyxto monotherapy is indicated for the treatment of chronic lymphocytic leukaemia (CLL) in the presence of 17p deletion or TP53 mutation in adult patients who are unsuitable for or have failed a B-cell receptor pathway inhibitor.
- Venclyxto monotherapy is indicated for the treatment of CLL in the absence of 17p deletion or TP53 mutation in adult patients who have failed both chemo-immunotherapy and a B-cell receptor pathway inhibitor.

Authorised pharmaceutical form(s):

Film-coated tablets

Authorised route(s) of administration:

Oral use