MHRA
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

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Decision Cover Letter

Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan and to the deferral

MHRA-100092-PIP01-21-M01

Scope of the Application

Active Substance(s)

VENETOCLAX

Condition(s)

Haematopoietic and lymphoid malignant neoplasms, Treatment of solid tumour malignant neoplasms

Pharmaceutical Form(s)

Film-coated tablets; Tablet for oral suspension

Route(s) of Administration

Oral use

Name / Corporate name of the PIP applicant

AbbVie Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, AbbVie Ltd submitted to the licensing authority on 19/04/2021 18:48 BST an application for a Modification

The procedure started on 19/10/2021 09:59 BST

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to accept change(s) to the agreed paediatric investigation plan and to the deferral

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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Final Decision Letter

MHRA-100092-PIP01-21-M01

Of 21/10/2021 07:02 BST

On the adopted decision for VENETOCLAX (MHRA-100092-PIP01-21-M01) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan)

This decision applies to a Modification for VENETOCLAX, Film-coated tablets; Tablet for oral suspension , Oral use .

This decision is addressed to AbbVie Ltd, AbbVie House, Vanwall Business Park, Vanwall, Maidenhead, United Kingdom, SL6 4UB

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

Condition 1: Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue. Condition 2 : Treatment of solid malignant tumours.

2.2 Indication(s) targeted by the PIP:

Condition 1: 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). Condition 2: Treatment of patients with relapsed or refractory neuroblastoma in patients from birth to less than 18 years of age.

2.3 Subset(s) of the paediatric population concerned by the paediatric development:

From birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Film-coated tablet; Tablet for oral suspension

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 (For Condition 1 and 2) Development of an age appropriate tablet for oral suspension.
Non-Clinical Studies	1	Study 2 (For Condition 1 and 2)Definitive juvenile toxicity study to determine the potential effects of venetoclax on development
Clinical Studies	3	Study 3 (M13-833) (For Condition1 and 2) Open-label dose determination (Part 1) and cohort expansion (Part 2) study in paediatric (and young adult) patients with select relapsed or refractory solid and haematologic malignancies; Study 4 (Condition 2 only) 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 M13-833 (study 3); Study 5 (B19-061) (Condition 1 and added in MHRA-100092-PIP01-21-M01) Randomized, open label, controlled, global, Phase 2 study to evaluate the efficacy of venetoclax (VEN) in combination with fludarabine and high dose cytarabine (FLA), and gemtuzumab ozogamicin (GO) (FLA

		+GO+VEN) compared with FLA+GO alone in paediatric and young adult patients with relapsed or refractory (R/R) acute myeloid leukaemia (AML).
Extrapolation, Modeling & Simulation Studies	0	Not applicable
Other Studies	0	Not applicable
Other Measures	0	Not applicable

3. Follow-up, completion and deferral of a PIP:

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	31/12/2029
Deferral of one or more studies contained in the paediatric investigation plan:	Yes