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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

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

MHRA-100260-PIP01-21-M01

Scope of the Application

Active Substance(s)

CEMIPLIMAB

Condition(s)

Treatment of all malignant neoplasms (except haematopoietic and lymphoid tissue)

Pharmaceutical Form(s)

Concentrate for solution for infusion

Route(s) of Administration

Intravenous use

Name / Corporate name of the PIP applicant

Regeneron Ireland DAC

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Regeneron Ireland DAC submitted to the licensing authority on 24/09/2021 22:15 BST an application for a Modification

The procedure started on 29/07/2022 15:04 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.

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

gov.uk/mhra

Final Decision Letter

MHRA-100260-PIP01-21-M01

Of 12/08/2022 11:44 BST

On the adopted decision for CEMIPLIMAB (MHRA-100260-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 CEMIPLIMAB, Concentrate for solution for infusion , Intravenous use .

This decision is addressed to Regeneron Ireland DAC, One Warrington Place, Dublin, Ireland, D02 HH27

ANNEX I

1. Waiver

1.1 Condition:

Not applicable

2. Paediatric Investigation Plan:

2.1 Condition(s):

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

2.2 Indication(s) targeted by the PIP:

Treatment of newly-diagnosed or recurrent high-grade glioma or with a newly-diagnosed diffuse intrinsic pontine gliomas

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

The paediatric population from birth to less than 18 years of age

2.4 Pharmaceutical Form(s):

Concentrate for solution for infusion

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	0	Not applicable
Non-Clinical Studies	2	Study 1 Collection and analysis of data from literature and databases of paediatric tumour samples relative to PD- 1/PD-L1 expression, tumour genetic mutations and tumour gene and tumour associated/ neoantigens expression. Study 2 Non-clinical biomarker study in paediatric tumour tissues.
Clinical Studies	1	Study 3 Multi-centre, open-label trial to evaluate the safety, pharmacokinetics, pharmacodynamics and anti-tumour activity of cemiplimab in patients from birth to less than 18 years of age with a recurrent or refractory solid or central nervous system tumour and with an expansion cohort for patients with recurrent or refractory solid tumour (Phase 1), and to evaluate the safety and efficacy of cemiplimab used in combination with radiotherapy in patients from birth to less than 18 years of age (and adults), using a staggered approach for children younger than 3 years of age, with a newly diagnosed diffuse intrinsic pontine glioma (DIPG), or a newly diagnosed or recurrent high-grade glioma (HGG) (Efficacy Phase).
Extrapolation, Modeling & Simulation Studies	1	Study 4 Population PK model to simulate and predict the exposure of cemiplimab in children from birth to less than 18 years of age with a solid tumour or a DIPG or a HGG.
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:	31/05/2025
Deferral of one or more studies contained in the paediatric investigation plan:	Yes