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

**gov.uk/mhra**

# Decision Cover Letter

## Decision of the licensing authority to:

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

MHRA-101773-PIP01-24-M01

# Scope of the Application

## Active Substance(s)

Tabelecleucel

## Condition(s)

Treatment of Epstein-Barr virus associated post-transplant lymphoproliferative disorder.

## Pharmaceutical Form(s)

Dispersion for injection

## Route(s) of Administration

INTRAVENOUS USE

## Name / Corporate name of the PIP applicant

Pierre Fabre Limited

## Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Pierre Fabre Limited submitted to the licensing authority on 14/01/2025 13:56 GMT an application for a Modification

The procedure started on 31/01/2025 16:48 GMT

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-101773-PIP01-24-M01

Of 10/02/2025 14:54 GMT

On the adopted decision for Tabelecleucel (MHRA-101773-PIP01-24-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 Tabelecleucel, Dispersion for injection , INTRAVENOUS USE .

This decision is addressed to Pierre Fabre Limited, 250 Longwater Avenue, Reading, UNITED KINGDOM, RG2 6GP

## ANNEX I

### 1. Waiver

### 1.1 Condition:

| |
|---|
| Not applicable. |

### 2. Paediatric Investigation Plan:

### 2.1 Condition(s):

| |
|---|
| Treatment of Epstein-Barr virus associated post-transplant lymphoproliferative disorder. |

### 2.2 Indication(s) targeted by the PIP:

Treatment of patients with Epstein-Barr virus-associated post-transplant lymphoproliferative disease (EBV+ PTLD) who have received one prior therapy for an EBV-associated lymphoproliferative disorder.

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

The paediatric population from birth to less than18 years of age.

### 2.4 Pharmaceutical Form(s):

Dispersion for injection.

### 2.5 Studies:

| Study Type | Number of Studies | Study Description |
|---|---|---|
| **Quality Measures** | 0 | Not applicable. |
| **Non-Clinical Studies** | 0 | Not applicable. |
| **Clinical Studies** | 5 | Study 1 (95-024) Open-label, single-arm trial to evaluate safety and activity of Epstein-Barr virus cytotoxic T lymphocytes (EBV-CTLs) in patients from birth to less than 18 years of age (and adults) with Epstein-Barr virus (EBV)-associated malignancy or at high risk of developing an EBV-associated lymphoproliferative disorder (including lymphoma) due to EBV viraemia following an allogeneic haematopoietic cell transplant (HCT) or solid organ transplant (SOT). Study 2 (11-130) Open-label, single-arm trial to evaluate safety and activity of tabelecleucel in patients from birth to less than 18 years of age (and adults) with EBV-associated malignancy or with an EBV viraemia following previous treatment for an EBV-associated lymphoproliferative disorder with chemotherapy and/ or rituximab. Study 3 (ATA129-EBV-302) Open-label, single-arm trial to evaluate safety and activity of tabelecleucel in SOT or HCT transplant patients from birth to less than 18 years of age (and adults) with biopsy-proven EBV-associated post-transplant lymphoproliferative |

| | | |
|---|---|---|
| | | disease (PTLD) following (1) SOT after failure of rituximab (Subgroup A) and rituximab plus chemotherapy (Subgroup B) or (2) allogeneic HCT after failure of rituximab. Study 4 (ATA129-EBV-205) Open-label, single-arm, adaptive two-stage trial of tabelecleucel to evaluate safety and activity in patients from birth to less than 18 years of age (and adults) with a) EBV+ PTLD involving the central nervous system (CNS) and b) EBV+ PTLD where first-line rituximab or chemotherapy are not appropriate (including CD20-negative disease). Study 5 (ATA129-RS002) Open label, non-interventional, retrospective chart review study of treatment outcomes in patients from birth to less than 18 years of age (and adults) with EBV-associated PTLD after HCT or SOT who are refractory to rituximab or rituximab plus chemotherapy or who have relapsed after treatment with those agents. |
| **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:** | 31/12/2028 |
| **Deferral of one or more studies contained in the paediatric investigation plan:** | Yes |