

EMA/694552/2020

## European Medicines Agency decision P/0002/2021

of 5 January 2021

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for autologous peripheral blood T cells CD4- and CD8-selected and CD3- and CD28-activated transduced with retroviral vector expressing anti-CD19 CD28/CD3-zeta chimeric antigen receptor and cultured (KTE-X19) (EMEA-001862-PIP03-20) 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<sup>1</sup>,

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<sup>2</sup>,

Having regard to the application submitted by Kite Pharma EU B.V. on 9 September 2020 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 11 December 2020, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation and Article 13 of said Regulation,

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 agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver.
- (2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.
- (3) It is therefore appropriate to adopt a decision granting a deferral.
- (4) It is therefore appropriate to adopt a decision granting a waiver.

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<sup>1</sup> OJ L 378, 27.12.2006, p.1.

<sup>2</sup> OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

**Article 1**

A paediatric investigation plan for autologous peripheral blood T cells CD4- and CD8-selected and CD3- and CD28-activated transduced with retroviral vector expressing anti-CD19 CD28/CD3-zeta chimeric antigen receptor and cultured (KTE-X19), dispersion for infusion, intravenous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby agreed.

**Article 2**

A deferral for autologous peripheral blood T cells CD4- and CD8-selected and CD3- and CD28-activated transduced with retroviral vector expressing anti-CD19 CD28/CD3-zeta chimeric antigen receptor and cultured (KTE-X19), dispersion for infusion, intravenous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

**Article 3**

A waiver for autologous peripheral blood T cells CD4- and CD8-selected and CD3- and CD28-activated transduced with retroviral vector expressing anti-CD19 CD28/CD3-zeta chimeric antigen receptor and cultured (KTE-X19), dispersion for infusion, intravenous use, the details of which are set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

**Article 4**

This decision is addressed to Kite Pharma EU B.V., Tufsteen 1, 2132 NT – Hoofddorp, The Netherlands.

EMA/PDCO/512886/2020  
Amsterdam, 11 December 2020

## Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan and a deferral and a waiver

EMA-001862-PIP03-20

### Scope of the application

#### Active substance(s):

Autologous peripheral blood T cells CD4- and CD8-selected and CD3- and CD28-activated transduced with retroviral vector expressing anti-CD19 CD28/CD3-zeta chimeric antigen receptor and cultured (KTE-X19)

#### Condition(s):

Treatment of mature B-cell neoplasms

#### Pharmaceutical form(s):

Dispersion for infusion

#### Route(s) of administration:

Intravenous use

#### Name/corporate name of the PIP applicant:

Kite Pharma EU B.V.

### Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Kite Pharma EU B.V. submitted for agreement to the European Medicines Agency on 9 September 2020 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 13 October 2020.

## Opinion

1. The Paediatric Committee, having assessed the proposed paediatric investigation plan in accordance with Article 17 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:
  - to agree the paediatric investigation plan in accordance with Article 17(1) of said Regulation;
  - to grant a deferral in accordance with Article 21 of said Regulation;
  - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

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 annex 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:

Treatment of mature B-cell neoplasms

The waiver applies to:

- the paediatric population weighing less than 6 kg;
- dispersion for infusion, intravenous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

# 2. Paediatric investigation plan

## 2.1. Condition:

Treatment of mature B-cell neoplasms

### 2.1.1. Indication(s) targeted by the PIP

Treatment of paediatric and adolescent subjects with relapsed or refractory B-cell non-Hodgkin lymphoma

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

Less than 18 years of age and weighing at least 6 kg.

### 2.1.3. Pharmaceutical form(s)

Dispersion for infusion

### 2.1.4. Measures

| Area                    | Number of measures | Description                                                                                                                                                                                                                                             |
|-------------------------|--------------------|---------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
| Quality-related studies | 1                  | <b>Study 1</b><br>Development of a formulation of KTE-X19 suitable for administration to paediatric patients with a minimum weight of 6 kg.                                                                                                             |
| Non-clinical studies    | 0                  | Not applicable.                                                                                                                                                                                                                                         |
| Clinical studies        | 1                  | <b>Study 2</b> (NHL portion of KTE-C19-104 (ZUMA-4))<br>Open-label, single arm trial to evaluate safety and activity of KTE-X19 in paediatric patients weighing at least 6 kg with relapsed or refractory B-cell non-Hodgkin lymphoma (r/r B-cell NHL). |

| Area                                            | Number of measures | Description     |
|-------------------------------------------------|--------------------|-----------------|
| 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 December 2023 |
| Deferral for one or more measures contained in the paediatric investigation plan:     | Yes              |