EMA/630189/2019

European Medicines Agency decision

P/0378/2019

of 4 December 2019

on the acceptance of a modification of an agreed paediatric investigation plan for rivogenlecleucel (EMEA-001869-PIP01-15-M02) 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[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/0138/2017 issued on 7 June 2017 and the decision P/0194/2018 issued on 17 July 2018,

Having regard to the application submitted by Bellicum Pharma Ltd on 12 July 2019 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 18 October 2019, 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.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

[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 rivogenlecleucel, dispersion for infusion, intravenous use, 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 Bellicum Pharma Ltd, The Henley Building, Fairview Estate, Newtown Road, RG9 1HG - Henley-on-Thames, United Kingdom.

EMA/PDCO/412734/2019
Amsterdam, 18 October 2019

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

EMEA-001869-PIP01-15-M02

Scope of the application

Active substance(s):

Rivogenlecleucel

Condition(s):

Adjunctive treatment in haematopoietic stem cell transplantation

Pharmaceutical form(s):

Dispersion for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Bellicum Pharma Ltd

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Bellicum Pharma Ltd submitted to the European Medicines Agency on 12 July 2019 an application for modification of the agreed paediatric investigation plan as set out in the European Medicines Agency's decision P/0138/2017 issued on 7 June 2017 and the decision P/0194/2018 issued on 17 July 2018.

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

The procedure started on 20 August 2019.

Scope of the modification

Some measures 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 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 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

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Adjunctive treatment in haematopoietic stem cell transplantation

2.1.1. Indication(s) targeted by the PIP

Treatment of immunodeficiency after mismatched, related, allogeneic transplantation in paediatric patients with malignant and non-malignant disorders amenable to haematopoietic stem cell transplantation

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)

Dispersion for infusion

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	0	Not applicable
Clinical studies	3	**Study 1 (BP-004):** Open-label, non-randomised, externally-controlled, single-arm trial with 2 phases to determine the highest tolerated dose (Phase 1) and to evaluate safety and activity (Phase 2) of rivogenlecleucel) and of rimiducid in children from birth to less than 18 years of age whose disease is deemed curable by haematopoietic stem cell transplantation (HSCT) but who do not have a matched donor (related or unrelated). (Same study as Study 1 in the PIP for rimiducid EMEA-001870-PIP01-15). **Study 2 (C-004):** Observational study in children from birth to less than 18 years of age whose disease is deemed curable by HSCT but who do not have a matched related donor but who have an alternative eligible matched unrelated donor (MUD), to collect safety and efficacy data of MUD transplant in these patients with haematopoietic disorders, both malignant and non-malignant. (Same study as Study 3 in EMEA-001870-PIP01-15).

		Study 3 (BP-U-004) *(added in procedure EMEA-001869-PIP01-15-M02):* Open-label, non-randomised, uncontrolled, single-arm trial with 2 phases to determine the maximum tolerated dose (Phase 1) and to evaluate safety and activity (Phase 2) of rivogenlecleucel and of rimiducid in children from 1 month to less than 18 years of age (and adults) whose disease is deemed curable by haematopoietic stem cell transplantation (HSCT) but who do not have a matched donor (related or unrelated).
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 March 2019
Deferral for one or more measures contained in the paediatric investigation plan:	No