EMA/293457/2017

European Medicines Agency decision

P/0137/2017

of 7 June 2017

on the agreement of a paediatric investigation plan and on the refusal of a deferral for rimiducid (EMEA-001870-PIP01-15) 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.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Contact EMA www.ema.europa.eu/contact

An agency of the European Union

European Medicines Agency decision

P/0137/2017

of 7 June 2017

on the agreement of a paediatric investigation plan and on the refusal of a deferral for rimiducid (EMEA-001870-PIP01-15) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 application submitted by Bellicum Pharma Ltd. on 20 November 2015 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 21 April 2017, in accordance with Article 18 of Regulation (EC) No 1901/2006 and Article 21 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 refusal of a deferral.

(2) It is therefore appropriate to adopt a decision agreeing a paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision refusing a deferral.

[1] OJ L 378, 27.12.2006, p.1.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for rimiducid, solution 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 rimiducid, solution 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 refused.

Article 3

This decision is addressed to Bellicum Pharma Ltd., Cooley (UK) LLP, Dashwood, Old Broad St, EC2M 1QS – London, United Kingdom.

EMA/PDCO/115488/2017
London, 21 April 2017

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

EMEA-001870-PIP01-15

Scope of the application

Active substance(s):

Rimiducid

Condition(s):

Treatment of graft versus host disease

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Bellicum Pharma Ltd.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Bellicum Pharma Ltd. submitted for agreement to the European Medicines Agency on 20 November 2015 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 2 February 2016.

Supplementary information was provided by the applicant on 30 January 2017. The applicant proposed modifications to the paediatric investigation plan.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5510
Send a question via our website www.ema.europa.eu/contact

An agency of the European Un on

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 18 of said Regulation;
   - to refuse a deferral in accordance with Article 21 of said Regulation.

   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 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:

Treatment of graft versus host disease

2.1.1. Indication(s) targeted by the PIP

Treatment of graft versus host disease (GvHD) in paediatric patients who have received a mismatched, related, allogeneic haematopoietic stem cell transplantation together with expanded donor-derived allogenic T cells transduced with the retroviral vector expressing the transgenes for inducible caspase9 and the truncated CD19 selectable marker (BPX-501)

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)

Solution 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 expanded donor-derived allogenic T cells transduced with the retroviral vector expressing the transgenes for inducible caspase9 and the truncated CD19 selectable marker (BPX-501) 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 EMEA-001869-PIP01-15) Study 2 (BP-I-008) Open-label, non-controlled trial to evaluate

		pharmacokinetics and safety of rimiducid in children from 2 months to less than 18 years of age with recurrent or minimal residual disease (MRD) hematologic malignancies after prior allogeneic transplantation Study 3 (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 2 in EMEA-001869-PIP01-15)
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:	No
Date of completion of the paediatric investigation plan:	By April 2018
Deferral for one or more measures contained in the paediatric investigation plan:	No