



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

European Medicines Agency decision

P/0106/2020

of 20 March 2020

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for autologous CD34+ haematopoietic stem cells transduced ex vivo with a lentiviral vector encoding for the human Beta 2 Integrin/CD18 gene (EMEA-002562-PIP01-19) 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 726/2004¹,

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 Agency²,

Having regard to the application submitted by Rocket Pharmaceuticals, Inc. on 19 February 2019 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 31 January 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.

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 autologous CD34+ haematopoietic stem cells transduced ex vivo with a lentiviral vector encoding for the human Beta 2 Integrin/CD18 gene, 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 CD34+ haematopoietic stem cells transduced ex vivo with a lentiviral vector encoding for the human Beta 2 Integrin/CD18 gene, 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 CD34+ haematopoietic stem cells transduced ex vivo with a lentiviral vector encoding for the human Beta 2 Integrin/CD18 gene, 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 Rocket Pharmaceuticals, Inc., 350 Fifth Ave, Suit 7530, 10118 - New York, United States.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/600355/2019
Amsterdam, 31 January 2020

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

EMA-002562-PIP01-19

Scope of the application

Active substance(s):

Autologous CD34+ haematopoietic stem cells transduced *ex vivo* with a lentiviral vector encoding for the human Beta 2 Integrin/CD18 gene

Condition(s):

Treatment of leukocyte adhesion deficiency type I

Pharmaceutical form(s):

Dispersion for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Rocket Pharmaceuticals, Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Rocket Pharmaceuticals, Inc. submitted for agreement to the European Medicines Agency on 19 February 2019 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 1 April 2019.

Supplementary information was provided by the applicant on 28 October 2019. The applicant proposed modifications to the paediatric investigation plan and requested a deferral.



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 leukocyte adhesion deficiency type I

The waiver applies to:

- the paediatric population from birth to less than 3 months of age;
- 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 leukocyte adhesion deficiency type I

2.1.1. Indication(s) targeted by the PIP

Treatment of severe leukocyte adhesion deficiency type I

Treatment of moderate leukocyte adhesion deficiency type I

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

From 3 months 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: Open-label uncontrolled trial to evaluate safety and efficacy of autologous CD34+ haematopoietic stem cells transduced <i>ex vivo</i> with a lentiviral vector encoding for the human Beta 2 Integrin/CD18 gene (hereafter referred to as RP-L201) in children from 3 months of age with severe leukocyte adhesion deficiency type I (LAD-I).

		Study 2: Open-label uncontrolled trial to evaluate safety and efficacy of RP-L201 in children from 3 months of age with severe LAD-I. Study 3: Open-label uncontrolled trial to evaluate safety and efficacy of RP-L201 in children and adolescents from 2 years of age with moderate LAD-I.
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 2026
Deferral for one or more measures contained in the paediatric investigation plan:	Yes