



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/318167/2021

European Medicines Agency decision P/0247/2021

of 9 July 2021

on the agreement of a paediatric investigation plan for allogeneic skin-derived ATP-binding cassette, sub-family B, member 5 (ABCB5)-positive mesenchymal stem cells (allo-APZ2-EB) (EMEA-002875-PIP01-20) 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.



European Medicines Agency decision

P/0247/2021

of 9 July 2021

on the agreement of a paediatric investigation plan for allogeneic skin-derived ATP-binding cassette, sub-family B, member 5 (ABCB5)-positive mesenchymal stem cells (allo-APZ2-EB) (EMEA-002875-PIP01-20) 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 726/2004¹,

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 RHEACELL GmbH & Co. KG on 10 August 2020 under Article 16(1) of Regulation (EC) No 1901/2006, also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 21 May 2021, in accordance with Article 17 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 agreement of a paediatric investigation plan.
- (2) It is therefore appropriate to adopt a decision agreeing a 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

A paediatric investigation plan for allogeneic skin-derived ATP-binding cassette, sub-family B, member 5 (ABCB5)-positive mesenchymal stem cells (allo-APZ2-EB), suspension 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

This decision is addressed to RHEACELL GmbH & Co. KG, Im Neuenheimer Feld 517, 69120 – Heidelberg, Germany.



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/132722/2021
Amsterdam, 21 May 2021

Opinion of the Paediatric Committee on the agreement of a Paediatric Investigation Plan

EMA-002875-PIP01-20

Scope of the application

Active substance(s):

Allogeneic skin-derived ATP-binding cassette, sub-family B, member 5 (ABCB5)-positive mesenchymal stem cells (allo-APZ2-EB)

Condition(s):

Treatment of epidermolysis bullosa

Pharmaceutical form(s):

Suspension for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

RHEACELL GmbH & Co. KG

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, RHEACELL GmbH & Co. KG submitted for agreement to the European Medicines Agency on 10 August 2020 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 15 September 2020.

Supplementary information was provided by the applicant on 15 February 2021. The applicant proposed modifications to the paediatric investigation plan and withdrew its request for a waiver.



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.

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 epidermolysis bullosa

2.1.1. Indication(s) targeted by the PIP

Treatment of recessive dystrophic epidermolysis bullosa and junctional epidermolysis bullosa

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)

Suspension 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	1	Study 1 Double-blind, randomized, placebo-controlled clinical trial with a efficacy phase and a single-arm open-label follow-up phase to investigate the efficacy of the investigational medicinal product allo-APZ2-EB in patients from birth to less than 18 years of age (and adults) with recessive dystrophic epidermolysis bullosa compared to placebo, including a second open-label study arm to investigate the efficacy of allo-APZ2-EB in patients with junctional epidermolysis bullosa (allo-APZ2-EB-III)
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 November 2024
Deferral for one or more measures contained in the paediatric investigation plan:	No