EMA/25307/2025

European Medicines Agency decision

P/0004/2025

of 27 January 2025

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for adeno-associated virus serotype 9 vector containing the human *LAMP2* isoform B transgene (RP-A501) (EMEA-003639-PIP01-24), 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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Rocket Pharmaceuticals, B.V. on 29 April 2024 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 13 December 2024, 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for adeno-associated virus serotype 9 vector containing the human *LAMP2* isoform B transgene (RP-A501), 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 adeno-associated virus serotype 9 vector containing the human *LAMP2* isoform B transgene (RP-A501), 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 granted.

Article 3

A waiver for adeno-associated virus serotype 9 vector containing the human *LAMP2* isoform B transgene (RP-A501), 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 granted.

Article 4

This decision is addressed to Rocket Pharmaceuticals, B.V., Kingsfordweg 151, 1043 GR – Amsterdam, The Netherlands.

EMA/PDCO/450286/2024
Amsterdam, 13 December 2024

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

EMEA-003639-PIP01-24

Scope of the application

Active substance(s):

Adeno-associated virus serotype 9 vector containing the human *LAMP2* isoform B transgene (RP-A501)

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of Danon disease

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Rocket Pharmaceuticals, B.V.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Rocket Pharmaceuticals, B.V. submitted for agreement to the European Medicines Agency on 29 April 2024 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 27 May 2024.

Supplementary information was provided by the applicant on 5 September 2024. The applicant proposed modifications to the paediatric investigation plan.

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 Paediatric Committee members of Liechtenstein and Norway agree 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 annexes 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 Danon Disease

The waiver applies to:

- the paediatric population from birth to less than 3 years;
- solution for infusion, intravenous use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of Danon Disease

2.1.1. Indication(s) targeted by the PIP

Treatment of Danon Disease

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

From 3 years to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Solution for infusion

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**Study 1** Open-label, non-randomised, single dose trial to evaluate safety and efficacy of RP-A501 in male patients from 8 years to less than 18 years of age (and adults) with Danon disease with any confirmed lysosome-associated membrane protein 2 (*LAMP2*) mutation(s) and cardiac involvement. **Study 2** Open-label, single-arm, single dose trial to evaluate safety and efficacy of RP-A501 in male patients from 8 years to less than 18 years of age (and adults) with Danon disease

	with a pathogenic or likely pathogenic *LAMP2* mutation(s) and evidence of left ventricular hypertrophy with preserved systolic function. **Study 3** Open-label, single-arm, single dose trial to evaluate safety and efficacy of RP-A501 in male patients from 3 to less than 8 years of age with Danon disease with a pathogenic or likely pathogenic *LAMP2* mutation(s) and evidence of left ventricular hypertrophy with preserved systolic function. **Study 4** Open-label, single-arm, single dose trial to evaluate safety and efficacy of RP-A501 in female patients with Danon disease with a pathogenic or likely pathogenic *LAMP2* mutation(s) and evidence of left ventricular hypertrophy with preserved systolic function.
Modelling and simulation analyses	Not applicable
Other studies	**Study 5** Observational study with both retrospective and prospective data collection designed to describe the natural history of Danon disease including the signs and symptoms, key clinical events, and impact of the disease on quality of life.
Extrapolation plan	Studies 1, 2 and 5 are part of an extrapolation plan covering the paediatric population from 3 years to less than 8 years of age, as agreed by the PDCO.

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 2034
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.