EMA/151831/2021

European Medicines Agency decision

P/0128/2021

of 14 April 2021

on the agreement of a paediatric investigation plan and on the granting of a deferral for adeno-associated virus, serotype 9 (AAV9)-based non-replicating, self-complementary recombinant vector containing an expression cassette for the human ASPA transgene (scAAV9-CB6-hASPAopt) (EMEA-002779-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.

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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 application submitted by Aspa Therapeutics, Inc. on 24 March 2020 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 26 February 2021, in accordance with Article 17 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 granting 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 granting 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 adeno-associated virus, serotype 9 (AAV9)-based non-replicating, self-complementary recombinant vector containing an expression cassette for the human ASPA transgene (scAAV9-CB6-hASPAopt), solution for injection, 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 (AAV9)-based non-replicating, self-complementary recombinant vector containing an expression cassette for the human ASPA transgene (scAAV9-CB6-hASPAopt), solution for injection, 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

This decision is addressed to Aspa Therapeutics, Inc., 421 Kipling Street, CA 94301 - Palo Alto, United States.

EMA/PDCO/654361/2020
Amsterdam, 26 February 2021

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

EMEA-002779-PIP01-20

Scope of the application

Active substance(s):

Adeno-associated virus, serotype 9 (AAV9)-based non-replicating, self-complementary recombinant vector containing an expression cassette for the human ASPA transgene (scAAV9-CB6-hASPAopt)

Condition(s):

Treatment of Canavan disease

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Aspa Therapeutics, Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Aspa Therapeutics, Inc. submitted for agreement to the European Medicines Agency on 24 March 2020 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 30 April 2020.

Supplementary information was provided by the applicant on 27 November 2020. 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.

   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 Canavan disease

2.1.1. Indication(s) targeted by the PIP

Treatment of Canavan disease

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 injection

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	1	**Study 1** Safety and biodistribution study of scAAV9-CB6-hASPAopt in C57BL/6 Mice
Clinical studies	3	**Study 2** Retrospective medical history and prospective observational study of patients with Canavan disease for assessment of natural history of Canavan disease. (CVN-101) **Study 3** Open-label, externally controlled study to evaluate safety and activity at 2 planned dose levels of scAAV9-CB6-hASPAopt in patients 30 months of age or younger with Canavan disease. (CVN-102) **Study 4** Open-label, externally controlled study to evaluate safety and activity of scAAV9-CB6-hASPAopt in patients older than 30 months of age with Canavan disease.

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 2031
Deferral for one or more measures contained in the paediatric investigation plan:	Yes