EMADOC-1700519818-2039246

# European Medicines Agency decision

EMA/PE/0000232894

of 14 April 2025

on the agreement of a paediatric investigation plan and on the granting of a deferral for adeno-associated viral vector serotype 9 containing the human MECP2 gene 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 Taysha Gene Therapies Inc. on 26 April 2024 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 28 February 2025, 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, 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 viral vector serotype 9 containing the human MECP2 gene, 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 viral vector serotype 9 containing the human MECP2 gene, 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 Taysha Gene Therapies Inc., 3000 Pegasus Park Drive Suite 1430, Dallas, TX 75247-6204, United States.

EMADOC-1700519818-1804090 Corr[1]
Amsterdam, 28 February 2025

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

EMA/PE/0000232894

## Scope of the application

**Active substance(s):**

Adeno-associated viral vector serotype 9 containing the human MECP2 gene (TSHA-102)

**Invented name and authorisation status:**

See Annex II

**Condition(s):**

Treatment of Rett syndrome

**Pharmaceutical form(s):**

Solution for infusion

**Route(s) of administration:**

Intrathecal use

**Name/corporate name of the PIP applicant:**

Taysha Gene Therapies Inc.

## Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Taysha Gene Therapies Inc. submitted for agreement to the European Medicines Agency on 26 April 2024 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 27 May 2024.

Supplementary information was provided by the applicant on 21 November 2024. The applicant proposed modifications to the paediatric investigation plan.

[1] 18 March 2025.

# 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 Paediatric Committee member of Norway 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 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

Not applicable

# 2. Paediatric investigation plan

## *2.1. Condition:*

Treatment of Rett syndrome

### 2.1.1. Indication(s) targeted by the PIP

Treatment of Rett syndrome

### 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 | Description |
|---|---|
| Quality-related studies | Not applicable |
| Non-clinical studies | Not applicable |
| Clinical studies | **Study 1 (TSHA-102-CL-101)**<br><br>Open-label, randomized, 2-part study including a dose-escalation (Part A) and dose expansion (Part B) to evaluate the safety, efficacy, and durability of a single intrathecal dose of TSHA-102 in female adolescents from 12 to less than 18 years of age (and adults) (Part A), and female participants from 9 years to less than 18 years of age (and adults) (Part B) with Rett syndrome with a documented MECP2 loss-of-function gene mutation.<br><br>**Study 2 (TSHA-102-CL-102)**<br><br>Open-label, randomized, 2-part study including a dose-escalation (Part A) and dose expansion (Part B) to evaluate the safety, efficacy, and durability of a single intrathecal dose of TSHA-102 in female children from 2 to less than 9 years of age with Rett syndrome with a documented MECP2 loss-of-function gene mutation.<br><br>**Study 3**<br><br>Study to assess the safety, efficacy and durability of a single intrathecal dose of TSHA-102 in female children from birth to less than |

| | |
|---|---|
| | 2 years of age with Rett syndrome with a documented MECP2 loss-of-function gene mutation.<br>Further details of key elements need to be agreed prior to initiation of the study. |
| Modelling and simulation analyses | Not applicable |
| Other studies | Not applicable |
| Extrapolation plan | 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 December 2033 |
| 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.**