EMA/105519/2024

European Medicines Agency decision

P/0179/2024

of 6 May 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for sevasemten (EMEA-003394-PIP01-23), 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 FGK Representative Service GmbH on 20 January 2023 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 23 February 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, following a re-examination procedure of the Paediatric Committee's opinion according to Article 25(3) of Regulation (EC) No 1901/2006, 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 sevasemten, tablet, liquid age-appropriate formulation, oral 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 sevasemten, tablet, liquid age-appropriate formulation, oral 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 sevasemten, tablet, liquid age-appropriate formulation, oral 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 FGK Representative Service GmbH Germany, Heimeranstr. 35, 80339 – Munich, Germany.

EMA/PDCO/144866/2024
Amsterdam, 26 April 2024

Final opinion of the Paediatric Committee on the agreement of a Paediatric Investigation plan and a deferral and a waiver

EMEA-003394-PIP01-23

Scope of the application

Active substance(s):

Sevasemten

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of dystrophinopathies

Pharmaceutical form(s):

Tablet

Liquid age-appropriate formulation

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

FGK Representative Service GmbH

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, FGK Representative Service GmbH submitted for agreement to the European Medicines Agency on 20 January 2023 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.

An Opinion was adopted by the Paediatric Committee on 23 February 2024 for the above mentioned product. FGK Representative Service GmbH received the Paediatric Committee Opinion on 4 March 2024.

On 3 April 2024 FGK Representative Service GmbH submitted to the European Medicines Agency a written request, including detailed grounds for re-examination of the Opinion.

The re-examination procedure started on 4 April 2024.

Final Opinion

1. The Paediatric Committee, having assessed the detailed grounds for re-examination, in accordance with Article 25(3) of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report :

   1.1. to revise its opinion and

   - 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 Regulation (EC) No 1901/2006 as amended, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

   1.2. following re-examination, to amend the measures.

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 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(s):

Treatment of dystrophinopathies.

The waiver applies to:

- the paediatric population from birth to less than 6 months of age;
- tablet, oral 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 dystrophinopathies.

2.1.1. Indication(s) targeted by the PIP

Treatment of Duchenne muscular dystrophy

Treatment of Becker muscular dystrophy

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

From 6 months to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Tablet.

Liquid age-appropriate formulation.

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Development of an age appropriate liquid formulation obtained from reconstitution of the existing tablet formulation in water in an oral syringe (EDG-5506 – CMC 1).
Non-clinical studies	Not applicable.
Clinical studies	**Study 2** Randomized, double-blind, parallel-group, placebo-controlled, dose ranging study in ambulant (adults and) adolescent patients with Becker

	muscular dystrophy (BMD) to evaluate the effect of sevasemten on safety, biomarkers, PK, and activity (EDG-5506-201). **Study 3** Open-label extension study in ambulant (adults and) and adolescent patients with Becker muscular dystrophy (BMD) who completed BMD interventional PIP Study 2 EDG-5506-201 (EDG-5506-203) for safety and pharmacodynamic assessments. **Study 4** Randomized, double-blind, placebo-controlled study to evaluate PK, biomarker response, safety, and efficacy of sevasemten in ambulant paediatric patients aged 4 to less than 10 years with Duchenne muscular dystrophy (DMD) and to support dose selection for Study 6 EDG-5506-310 (EDG-5506-210). **Study 5** 12-month, double-blind, randomized, placebo-controlled, parallel-group pivotal study of efficacy and safety in ambulant paediatric patients aged 4 years to less than 10 years of age with confirmed mutation in the DMD gene and clinical phenotype of DMD (EDG-5506-310). **Study 6** Randomized, double-blind, placebo-controlled study to evaluate the effect of sevasemten on efficacy, safety and biomarker response and PK/PD in non-ambulant paediatric patients from 10 years to less than 18 years of age with DMD (EDG-5506-213). **Study 7** Randomized, double-blind, placebo-controlled part (Part A) and with an open-label part (Part B) to evaluate the effect of sevasemten on safety, PK and biomarkers and efficacy in DMD patients from 2 to less than 4 years of age (EDG-5506-212). **Study 8** Randomized, double-blind, placebo-controlled study (Part A) with an open-label part (Part B) to evaluate the effect of sevasemten on biomarkers, safety, PK and efficacy in patients from 6 months to less than 2 years of age with DMD (EDG-5506-214). **Study 9** Open-label extension study for patients aged 4 years to less than 18 years with DMD completing the PIP Studies 4 (EDG-5506-210) and 5 (EDG-5506-310) for safety and pharmacodynamic assessments.
Modelling and simulation analyses	**Study 10** Population pharmacokinetic model and exposure- response model to guide dose selection in different age groups and analyse PK/PD relationship in

	the various age groups to support extrapolation of efficacy in DMD and BMD.
Other studies	Not applicable.
Extrapolation plan	Studies 2, 6, 7, 8, 10 are part of an extrapolation plan covering the paediatric population from 6 months to less than 4 years of age (DMD), and from 12 to less than 18 years of age (BMD) 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:	No
Date of completion of the paediatric investigation plan:	By March 2031
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.