

EMA/364883/2024

European Medicines Agency decision

P/0302/2024

of 16 August 2024

on the acceptance of a modification of an agreed paediatric investigation plan for osivelotor (EMA-003241-PIP01-22-M01), 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 726/2004¹,

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 Agency²,

Having regard to the European Medicines Agency's decision P/0074/2023 issued on 10 March 2023,

Having regard to the application submitted by Pfizer Europe MA EEIG on 22 March 2024 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 28 June 2024, in accordance with Article 22 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 acceptance of changes to the agreed paediatric investigation plan.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

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

Changes to the agreed paediatric investigation plan for osivelotor, tablet, age-appropriate oral solid dosage form, oral use, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision is addressed to Pfizer Europe MA EEIG, Boulevard de la Plaine 17, 1050 – Brussels, Belgium.

EMA/PDCO/145864/2024
Amsterdam, 28 June 2024

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-003241-PIP01-22-M01

Scope of the application

Active substance(s):

Osivelotor

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of sickle cell disease

Pharmaceutical form(s):

Tablet

Age-appropriate oral solid dosage form

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Pfizer Europe MA EEIG

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Pfizer Europe MA EEIG submitted to the European Medicines Agency on 22 March 2024 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0074/2023 issued on 10 March 2023.

The application for modification proposed changes to the agreed paediatric investigation plan.

The procedure started on 29 April 2024.

Scope of the modification

Some measures of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:
 - to agree to changes to the paediatric investigation plan in the scope set out in the Annex I of this opinion.

The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the 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 sickle cell disease

The waiver applies to:

- the paediatric population from birth to less than 6 months of age;
- tablet, age-appropriate oral solid dosage form, 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 sickle cell disease

2.1.1. Indication(s) targeted by the PIP

Treatment of sickle cell disease

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

Age-appropriate oral solid dosage form

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Development of age-appropriate oral solid dosage form
Non-clinical studies	Not applicable
Clinical studies	Study 2 C5351004 (GBT021601-021) Part B Double-blind, randomised, placebo-controlled study to evaluate safety and efficacy of osivelotor in adolescents from 12 years to less than 18 years of age (and adults) with sickle cell disease.

	Study 3 C5351004 (GBT021601-021) Part C Open-label, uncontrolled study to evaluate pharmacokinetics and safety of osivelotor in children and adolescents from 6 months to less than 18 years of age with sickle cell disease.
Modelling and simulation studies	Study 4 Modelling and simulation study to support dose finding in children from 12 years to less than 18 years of age with sickle cell disease. Study 5 Modelling and simulation study to support dose finding in children from 6 months to less than 12 years of age with sickle cell disease.
Other studies	Not applicable
Extrapolation plan	Studies 2, 3, 4 and 5 are part of the extrapolation plan of efficacy data from adult and adolescents to the paediatric population from 6 months to less than 12 years of age with sickle cell disease.

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 June 2028
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.