EMA/173847/2023

European Medicines Agency decision

P/0145/2023

of 21 April 2023

on the acceptance of a modification of an agreed paediatric investigation plan for sparsentan, (EMEA-001984-PIP02-20-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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on the acceptance of a modification of an agreed paediatric investigation plan for sparsentan, (EMEA-001984-PIP02-20-M01) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 European Medicines Agency's decision P/0021/2021 issued on 27 January 2021,

Having regard to the application submitted by Vifor (international) AG on 14 December 2022 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 31 March 2023, 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 and to the deferral.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan, including changes to the 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

Changes to the agreed paediatric investigation plan for sparsentan, tablet, age-appropriate oral liquid dosage form, oral use, including changes to the deferral, 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 Vifor (International) AG, 37 Rechenstrasse, 9014 - St gallen, Switzerland.

EMA/PDCO/3574/2023
Amsterdam, 31 March 2023

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMEA-001984-PIP02-20-M01

Scope of the application

Active substance(s):

Sparsentan

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of focal segmental glomerulosclerosis

Pharmaceutical form(s):

Tablet

Age-appropriate oral liquid dosage form

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Vifor (international) AG

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Vifor (international) AG submitted to the European Medicines Agency on 14 December 2022 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0021/2021 issued on 27 January 2021.

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

The procedure started on 30 January 2023.

Scope of the modification

Some measures and timelines 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 and to the deferral 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 focal segmental glomerulosclerosis

The waiver applies to:

- the paediatric population from birth to less than 1 year of age;
- tablet and age-appropriate oral liquid dosage form, oral use;
- on the grounds that the specific medicinal product is likely to be unsafe.

2. Paediatric investigation plan

2.1. Condition:

Treatment of focal segmental glomerulosclerosis

2.1.1. Indication(s) targeted by the PIP

Treatment of focal segmental glomerulosclerosis

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

From 1 year to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Tablet

Age-appropriate oral liquid dosage form

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1 (FORM-DEV1)** Age-appropriate oral liquid dosage form and suitable medical administration device for dosing children 1 year of age and older
Non-clinical studies	Not applicable
Clinical studies	**Study 2 (RET-D-001/DUET Extension)** Open-label treatment extension phase of study RET-D-001 (DUET), following its 8-week double-blind active-controlled (irbesartan) treatment period, to assess the long-term safety and sustainability of effect of sparsentan for up to 400 weeks, in patients from 8 to less than 18 years of age (and adults) with biopsy-proven focal segmental glomerulosclerosis (FSGS) or documentation of a genetic mutation in a podocyte protein associated with FSGS

	Study 3 (021FSGS16010/DUPLEX) Randomised, double-blind, active-control, parallel group study, to assess the long-term efficacy and safety of sparsentan compared to irbesartan in patients from 8 to less than 18 years of age (and adults) with biopsy-proven primary focal segmental glomerulosclerosis (FSGS) or documentation of a genetic mutation in a podocyte protein associated with FSGS **Study 4 (021-PED1)** Open-label, uncontrolled, 2-part study to evaluate the pharmacokinetics and pharmacodynamics (part 1: 12-weeks), safety, and efficacy (part 2: 96-weeks) of once daily oral sparsentan (oral liquid suspension formulation) in children from 1 to less than 18 years of age with focal segmental glomerulosclerosis (FSGS) or minimal change disease (MCD)
Extrapolation, modelling and simulation studies	**Study 5** Physiologically based PK (PBPK) model to assess the impact of the physiochemical properties of a new paediatric oral suspension formulation under conditions of use, to support dose selection for paediatric patients from 1 year of age. **Study 6** Population pharmacokinetic (PopPK) modelling and simulation study to evaluate the dose-exposure relationship in adults and in each paediatric subpopulation and disease population from 1 to less than 18 years of age
Other studies	**Study 7 (021FSGS16010/DUPLEX [interim analysis])** Interim analysis of study 021FSGS16010 (DUPLEX) to assess the long-term efficacy and safety of sparsentan compared to irbesartan in patients from 8 to less than 18 years of age (and adults) with focal segmental glomerulosclerosis (FSGS)
Other measures	Not applicable

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 June 2026
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.