

EMA/33163/2021

European Medicines Agency decision P/0021/2021

of 27 January 2021

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for sparsentan (EMA-001984-PIP02-20) 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 726/2004¹,

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 Agency²,

Having regard to the application submitted by Retrophin Europe Limited on 25 March 2020 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 11 December 2020, 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 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.

² OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for sparsentan, tablet, age-appropriate oral liquid dosage form, 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 sparsentan, tablet, age-appropriate oral liquid dosage form, 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 sparsentan, tablet, age-appropriate oral liquid dosage form, 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 Travers Therapeutics Ireland Ltd., 2nd Floor, Palmerston House, Fenian Street, D02 WD37 – Dublin, Ireland.

EMA/PDCO/527034/2020
Amsterdam, 11 December 2020

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

EMA-001984-PIP02-20

Scope of the application

Active substance(s):

Sparsentan

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:

Traverse Therapeutics Ireland Ltd.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Retrophin Europe Limited submitted for agreement to the European Medicines Agency on 25 March 2020 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.

The procedure started on 30 April 2020.

Supplementary information was provided by the applicant on 11 September 2020. The applicant proposed modifications to the paediatric investigation plan.

On 24 November 2020 Retrophin Europe Limited requested to transfer the paediatric investigation plan to Traverse Therapeutics Ireland Ltd.

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;
 - 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)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population.

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 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 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

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 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	Number of measures	Description
Quality-related studies	1	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	0	Not applicable
Clinical studies	3	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	2	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	1	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	0	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 November 2025
Deferral for one or more measures contained in the paediatric investigation plan:	Yes