EMA/33164/2021

European Medicines Agency decision

P/0024/2021

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 (EMEA-001984-PIP03-20) 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 726/2004[1],

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 Agency[2],

Having regard to the application submitted by Retrophin Europe Limited on 20 April 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 Travere Therapeutics Ireland Ltd., 2nd Floor, Palmerston House, Fenian Street, D02 WD37 – Dublin, Ireland.

EMA/PDCO/526097/2020
Amsterdam, 11 December 2020

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

EMEA-001984-PIP03-20

Scope of the application

Active substance(s):

Sparsentan

Condition(s):

Treatment of immunoglobulin A nephropathy

Pharmaceutical form(s):

Tablet

Age-appropriate oral liquid dosage form

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Travere 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 20 April 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 26 May 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 Travere 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)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

   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 immunoglobulin A nephropathy

The waiver applies to:

- the paediatric population from birth to less than 2 year;
- tablet, age-appropriate oral liquid dosage form, oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric investigation plan

2.1. Condition:

Treatment of immunoglobulin A nephropathy

2.1.1. Indication(s) targeted by the PIP

Treatment of immunoglobulin A nephropathy

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

From 2 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 2 years of age and older
Non-clinical studies	0	Not applicable

Clinical studies	1	**Study 2 (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 2 to less than 18 years of age with immunoglobulin A nephropathy (IgAN), Henoch-Schonlein purpura nephritis (HSPN), or Alport Syndrome
Extrapolation, modelling and simulation studies	2	**Study 3** 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 2 years of age. **Study 4** 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	0	Not applicable
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