EMA/635386/2020

European Medicines Agency decision

P/0489/2020

of 21 December 2020

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for voxelotor (EMEA-002356-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 726/2004[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 Synteract GmbH on 24 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 13 November 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 refusing 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 voxelotor, tablet, powder for oral suspension, 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 voxelotor, tablet, powder for oral suspension, 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 voxelotor, tablet, powder for oral suspension, 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 Synteract GmbH, Albrechtstrasse 14, 80636 - Munich, Germany.

EMA/PDCO/444985/2020
Amsterdam, 13 November 2020

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

EMEA-002356-PIP02-20

Scope of the application

Active substance(s):

Voxelotor

Condition(s):

Treatment of sickle cell disease

Pharmaceutical form(s):

Tablet

Powder for oral suspension

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Synteract GmbH

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Synteract GmbH submitted for agreement to the European Medicines Agency on 24 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 10 August 2020. The applicant proposed modifications to the paediatric investigation plan and waiver.

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 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 sickle cell disease

The waiver applies to:

- the paediatric population from birth to less than 6 months of age;
- tablet, powder for oral suspension, oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

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 (SCD)

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

Powder for oral suspension

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1** Development of a powder for oral suspension (PfOS) for the paediatric population from 6 months to less than 12 years of age.
Non-clinical studies	0	Not applicable.
Clinical studies	6	**Study 2** Randomised, double-blind, placebo-controlled efficacy and safety study of voxelotor in paediatric patients from 12 to less than 18 years of age (and adults) with sickle cell disease (SCD). (GBT440-031).

		Study 3 Single arm, single and multiple dose study, evaluating the pharmacokinetics (PK), safety, tolerability, and treatment effect of voxelotor in paediatric patients from 4 years to less than 18 years of age with sickle cell disease (SCD). (GBT 440-007 Part C). **Study 4** Single arm, single and multiple dose study, evaluating the pharmacokinetics (PK), safety, tolerability, and treatment effect of voxelotor in paediatric patients from 6 months to less than 4 years of age with sickle cell disease (SCD). (GBT 440-007 Part D). **Study 5** Randomised, double-blind, placebo-controlled study to evaluate the effect of voxelotor on the arterial cerebral blood flow [evaluated with trans- cranial doppler (TCD) time averaged mean of the maximum velocity (TAMMV) at 24 weeks] in paediatric patients from 2 years to less than 15 years of age with sickle cell disease (SCD). (GBT440-032). **Study 6** Open-label extension (OLE) study to assess the safety of long-term treatment and SCD-related complications of voxelotor in paediatric patients from 12 years to less than 18 years of age who have completed Study 2 GBT440-031. (GBT440-034). **Study 7** Open-label extension (OLE) study to assess the safety and SCD-related complications with long-term treatment with voxelotor in paediatric patients from 4 years to less than 18 years of age who have completed treatment in voxelotor paediatric clinical studies. (GBT440-038).
Extrapolation, modelling and simulation studies	0	Not applicable.
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:	No
Date of completion of the paediatric investigation plan:	By June 2027
Deferral for one or more measures contained in the paediatric investigation plan:	Yes