EMA/853094/2022

European Medicines Agency decision

P/0491/2022

of 2 December 2022

on the acceptance of a modification of an agreed paediatric investigation plan for voxelotor (EMEA-002356-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 voxelotor (EMEA-002356-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/0489/2020 issued on 21 December 2020,

Having regard to the application submitted by Global Blood Therapeutics Netherlands B. V. on 30 June 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 14 October 2022, 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 voxelotor, tablet, dispersible tablet, 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 Global Blood Therapeutics Netherlands B. V., 3051 Strawinskylaan, 1077ZX – Amsterdam, The Netherlands.

EMA/PDCO/638755/2022
Amsterdam, 14 October 2022

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

EMEA-002356-PIP02-20-M01

Scope of the application

Active substance(s):

Voxelotor

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of sickle cell disease

Pharmaceutical form(s):

Tablet

Dispersible tablet

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Global Blood Therapeutics Netherlands B. V.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Global Blood Therapeutics Netherlands B. V. submitted to the European Medicines Agency on 30 June 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/0489/2020 issued on 21 December 2020.

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

The procedure started on 16 August 2022.

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified.

Pharmaceutical form was amended.

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.
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, 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

Dispersible tablet

2.1.4. Measures

Area	**Description**
Quality-related studies	**Study 1** Development of a dispersible tablet for the paediatric population from 6 months to less than 12 years of age.
Non-clinical studies	Not applicable
Clinical studies	**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 6 months to less than 18 years of age who have completed treatment in voxelotor paediatric clinical studies. (GBT440-038).
Extrapolation, modelling and simulation studies	Not applicable
Other studies	Not applicable
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:	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

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

The product is not authorised anywhere in the European Community.