EMA/471086/2021

European Medicines Agency decision

P/0387/2021

of 8 September 2021

on the agreement of a paediatric investigation plan and on the granting of a deferral for ribitol (EMEA-002887-PIP01-20) 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 Premier Research Group S.L. on 11 September 2020 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 23 July 2021, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 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.

(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.

[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 ribitol, powder for oral use, age-appropriate oral 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 ribitol, powder for oral use, age-appropriate oral 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

This decision is addressed to Premier Research Group S.L., Camino de la Zarzuela 19, 1B, 28023 - Madrid, Spain.

EMA/PDCO/273942/2021
Amsterdam, 23 July 2021

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

EMEA-002887-PIP01-20

Scope of the application

Active substance(s):

Ribitol

Condition(s):

Treatment of limb-girdle muscular dystrophy

Pharmaceutical form(s):

Powder for oral use

Age-appropriate oral dosage form

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

Premier Research Group S.L.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Premier Research Group S.L. submitted for agreement to the European Medicines Agency on 11 September 2020 an application for a paediatric investigation plan for the above mentioned medicinal product and a waiver under Article 13 of said Regulation.

The procedure started on 13 October 2020.

Supplementary information was provided by the applicant on 17 April 2021. The applicant proposed modifications to the paediatric investigation plan and requested a deferral and withdrew its request for a 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.

   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 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

Not applicable.

2. Paediatric investigation plan

2.1. Condition:

Treatment of limb-girdle muscular dystrophy

2.1.1. Indication(s) targeted by the PIP

Treatment of paediatric patients from birth to less than 18 years of age with a genetically confirmed diagnosis of Limb-Girdle muscular dystrophy (LGMD) type 2i/ LGMD R9-fukutin related or subtypes LGMD2M and LGMD2U

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Powder for oral use

Age-appropriate oral dosage form

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	**Study 1** Development of an age-appropriate oral dosage form (powder for oral use formulation in sachets) suitable for children younger than 12 years of age including age-appropriate excipients and age-appropriate flavourings
Non-clinical studies	1	**Study 2** Definitive juvenile toxicity study in rats
Clinical studies	4	**Study 3** Open label, single arm study to assess the safety, tolerability and pharmacokinetics of ascending doses of ribitol in paediatric patients from 12 years to less than 18 years of age (and adults) with Limb Girdle muscular dystrophy (LGMD) 2I (MLB-01-003) **Study 4** Double blind, placebo controlled study to assess pharmacokinetics, safety and efficacy of ribitol compared to placebo in paediatric patients

		from birth to less than 18 years of age (and adults) with Limb Girdle muscular dystrophy (LGMD) 2I (MLB-01-004) **Study 5** Open label, single arm study to assess the safety, tolerability, pharmacokinetics and clinical activity of ribitol in paediatric patients from birth to less than 18 years of age (and adults) with Limb Girdle muscular dystrophy type 2M and 2U (MLB-01-006) **Study 6** Observational study aimed at collecting data on clinical outcome assessments in paediatric patients from birth to less than 18 years of age (and adults) with Limb Girdle muscular dystrophy type 2 (MLB-01-001)
Extrapolation, modelling and simulation studies	2	**Study 7** Modelling and simulation study to further determine the dose of ribitol to be used in paediatric patients from birth to less than 18 years of age with Limb Girdle muscular dystrophy type 2 **Study 8** Exposure-Response (E-R) model to describe the relationship between ribitol and ADG glycosylation in paediatric patients from birth to less than 18 years of age with Limb Girdle muscular dystrophy type 2
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 March 2026
Deferral for one or more measures contained in the paediatric investigation plan:	Yes