EMA/149480/2022

European Medicines Agency decision

P/0143/2022

of 13 April 2022

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for pamrevlumab (EMEA-002979-PIP01-21) 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 Union procedures for the authorisation and supervision of medicinal products for human use and establishing a European Medicines Agency[2],

Having regard to the application submitted by FibroGen, Inc. on 15 February 2021 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 25 February 2022, 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for pamrevlumab, solution for infusion, intravenous 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 pamrevlumab, solution for infusion, intravenous 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 pamrevlumab, solution for infusion, intravenous 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 FibroGen, Inc., 40 Illinois Street, 94158 - San Fransciso, United States.

EMA/PDCO/697848/2021 Corr
Amsterdam, 25 February 2022

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

EMEA-002979-PIP01-21

Scope of the application

Active substance(s):

Pamrevlumab

Condition(s):

Treatment of Duchenne muscular dystrophy

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

FibroGen, Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, FibroGen, Inc. submitted for agreement to the European Medicines Agency on 15 February 2021 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 23 March 2021.

Supplementary information was provided by the applicant on 22 November 2021. The applicant proposed modifications to the paediatric investigation plan and requested a deferral.

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 Duchenne muscular dystrophy (DMD)

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- solution for infusion, intravenous 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 Duchenne muscular dystrophy

2.1.1. Indication(s) targeted by the PIP

Treatment of Duchenne muscular dystrophy

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

From 2 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Solution for infusion

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable
Non-clinical studies	**Study 1** Juvenile 6-week toxicity study of pamrevlumab in rats, with a 1-month recovery period
Clinical studies	**Study 2** Open-label, pharmacodynamic and pharmacokinetic (PK) study of pamrevlumab in non-ambulant paediatric patients (and adults) from 12 years to less than 18 years of age with DMD (FGCL-3019-079)

	Study 3 Randomised, double-blind efficacy and safety study of pamrevlumab versus placebo in combination with systemic corticosteroids, in non-ambulant paediatric patients (and adults) from 12 years to less than 18 years of age with DMD (FGCL-3019-093) **Study 4** Randomised, double-blind efficacy and safety study of pamrevlumab versus placebo in combination with systemic corticosteroids, in ambulant paediatric patients from 6 years to less than 12 years of age with DMD (FGCL-3019-094) **Study 5** Single arm, two-part dose determination and dose expansion study to evaluate the safety of pamrevlumab in combination with Standard of Care in ambulant paediatric patients from 2 years to less than 6 years of age with DMD (FGCL-3019-110)
Extrapolation, modelling and simulation studies	**Study 6** Physiologically based pharmacokinetic (PK) modelling to describe PK of pamrevlumab (FIB-1B) **Study 7** Population PK model and exposure-response (ER) model that utilises post-hoc estimates from the population PK model to describe and compare the PK exposures and ER relationships of pamrevlumab between the different age groups with DMD.
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 March 2028
Deferral for one or more measures contained in the paediatric investigation plan:	Yes