EMA/484648/2024

European Medicines Agency decision

P/0377/2024

of 24 October 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for dazukibart (EMEA-003089-PIP02-23) 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 Pfizer Europe MA EEIG on 6 September 2023 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 16 October 2024, 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 dazukibart, concentrate for solution for infusion, parenteral 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 dazukibart, concentrate for solution for infusion, parenteral 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 dazukibart, concentrate for solution for infusion, parenteral 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 Pfizer Europe MA EEIG, Boulevard de la Plaine 17, 1050 – Brussels, Belgium.

EMA/PDCO/290116/2024 Rev [1]
Amsterdam, 16 October 2024

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

EMEA-003089-PIP02-23

Scope of the application

Active substance(s):

Dazukibart

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of idiopathic inflammatory myopathy

Pharmaceutical form(s):

Concentrate for solution for infusion

Route(s) of administration:

Parenteral use

Name/corporate name of the PIP applicant:

Pfizer Europe MA EEIG

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Pfizer Europe MA EEIG submitted for agreement to the European Medicines Agency on 6 September 2023 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 16 October 2023.

[1] Revision 16 October 2024

Supplementary information was provided by the applicant on 30 May 2024. The applicant proposed modifications to the paediatric investigation plan.

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)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset of the paediatric population.

   The Paediatric Committee member of Norway 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 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 idiopathic inflammatory myopathy

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- concentrate for solution for infusion, parenteral use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset.

2. Paediatric investigation plan

2.1. Condition:

Treatment of idiopathic inflammatory myopathy

2.1.1. Indication(s) targeted by the PIP

Treatment of idiopathic inflammatory myopathy (IIM)

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)

Concentrate for solution for infusion

2.1.4. Measures

Area	Description
Quality-related studies	**Study 1** Development of a pharmaceutical form of appropriate strength that is suitable for administration in paediatric patients from 2 years of age.
Non-clinical studies	**Study 2 (20LJ053)** 6-month chronic toxicity study of dazukibart in cynomolgus monkeys. **Study 3 (21GR024)** Enhanced pre- and postnatal development toxicity study to evaluate the potential effects of dazukibart in cynomolgus monkeys on pregnancy and parturition, embryo-fetal

	development, and the survival, growth, and postnatal development of offspring.
Clinical studies	**Study 4 (C0251009)** Double-blind, randomised, placebo controlled trial to evaluate pharmacokinetics, safety, and efficacy of dazukibart as add-on to standard of care in adolescents from 12 years to less than 18 years of age with active juvenile idiopathic inflammatory myopathy (JIIM). **Study 5 (C0251016)** Single-arm, open-label trial to evaluate dazukibart pharmacokinetics, pharmacodynamics, safety, and activity as add-on to standard of care in children from 2 years to less than 12 years of age with active juvenile idiopathic inflammatory myopathy.
Modelling and simulation analyses	**Study 6** Population Pharmacokinetic and Pharmacodynamic model in adult dermatomyositis and polymyositis (DM and PM), and juvenile dermatomyositis and polymyositis (JDM and JPM) to confirm dosing for adolescent subjects (from 12 years to less than 18 years of age) and to provide initial dosing recommendation to be used in PIP Study 5 (C0251016) (from 2 years to less than 12 years of age) population. **Study 7** Exposure-response Model in adult DM and PM, and adolescent JDM and JPM patients (from 12 years to less than 18 years of age) to be used as a basis for extrapolation of efficacy from adolescent JDM population to the adolescent JPM population. **Study 8** Population Pharmacokinetic and Pharmacodynamic model in children from 2 years to less than 12 years of age to characterize the PK in paediatric participants and to simulate exposure and biomarker levels to be used as a basis for extrapolation for the children JDM and JPM population. **Study 9** Exposure-response model in children from 2 years to less than 12 years of age to be used as a basis for extrapolation of efficacy from adolescents with JDM and JPM to children with JDM and children with JPM.
Other studies	Not applicable

Extrapolation plan	Studies 4, 5, 6, 7, 8 and 9 are part of the extrapolation plan of efficacy data from adult, adolescent (from 12 years to less than 18 years) and children (from 2 to less than 12 years) patients with the condition of IIM.

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 May 2035
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.