

EMA/127821/2023

European Medicines Agency decision

P/0141/2023

of 14 April 2023

on the acceptance of a modification of an agreed paediatric investigation plan for zilucoplan (EMA-002747-PIP01-20-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 for zilucoplan (EMA-002747-PIP01-20-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 726/2004¹,

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 Agency²,

Having regard to the European Medicines Agency's decision P/0143/2021 issued on 14 April 2021,

Having regard to the application submitted by UCB Pharma SA on 18 November 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 24 February 2023, 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 zilucoplan, solution for injection in pre-filled syringe, age-appropriate dosage form for parenteral use, subcutaneous 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 UCB Pharma SA, 60 Allée de la Recherche, 1070 – Bruxelles, Belgium.

EMA/PDCO/905241/2022
Amsterdam, 24 February 2023

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

EMA-002747-PIP01-20-M01

Scope of the application

Active substance(s):

Zilucoplan

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of myasthenia gravis

Pharmaceutical form(s):

Solution for injection in pre-filled syringe

Age-appropriate dosage form for parenteral use

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

UCB Pharma SA

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, UCB Pharma SA submitted to the European Medicines Agency on 18 November 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/0143/2021 issued on 14 April 2021.

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

The procedure started on 3 January 2023.

Scope of the modification

Some measures of the Paediatric Investigation Plan have been modified.

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.

The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

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 myasthenia gravis

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- solution for injection in pre-filled syringe, age-appropriate dosage form for parenteral use, subcutaneous use;
- on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s).

2. Paediatric investigation plan

2.1. Condition:

Treatment of myasthenia gravis

2.1.1. Indication(s) targeted by the PIP

Treatment of acetylcholine antibody receptor positive (AChR-Ab+) generalised myasthenia gravis

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 injection in pre-filled syringe

Age-appropriate dosage form for parenteral use

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Development of an age-appropriate dosage form for parenteral use appropriate for children from 2 years to less than 12 years of age, including development of a medical administration device with suitable graduation appropriate for children with body weight from 10 to less than 30 kg
Non-clinical studies	Not applicable.

Clinical studies	Study 2 Open-label, uncontrolled trial to evaluate pharmacokinetics, pharmacodynamics, safety, tolerability and activity of zilucoplan in children from 2 years to less than 18 years of age with generalised myasthenia gravis
Extrapolation, modelling and simulation studies	Study 3 Modelling and simulation study to establish the doses of zilucoplan in children from 2 years to less than 18 years of age with generalised myasthenia gravis Study 4 Analysis of existing data to extrapolate efficacy of zilucoplan from adults with generalised myasthenia gravis (gMG) to the paediatric population
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:	Yes
Date of completion of the paediatric investigation plan:	By September 2026
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.