EMA/401558/2023

European Medicines Agency decision

P/0437/2023

of 27 October 2023

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for uproleselan (EMEA-003307-PIP01-22) 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.

European Medicines Agency decision

P/0437/2023

of 27 October 2023

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for uproleselan (EMEA-003307-PIP01-22) 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 application submitted by GlycoMimetics, Inc. on 9 September 2022 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 8 September 2023, 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 uproleselan, solution for injection, 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 uproleselan, solution for injection, 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 uproleselan, solution for injection, 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 GlycoMimetics, Inc., 9708 Medical Center Drive, 20850 - Rockville, United States.

EMA/PDCO/267243/2023 Corr [1]
Amsterdam, 8 September 2023

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

EMEA-003307-PIP01-22

Scope of the application

Active substance(s):

Uproleselan

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of acute myeloid leukaemia

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

GlycoMimetics, Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, GlycoMimetics, Inc. submitted for agreement to the European Medicines Agency on 9 September 2022 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 17 October 2022.

Supplementary information was provided by the applicant on 2 May 2023. The applicant proposed modifications to the paediatric investigation plan.

[1] 26 October 2023

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 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 acute myeloid leukaemia

The waiver applies to:

- the paediatric population from birth to less than 28 days of age;
- solution for injection, intravenous 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 acute myeloid leukaemia

2.1.1. Indication(s) targeted by the PIP

In combination with chemotherapy, for the treatment of relapsed/ refractory acute myeloid leukaemia

In combination with chemotherapy, for the treatment of newly-diagnosed acute myeloid leukaemia

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

From 28 days to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Solution for injection.

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	Not applicable
Clinical studies	**Study 1** (PEPN2113) Open-label, single arm trial to evaluate the pharmacokinetics, safety and antitumor activity of uproleselan used in combination with fludarabine and cytarabine (FLA) in children from 1 year to less than 18 years of age with second or greater relapse or refractory acute myeloid leukaemia (AML), myelodysplastic syndrome (MDS) or mixed phenotype acute leukaemia (MPAL), including a dose-finding phase and an expansion phase.

	Study 2 (GMI-1271-30x) Randomised, double-blind, multicentre trial to evaluate the efficacy, safety and pharmacokinetics of uproleselan used in combination with chemotherapy compared to chemotherapy alone in children from 28 days to less than 18 years of age with relapsed or refractory acute myeloid leukaemia (AML). **Study 3** (GMI-1271-30y) Randomised, double-blind, multicentre trial to evaluate the efficacy, safety and pharmacokinetics of uproleselan used in combination with chemotherapy compared to chemotherapy alone in children from 28 days to less than 18 years of age with newly diagnosed acute myeloid leukaemia (AML).
Modelling and simulation studies	Not applicable
Other studies	Not applicable
Extrapolation plan	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:	December 2033
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.