EMA/413936/2024

European Medicines Agency decision

P/0332/2024

of 13 September 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral for mannose-1-phosphate (GLM101), (EMEA-003460-PIP01-23) 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 Glycomine Inc on 30 May 2023 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 26 July 2024, 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for mannose-1-phosphate (GLM101), 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 mannose-1-phosphate (GLM101), 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

This decision is addressed to Glycomine Inc, 733 Industrial Road, 94070 - San Carlos, USA.

EMA/PDCO/224528/2024
Amsterdam, 26 July 2024

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

EMEA-003460-PIP01-23

Scope of the application

Active substance(s):

Mannose-1-phosphate (GLM101)

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of phosphomannomutase 2-congenital disorder of glycosylation

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Glycomine Inc

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Glycomine Inc submitted for agreement to the European Medicines Agency on 30 May 2023 an application for a paediatric investigation plan for the above mentioned medicinal product.

The procedure started on 10 July 2023.

Supplementary information was provided by the applicant on 26 April 2024. 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.

   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 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

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Treatment of phosphomannomutase 2-congenital disorder of glycosylation

2.1.1. Indication(s) targeted by the PIP

Treatment of phosphomannomutase 2-congenital disorder of glycosylation (PMM2-CDG)

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)

Solution for infusion

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	**Study 1 (20347168)** Juvenile animal study in rats to support evaluation of safety of mannose-1-phosphate in the paediatric population.
Clinical studies	**Study 2 (GLY-000)** Observational study to evaluate natural history of the disease in patients with phosphomannomutase 2-congenital disorder of glycosylation (PMM2-CDG). **Study 3 (GLM101-002, 2022-000565-40)** Open-label, uncontrolled study to evaluate safety, tolerability, pharmacokinetic and pharmacodynamic profile of mannose-1-phosphate (GLM101) in paediatric patients from 2 years to less than 18 years of age (and adults) with PMM2-CDG. **Study 4 (GLM101-004)** Open-label, uncontrolled study to evaluate safety, tolerability and activity of mannose-1-phosphate (GLM101) in paediatric patients from birth to 3 years of age with PMM2-CDG.

	Study 5 (GLM101-007) Open-label study to evaluate long-term safety and activity of GLM101 in paediatric PMM2-CDG patients who completed Study 2 or 3 for long-term follow-up and continued access. **Study 6 (GLM101-005)** Double-blind randomised placebo controlled study to evaluate safety and efficacy of GLM101 in paediatric patients from 4 years to less than 18 year of age (and adults).
Modelling and simulation analyses	**Study 7** Modelling and simulation population pharmacokinetics study to evaluate the use of the product in the treatment of PMM2-CDG in children from birth to less than 18 years of age.
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:	Yes
Date of completion of the paediatric investigation plan:	By 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.