EMA/412945/2024

European Medicines Agency decision

P/0327/2024

of 13 September 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for seralutinib (EMEA-002972-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 Gossamer Bio 002 Limited on 6 August 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 26 July 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 seralutinib, inhalation powder, hard capsule, inhalation 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 seralutinib, inhalation powder, hard capsule, inhalation 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 seralutinib, inhalation powder, hard capsule, inhalation 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 Gossamer Bio 002 Limited, 10 Earlsfort Terrace, D02T380 – Dublin, Ireland.

EMA/PDCO/244156/2024
Amsterdam, 26 July 2024

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

EMEA-002972-PIP02-23

Scope of the application

Active substance(s):

Seralutinib

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of pulmonary arterial hypertension

Pharmaceutical form(s):

Inhalation powder, hard capsule

Route(s) of administration:

Inhalation use

Name/corporate name of the PIP applicant:

Gossamer Bio 002 Limited

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Gossamer Bio 002 Limited submitted for agreement to the European Medicines Agency on 6 August 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 11 September 2023.

Supplementary information was provided by the applicant on 29 April 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)(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 pulmonary arterial hypertension

The waiver applies to:

- the paediatric population from birth to less than 4 years of age;
- Inhalation powder, hard capsule, inhalation use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

2. Paediatric investigation plan

2.1. Condition:

Treatment of pulmonary arterial hypertension

2.1.1. Indication(s) targeted by the PIP

Treatment of symptomatic pulmonary arterial hypertension in children aged 4 years and over who are Functional Class II-IV.

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

From 4 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Inhalation powder, hard capsule

2.1.4. Measures

Area	Description
Quality-related studies	Study 1 Development of an age-appropriate dosage strength Study 2 Usability and acceptability study
Non-clinical studies	Study 3 Dose-range finding study Study 4 Juvenile toxicity study

Clinical studies	Study 5 Randomised, double-blind, placebo-controlled study to evaluate the pharmacodynamics, pharmacokinetics, efficacy and safety of seralutinib as add-on to standard of care in patients from 4 years to less than 18 years with pulmonary arterial hypertension (PAH).
Modelling and simulation analyses	Study 6 I*n silico* computational fluid dynamics (CFD) model to simulate the behaviour of the inhaled seralutinib drug product to support initial paediatric dose selection. Study 7 Modelling and simulation analyses to support dose finding of seralutinib in children from 4 years to less than 18 years of age with pulmonary arterial hypertension to be used in clinical study GB002-2103 (Study 5). Study 8 Use of PopPK/PD to support extrapolation of efficacy of seralutinib to treat PAH from adults to children aged 4 years to less than 18 years.
Other studies	Study 9 Analysis of in-house, external clinical and literature data on mechanism of action, pharmacodynamics, efficacy, and safety of inhaled platelet derived growth factor receptor/ tyrosine-protein kinase kit (PDGFR/cKit) inhibitors in the treatment of pulmonary arterial hypertension.
Extrapolation plan	Studies 5, 6, 7, 8 and 9 are part of the extrapolation plan of efficacy data from adult to the paediatric population from 4 years to less than 18 years of age with pulmonary arterial hypertension.

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 2032
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.