EMA/128509/2024

European Medicines Agency decision

P/0089/2024

of 27 March 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral for sargramostim (EMEA-003568-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 Partner Therapeutics, Inc. on 18 December 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 22 March 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 sargramostim, powder for solution for injection, subcutaneous 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 sargramostim, powder for solution for injection, subcutaneous 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 Partner Therapeutics, Inc., 19 Muzzey Street, MA 02421 – Lexington, USA.

EMA/PDCO/22801/2024
Amsterdam, 22 March 2024

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

EMEA-003568-PIP01-23

Scope of the application

Active substance(s):

Sargramostim

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of acute radiation syndrome

Pharmaceutical form(s):

Powder for solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Partner Therapeutics, Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Partner Therapeutics, Inc. submitted for agreement to the European Medicines Agency on 18 December 2023 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 22 January 2024.

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 acute radiation syndrome

2.1.1. Indication(s) targeted by the PIP

Treatment of children acutely exposed to myelosuppressive doses of radiation (hematopoietic syndrome of acute radiation syndrome).

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)

Powder for solution for injection

2.1.4. Measures

Area	**Description**
Quality-related studies	Not applicable
Non-clinical studies	**Study 1 (TSK0143)** Pilot efficacy study of sargramostim in irradiated rhesus monkeys. **Study 2 (TSK0144, TSK0144-amend1)** Randomized, blinded, placebo-controlled efficacy study of sargramostim in irradiated rhesus monkeys. **Study 3** (**FY14-045, FY14-045-amend1)** Nonclinical study evaluating the survival benefit from sargramostim relative to control groups in an established haematopoietic subsyndrome of acute radiation syndrome (H-ARS) model in male rhesus monkeys. **Study 4 (1017-3493)** Sargramostim - blinded time to dosing efficacy study in irradiated rhesus monkeys that have received minimal supportive care.
Clinical studies	**Study 5 (PTX-01-009 H-ARS)** Retrospective, observational study to assess the clinical benefit and safety of sargramostim in children from birth to less than 18 years of

	age exposed to myelosuppressive doses of radiation following a radiological and/or nuclear incident in a European Union member state or country from the European Economic Area.
Modelling and simulation analyses	**Study 6 (POH0547)** Population Pharmacokinetic (PopPK) analysis of sargramostim in healthy adults and application to exposure simulation for adult and paediatric populations.
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:	Six months after the conclusion of study 5. Study 5 is to be conducted in case a radiological and/or nuclear event in a European Union member state or country from the European Economic Area in which children from birth to 18 years of age have confirmed radiation exposure > 0.7 Gy.
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.