EMA/483453/2024

European Medicines Agency decision

P/0372/2024

of 25 October 2024

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for lenacapavir sodium (Sunleca), (EMEA-002740-PIP03-24) 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 Gilead Sciences International Limited (GSIL) on 3 June 2024 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 6 September 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 lenacapavir sodium (Sunleca), solution for injection, film-coated tablet, age-appropriate oral and/or subcutaneous dosage form , subcutaneous use, oral 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 lenacapavir sodium (Sunleca), solution for injection, film-coated tablet, age-appropriate oral and/or subcutaneous dosage form, subcutaneous use, oral 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 lenacapavir sodium (Sunleca), solution for injection, film-coated tablet, age-appropriate oral and/or subcutaneous dosage form, subcutaneous use, oral 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 covers all conditions, indications, pharmaceutical forms, routes of administration, measures, timelines, waivers and deferrals, as agreed in the decision P/0005/2021 issued on 15 January 2021, including subsequent modifications thereof.

Article 5

This decision is addressed to Gilead Sciences International Limited (GSIL), Granta Park, Flowers Building, Great Abington, CB21 6GT – Cambridge, United Kingdom.

EMA/PDCO/379892/2024 Corr[1] Corr[2]
Amsterdam, 6 September 2024

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

EMEA-002740-PIP03-24

Scope of the application

Active substance(s):

Lenacapavir sodium

Invented name and authorisation status:

See Annex II

Condition(s):

Prevention of human immunodeficiency virus (HIV-1) infection

Pharmaceutical form(s):

Solution for injection

Film-coated tablet

Age-appropriate oral and/or subcutaneous dosage form

Route(s) of administration:

Subcutaneous use

Oral use

Name/corporate name of the PIP applicant:

Gilead Sciences International Limited (GSIL)

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Gilead Sciences International Limited (GSIL) submitted for agreement to the European Medicines Agency on 3 June 2024 an

[1] 18 September 2024
[2] 21 October 2024

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 8 July 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;
   - 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:

Prevention of human immunodeficiency virus (HIV-1) infection

The waiver applies to:

- the paediatric population from 2 years to less than 12 years of age;
- solution for injection, subcutaneous use; film-coated tablet, oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2. Paediatric investigation plan

2.1. Condition:

Prevention of human immunodeficiency virus (HIV-1) infection

2.1.1. Indication(s) targeted by the PIP

HIV-1 pre-exposure prophylaxis (PrEP) in adolescents

HIV-1 infant postnatal prophylaxis (PNP)

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

From birth to less than 2 years of age and from 12 to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Solution for injection

Film-coated tablet

2.1.4. Measures

Area	**Description**
Quality-related studies	**Study 1** Development of age-appropriate formulation of lenacapavir sodium (LEN) for infants and children from birth to less than 2 years of age for subcutaneous administration and/or for oral induction.
Non-clinical studies	**Study 2** Oral (gavage) juvenile toxicity study of LEN to determine the potential adverse effects of its long-term oral

	administration on neonatal growth and development in juvenile male and female rats (TX-200-2084).
Clinical studies	**Study 3** Double-blind, randomized study to evaluate safety and efficacy of subcutaneous lenacapavir (LEN) and oral emtricitabine/tenofovir alafenamide (F/TAF) for pre-exposure prophylaxis (PrEP) in adolescent girls from 16 to less than 18 years of age and young women at risk of HIV infection. GS-US-412-5624 (PURPOSE 1). **Study 4** Double-blind, randomized study to evaluate the efficacy and safety of subcutaneous lenacapavir (LEN) for HIV pre-exposure prophylaxis (PrEP) in cisgender men, transgender women, transgender men, and gender nonbinary people from 16 years of age who have sex with male partners and are at risk for HIV infection. GS-US-528-9023 (PURPOSE 2). **Study 5** Controlled with standard of care (SOC) study to evaluate efficacy pharmacokinetics (PK), safety and tolerability of lenacapavir (LEN) in infants and children from birth to less than 2 years of age exposed to HIV (and not diagnosed with HIV-1 infection).
Modelling and simulation analyses	**Study 6** Modelling and simulation study to support extrapolation of lenacapavir (LEN) safety and efficacy data from adults to adolescents from 12 to less than 18 years of age weighing at least 35 kg for pre-exposure prophylaxis (PrEP). **Study 7** Modelling and simulation study to select lenacapavir (LEN) dose(s) to be evaluated in the proposed post-natal prophylaxis clinical PIP Study 5.
Other studies	Not applicable.
Extrapolation plan	Studies 3, 4, 6 are part of an extrapolation plan covering the paediatric population from 12 years to less than 18 years of age, as agreed by the PDCO.

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:	By June 2031
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:

Condition(s) and authorised indication(s)

1. Treatment of human immunodeficiency virus (HIV-1) infection

Authorised indication(s):

- Sunlenca injection, in combination with other antiretroviral(s), is indicated for the treatment of adults with multidrug resistant HIV-1 infection for whom it is otherwise not possible to construct a suppressive anti-viral regimen
- Sunlenca tablet, in combination with other antiretroviral(s), is indicated for the treatment of adults with multidrug resistant HIV-1 infection for whom it is otherwise not possible to construct a suppressive anti-viral regimen, for oral loading prior to administration of long-acting Lenacapavir injection
  - Invented name(s): Sunleca
  - Authorised pharmaceutical form(s): solution for injection, film-coated tablet
  - Authorised route(s) of administration: subcutaneous use, oral use
  - Authorised via centralised procedure