**MHRA**
10 South Colonnade
Canary Wharf
London
E14 4PU
United Kingdom

**gov.uk/mhra**

# Decision Cover Letter

## Decision of the licensing authority to:

accept change(s) to the agreed paediatric investigation plan (MHRA-100428-PIP01-22-M01) and to the deferral

MHRA-100428-PIP01-22-M02

# Scope of the Application

## Active Substance(s)

LENACAPAVIR SODIUM

## Condition(s)

Treatment of human immunodeficiency virus (HIV-1) infection

## Pharmaceutical Form(s)

Solution for injection , Film-coated tablet, Age-appropriate oral solid dosage form

## Route(s) of Administration

SUBCUTANEOUS USE, ORAL USE

## Name / Corporate name of the PIP applicant

Gilead Sciences Ltd

## Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Gilead Sciences Ltd submitted to the licensing authority on 28/03/2024 17:19 GMT an application for a Modification

The procedure started on 04/06/2024 10:56 BST

1. The licensing authority, having assessed the application in accordance with the Human Medicines Regulations 2012, decides, as set out in the appended summary report:

to accept change(s) to the agreed paediatric investigation plan and to the deferral

2. The measures and timelines of the paediatric investigation plan are set out in the Annex I.

This decision is forwarded to the applicant, together with its annex and appendix.

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# Final Decision Letter

MHRA-100428-PIP01-22-M02

Of 02/08/2024 13:44 BST

On the adopted decision for LENACAPAVIR SODIUM (MHRA-100428-PIP01-22-M02) in accordance with the Human Medicines Regulations 2012.

The licensing authority, in accordance with the Human Medicines Regulations 2012, has adopted this decision:

Agreement on modification of a paediatric investigation plan (including modification of a waiver or deferral included in that paediatric investigation plan)

This decision applies to a Modification for LENACAPAVIR SODIUM, Solution for injection , Film-coated tablet, Age-appropriate oral solid dosage form , SUBCUTANEOUS USE, ORAL USE .

This decision is addressed to Gilead Sciences Ltd, 280 High Holborn, London, UNITED KINGDOM, WC1V 7EE

## ANNEX I

### 1. Waiver

### 1.1 Condition:

| |
|---|
| Treatment of human immunodeficiency virus (HIV-1) infection The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 2 years of age Pharmaceutical form(s): Solution for injection Film-coated tablet Age-appropriate oral solid dosage form Route(s) of administration: SUBCUTANEOUS USE ORAL USE Reason for granting waiver: 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(s):

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

## 2.2 Indication(s) targeted by the PIP:

Heavily treated experienced (HTE) children and adolescents (from 6 years to less than 18 years of age): in combination with an optimised background regimen for the treatment of patients infected with multidrug resistant (MDR) HIV-1 infection. Virologically suppressed (VS) children and adolescents (from 2 years to less than 18 years of age): in combination with partner agent as a complete regimen for the treatment of children and adolescents living with HIV-1 to replace the current antiretroviral therapy (ARV) regimen in those who are VS (HIV-1 RNA 50 copies/mL) on a stable ARV regimen.

## 2.3 Subset(s) of the paediatric population concerned by the paediatric development:

The paediatric population from 2 years to less than 18 years of age

## 2.4 Pharmaceutical Form(s):

Solution for injection Film-coated tablet Age-appropriate oral solid dosage form

## 2.5 Studies:

| Study Type | Number of Studies | Study Description |
|---|---|---|
| **Quality Measures** | 1 | Study 1 Development of an oral formulation for use in children from 2 years to less than 12 years. |
| **Non-Clinical Studies** | 0 | Not applicable. |
| **Clinical Studies** | 1 | Study 2 Open-label, single-arm, multicohort study to evaluate pharmacokinetics (PK), safety, tolerability, and activity of lenacapavir from 2 years to less than 18 years of age with HIV infection, who are virologically suppressed (VS). |
| **Extrapolation, Modeling & Simulation Studies** | 2 | Study 3 Development of a population PK model in the adult population to predict lenacapavir exposures in HTE and VS paediatric subjects for 2 years to less than 18 years of age and support lenacapavir paediatric dosing. Study 4 Analysis of similarity in exposure-response relationship to support the extrapolation of efficacy of lenacapavir in HTE children and adolescents from 6 years to less than 18 years of age. |

| | | |
|---|---|---|
| **Other Studies** | 0 | Not applicable. |
| **Other Measures** | 0 | Not applicable. |

## 3. Follow-up, completion and deferral of a PIP:

| | |
|---|---|
| **Concerns on potential long term safety and efficacy issues in relation to paediatric use:** | No |
| **Date of completion of the paediatric investigation plan:** | 31/12/2027 |
| **Deferral of one or more studies contained in the paediatric investigation plan:** | Yes |