**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-101234-PIP01-23-M01

# Scope of the Application

## Active Substance(s)

ALIROCUMAB

## Condition(s)

Treatment of elevated cholesterol

## Pharmaceutical Form(s)

Solution for injection

## Route(s) of Administration

SUBCUTANEOUS USE

## Name / Corporate name of the PIP applicant

sanofi-aventis recherche & developpement

## Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, sanofi-aventis recherche & developpement submitted to the licensing authority on 07/11/2023 22:24 GMT an application for a Modification

The procedure started on 09/01/2024 07:06 GMT

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

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-101234-PIP01-23-M01

Of 15/01/2024 07:18 GMT

On the adopted decision for ALIROCUMAB (MHRA-101234-PIP01-23-M01) 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 ALIROCUMAB, Solution for injection , SUBCUTANEOUS USE .

This decision is addressed to sanofi-aventis recherche & developpement, 1 avenue Pierre Brossolette, Chilly Marzain, FRANCE, 91385

# ANNEX I

## 1. Waiver

### 1.1 Condition:

| |
|---|
| Treatment of elevated cholesterol The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 8 years of age Pharmaceutical form(s): Solution for injection Route(s) of administration: SUBCUTANEOUS 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 elevated cholesterol |

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

| |
|---|
| Treatment of heterozygous and homozygous familial hypercholesterolemia |

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

| |
|---|
| The paediatric population from 8 years to less than 18 years of age |

## 2.4 Pharmaceutical Form(s):

| |
|---|
| Solution for injection |

## 2.5 Studies:

| Study Type | Number of Studies | Study Description |
|---|---|---|
| **Quality Measures** | 0 | Study 1 Deleted during procedure MHRA-101234-PIP01-23-M01. |
| **Non-Clinical Studies** | 0 | Not applicable. |
| **Clinical Studies** | 3 | Study 2 Open-label, 8-week, sequential, repeated dose-finding study to evaluate the efficacy and safety of alirocumab in children and adolescents from 8 to less than 18 years of age with heterozygous familial hypercholesterolaemia (HeFH), followed by an optional extension phase. Study 3 Double-blind, randomised, placebo-controlled study followed by an open-label extension evaluating the efficacy and long-term safety and tolerability of alirocumab in children and adolescents from 8 years to less than 18 years of age with heterozygous familial hypercholesterolaemia (HeFH). Study 4 Open-label, single-arm, exploratory study evaluating the activity and safety of alirocumab in children and adolescents from 8 years to less than 18 years of age with homozygous familial hypercholesterolaemia (HoFH). |
| **Extrapolation, Modeling & Simulation Studies** | 0 | Not applicable. |
| **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:** | Yes |
| **Date of completion of the paediatric investigation plan:** | 30/09/2023 |
| **Deferral of one or more studies contained in the paediatric investigation plan:** | Yes |