**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-100117-PIP01-21-M01) and to the deferral

MHRA-100117-PIP01-21-M02

# Scope of the Application

## Active Substance(s)

ISATUXIMAB

## Condition(s)

Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue

## Pharmaceutical Form(s)

Concentrate for solution for infusion

## Route(s) of Administration

INTRAVENOUS 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 12/12/2022 10:33 GMT an application for a Modification

The procedure started on 21/02/2023 09:15 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 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-100117-PIP01-21-M02

Of 06/03/2023 17:25 GMT

On the adopted decision for ISATUXIMAB (MHRA-100117-PIP01-21-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 ISATUXIMAB, Concentrate for solution for infusion , INTRAVENOUS USE .

This decision is addressed to Sanofi-Aventis Recherche & Developpement, 1 avenue Pierre Brossolette, Chilly-Mazarin, FRANCE, 91385

# ANNEX I

## 1. Waiver

### 1.1 Condition:

| |
|---|
| Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue The waiver applies / applied to: Paediatric Subset(s): The paediatric population from birth to less than 28 days of age Pharmaceutical form(s): Concentration for solution for infusion Route(s) of administration: Intravenous use Reason for granting waiver: on the grounds that clinical studies with the specific medicinal product cannot be expected to be of significant therapeutic benefit to or fulfil a therapeutic need of the specified paediatric subset(s). |

## 2. Paediatric Investigation Plan:

### 2.1 Condition(s):

| |
|---|
| Treatment of malignant neoplasms of the haematopoietic and lymphoid tissue |

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

Treatment of relapsed, refractory and newly-diagnosed acute lymphoblastic leukaemia in combination with standard treatment (chemotherapy) in paediatric patients from 28 days to less than 18 years of age. (Treatment indication for acute myeloid leukaemia deleted during procedure MHRA-100117-PIP01-21-M02 )

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

The paediatric population from 28 days to less than 18 years of age

## 2.4 Pharmaceutical Form(s):

Concentration for solution for infusion

## 2.5 Studies:

| Study Type | Number of Studies | Study Description |
|---|---|---|
| **Quality Measures** | 0 | Not applicable. |
| **Non-Clinical Studies** | 1 | Study 1 Paediatric preclinical testing study to evaluate the in vitro activity of isatuximab combinations with standards of care in preclinical models of acute myeloid leukaemia. |
| **Clinical Studies** | 3 | Study 2 Open-label, single-arm trial to evaluate pharmacokinetics, safety and antitumor activity of isatuximab used in combination with chemotherapy in children from 28 days to less than 18 years of age with relapsed/refractory B or T acute lymphoblastic leukaemia or acute myeloid leukaemia in first or second relapse. Study 3 Open-label, randomised controlled trial to evaluate the safety and efficacy of isatuximab used in combination with chemotherapy compared to chemotherapy in children from 28 days to less than 18 years of age with relapsed/refractory B or T acute lymphoblastic leukaemia or acute myeloid leukaemia in first or second relapse. The population to be included in the study is to be determined according to the |

| | | |
|---|---|---|
| | | results of Study 2. Study 4 Open-label, randomised controlled trial to evaluate the efficacy and safety of isatuximab used in combination with chemotherapy compared to chemotherapy in children from 28 days to less than 18 years of age with newly-diagnosed B or T acute lymphoblastic leukaemia or newly-diagnosed acute myeloid leukaemia. The population to be included in the study is to be determined according to the results of Study 3. |
| **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:** | No |
| **Date of completion of the paediatric investigation plan:** | 31/08/2035 |
| **Deferral of one or more studies contained in the paediatric investigation plan:** | Yes |