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

gov.uk/mhra

Decision Cover Letter

Decision of the licensing authority to:

agree a paediatric investigation plan and grant a deferral and grant a waiver

MHRA-100637-PIP01-22

Scope of the Application

Active Substance(s)

Mixture of 2 synthetic double-stranded N-Acetyl-galactosamine conjugated siRNA oligonucleotides that are directed against hepatitis B virus

Condition(s)

Treatment of chronic hepatitis D infection

Pharmaceutical Form(s)

Solution for injection; Age-appropriate dosage form for parenteral use.

Route(s) of Administration

SUBCUTANEOUS USE

Name / Corporate name of the PIP applicant

Janssen Cilag Ltd

Basis for the Decision

Pursuant to the Human Medicines Regulations 2012, Janssen Cilag Ltd submitted to the licensing authority on 15/08/2022 12:48 BST an application for a Paediatric Investigation Plan

The procedure started on 09/02/2023 17:11 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 agree a paediatric investigation plan and grant a deferral and grant a waiver

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.

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

gov.uk/mhra

Final Decision Letter

MHRA-100637-PIP01-22

Of 06/03/2023 11:42 GMT

On the adopted decision for Mixture of 2 synthetic double-stranded N-Acetyl-galactosamine conjugated siRNA oligonucleotides that are directed against hepatitis B virus (MHRA-100637-PIP01-22) 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 a paediatric investigation plan

This decision applies to a Paediatric Investigation Plan for Mixture of 2 synthetic double-stranded N-Acetyl-galactosamine conjugated siRNA oligonucleotides that are directed against hepatitis B virus, Solution for injection; Age-appropriate dosage form for parenteral use. , SUBCUTANEOUS USE .

This decision is addressed to Janssen Cilag Ltd, 50 - 100 Holmers Farm Way , High Wycombe, UNITED KINGDOM, HP12 4DP

ANNEX I

1. Waiver

1.1 Condition:

Treatment of chronic hepatitis D 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 Age-appropriate dosage form for parenteral use Route(s) of administration: SUBCUTANEOUS 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 chronic hepatitis D infection

2.2 Indication(s) targeted by the PIP:

Treatment of chronic hepatitis D infection

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

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

2.4 Pharmaceutical Form(s):

Solution for injection Age-appropriate dosage form for parenteral use

2.5 Studies:

Study Type	Number of Studies	Study Description
Quality Measures	1	Study 1 Development of an age-appropriate dosage form for parenteral use along with a dosing device, suitable for children from 2 years of age.
Non-Clinical Studies	0	Not applicable.
Clinical Studies	1	Study 2 Open-label, uncontrolled trial to evaluate pharmacokinetics, safety and activity of JNJ-73763989 in paediatric patients from 2 years to less than 18 years of age with chronic hepatitis D virus (HDV) infection.
Extrapolation, Modeling & Simulation Studies	2	Study 3 Modelling and simulation to characterise the pharmacokinetics and explore the pharmacodynamics of JNJ-73763989 in paediatric subjects from 2 years to less than 18 years of age with chronic HDV infection. Study 4 Extrapolation of pharmacokinetic, safety and efficacy data on JNJ-73763989 to paediatric subjects from 2 years to less than 18 years of age with chronic HDV infection.
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:	30/06/2028
Deferral of one or more studies contained in the paediatric investigation plan:	Yes