EMA/609177/2022

European Medicines Agency decision

P/0228/2022

of 8 July 2022

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for mixture of 2 synthetic double-stranded N-Acetyl-galactosamine conjugated siRNA oligonucleotides that are directed against hepatitis B virus (JNJ-73763989) (EMEA-002694-PIP02-21) 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 Janssen-Cilag International NV on 19 April 2021 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 20 May 2022, 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 mixture of 2 synthetic double-stranded N-Acetyl-galactosamine conjugated siRNA oligonucleotides that are directed against hepatitis B virus (JNJ-73763989), solution for injection, age-appropriate dosage form for parenteral use, subcutaneous 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 mixture of 2 synthetic double-stranded N-Acetyl-galactosamine conjugated siRNA oligonucleotides that are directed against hepatitis B virus (JNJ-73763989), solution for injection, age-appropriate dosage form for parenteral use, subcutaneous 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 mixture of 2 synthetic double-stranded N-Acetyl-galactosamine conjugated siRNA oligonucleotides that are directed against hepatitis B virus (JNJ-73763989), solution for injection, age-appropriate dosage form for parenteral use, subcutaneous 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 is addressed to Janssen-Cilag International NV, Turnhoutseweg 30, 2340 - Beerse, Belgium.

EMA/PDCO/147157/2022
Amsterdam, 20 May 2022

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

EMEA-002694-PIP02-21

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 (JNJ-73763989)

Condition(s):

Treatment of chronic hepatitis D virus 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 International NV

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Janssen-Cilag International NV submitted for agreement to the European Medicines Agency on 19 April 2021 an 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 25 May 2021.

Supplementary information was provided by the applicant on 21 February 2022. The applicant proposed modifications to the paediatric investigation plan.

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 annex 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:

Treatment of chronic hepatitis D virus infection

The waiver applies to:

- the paediatric population from birth to less than 2 years of age;
- solution for injection, age-appropriate dosage form for parenteral use, subcutaneous use;
- 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:

Treatment of chronic hepatitis D infection

2.1.1. Indication(s) targeted by the PIP

Treatment of chronic hepatitis D infection

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

From 2 years to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Solution for injection

Age-appropriate dosage form for parenteral use

2.1.4. Measures

Area	Description
Quality-related studies	**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	Not applicable.
Clinical studies	**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, modelling and simulation studies	**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	Not applicable.
Other measures	Not applicable.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By June 2028
Deferral for one or more measures contained in the paediatric investigation plan:	Yes