EMA/99042/2022

European Medicines Agency decision

P/0083/2022

of 11 March 2022

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for viltolarsen (EMEA-002853-PIP01-20) 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 NS Pharma, Inc. on 10 July 2020 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 21 January 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 viltolarsen, solution for infusion, intravenous 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 viltolarsen, solution for infusion, intravenous 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 viltolarsen, solution for infusion, intravenous 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 NS Pharma, Inc. 140 East Ridgewood Avenue, Suite 280S, Paramus, 07652 - New Jersey, USA.

EMA/619986/2021 Corr
Amsterdam, 21 January 2022

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

EMEA-002853-PIP01-20

Scope of the application

Active substance(s):

Viltolarsen

Condition(s):

Treatment of Duchenne muscular dystrophy

Pharmaceutical form(s):

Solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

NS Pharma, Inc.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, NS Pharma, Inc. submitted for agreement to the European Medicines Agency on 10 July 2020 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 18 August 2020.

Supplementary information was provided by the applicant on 18 October 2021. 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 Norwegian Paediatric Committee member 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 Duchenne muscular dystrophy

The waiver applies to:

- the paediatric population from birth to less than 6 months of age;
- solution for infusion, intravenous use;
- 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:

Treatment of Duchenne muscular dystrophy

2.1.1. Indication(s) targeted by the PIP

Treatment of Duchenne muscular dystrophy (DMD)

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

From 6 months to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Solution for infusion

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	0	Not applicable
Non-clinical studies	1	**Study 1** Carcinogenicity study in rats (TX11037).
Clinical studies	5	**Study 2** Randomized, double-blind, placebo-controlled, study to assess the efficacy and safety of viltolarsen in ambulant paediatric patients from 4 years to less than 8 years of age with Duchenne muscular dystrophy (DMD) (NS-065/NCNP-01-301)

		Study 3 Open-label extension study to assess the safety and efficacy of viltolarsen in ambulant paediatric patients with Duchenne muscular dystrophy (DMD) who have completed Study 2 NS-065/NCNP-01-301 (NS-065/NCNP-01-302) **Study 4** Open-label study to assess the efficacy and safety of viltolarsen in ambulant and non-ambulant paediatric patients from 8 years to less than 18 years of age with Duchenne muscular dystrophy (DMD) (NS-065/NCNP-01-211) **Study 5** Randomized, double-blind, placebo-controlled, study to assess the efficacy and safety of viltolarsen in non-ambulant paediatric patients from 8 years to less than 18 years of age with Duchenne muscular dystrophy (DMD) (NS-065/NCNP-01-311) **Study 6** Open-label study to assess sequentially the pharmacokinetics, safety, tolerability and efficacy of viltolarsen in two cohorts of ambulant paediatric patients from 2 to less than 4 years and from 6 months to less than 2 years of age with Duchenne muscular dystrophy. (DMD) (NS-065/NCNP-01-221)
Extrapolation, modelling and simulation studies	2	**Study 7** Population PK model to characterise PK of viltolarsen in paediatric patients and detect the covariates linked to variability of the exposure **Study 8** Modelling and simulation study to support dosing and to characterize PK/PD relationship to support extrapolation of efficacy for paediatric patients aged from 6 months up to 4 years of age or from 16 up to 18 years of age
Other studies	0	Not applicable
Other measures	0	Not applicable

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety/efficacy issues in relation to paediatric use:	No
Date of completion of the paediatric investigation plan:	By June 2033
Deferral for one or more measures contained in the paediatric investigation plan:	Yes