EMADOC-1700519818-1793517

European Medicines Agency decision

EMA/PE/0000181300

of 6 December 2024

on the acceptance of a modification of an agreed paediatric investigation plan for avalglucosidase alfa (Nexviadyme) 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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on the acceptance of a modification of an agreed paediatric investigation plan for avalglucosidase alfa (Nexviadyme) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 European Medicines Agency's decision P/0075/2017 issued on 17 March 2017, the decision P/0073/2019 issued on 22 March 2019, the decision P/0174/2020 issued on 13 May 2020 and the decision P/0360/2021 issued on 8 September 2021,

Having regard to the application submitted by Sanofi B.V. on 4 July 2024 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 18 October 2024, in accordance with Article 22 of Regulation (EC) No 1901/2006,

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 acceptance of changes to the agreed paediatric investigation plan and to the deferral.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan including changes to the deferral.

[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

Changes to the agreed paediatric investigation plan for avalglucosidase alfa (Nexviadyme), including changes to the deferral, are hereby accepted in the scope set out in the opinion of the Paediatric Committee of the European Medicines Agency annexed hereto, together with its appendices.

Article 2

This decision is addressed to Sanofi B.V., Paasheuvelweg 25, 1105 BP Amsterdam, Netherlands.

EMADOC-1700519818-1613614

Amsterdam, 18 October 2024

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan

EMA/PE/0000181300

Scope of the application

Active substance(s):

Avalglucosidase alfa

Invented name and authorisation status:

See Annex II

Condition(s):

Treatment of Pompe disease

Pharmaceutical form(s):

Powder for concentrate for solution for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Sanofi B.V.

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Sanofi B.V. submitted to the European Medicines Agency on 4 July 2024 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0075/2017 issued on 17 March 2017, the decision P/0073/2019 issued on 22 March 2019 and the decision P/0174/2020 issued on 13 May 2020 and the decision P/0360/2021 issued on 8 September 2021.

The application for modification proposed changes to the agreed paediatric investigation plan and to the deferral.

The procedure started on 19 August 2024.

Scope of the modification

Some timelines of the Paediatric Investigation Plan have been modified.

Opinion

1. The Paediatric Committee, having assessed the application in accordance with Article 22 of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

   - to agree to changes to the paediatric investigation plan and to the deferral in the scope set out in the Annex I of this opinion.

   The Paediatric Committee member of Norway agrees with the above-mentioned recommendation of the Paediatric Committee.

2. The measures and timelines of the paediatric investigation plan 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 annexes 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

2. Paediatric investigation plan

2.1. Condition

Treatment of Pompe disease

2.1.1. Indication(s) targeted by the PIP

Long-term ERT treatment of patients with Pompe disease (acid α-glucosidase deficiency)

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

From birth to less than 18 years of age

2.1.3. Pharmaceutical form(s)

Powder for concentrate for solution for infusion

2.1.4. Measures

Area	Description
Quality-related studies	Not applicable.
Non-clinical studies	Not applicable.
Clinical studies	**Study 1** deleted in EMEA-001945-PIP01-16-M02 **Study 2** (ACT14132) Open-label, multicentre, multinational, ascending dose, repeated intravenous infusion study of avalglucosidase alfa in treatment experienced paediatric patients from 6 months to less than 18 years of age with infantile-onset Pompe disease (IOPD) to evaluate the safety profile of and the pharmacokinetic profile of avalglucosidase alfa and to evaluate the preliminary efficacy of avalglucosidase alfa in comparison to alglucosidase alfa. **Study 3** (EFC14462) Open-label, multinational, multicentre study of avalglucosidase alfa in treatment-naïve paediatric patients from birth to less than 12 months of age with infantile onset Pompe disease (IOPD) to determine the safety, tolerability and effect of avalglucosidase alfa treatment.
Extrapolation, modelling and simulation studies	Not applicable
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:	No
Date of completion of the paediatric investigation plan:	By December 2025
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Information provided by the applicant:

Condition(s) and authorised indication(s)

1. Treatment of Pompe disease

Authorised indication(s):

- Nexviadyme (avalglucosidase alfa) is indicated for long-term enzyme replacement therapy for the treatment of patients with Pompe disease (acid α-glucosidase deficiency).
  - Invented name(s): Nexviadyme
  - Authorised pharmaceutical form(s): powder for concentrate for solution for infusion
  - Authorised route(s) of administration: intravenous use
  - Authorised via the centralised procedure