EMA/599313/2020

European Medicines Agency decision

P/0450/2020

of 1 December 2020

on the agreement of a paediatric investigation plan and on the granting of a deferral for bispecific antibody binding to clotting factor IX and X (Mim8) (EMEA-002762-PIP02-20) 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.

European Medicines Agency decision

P/0450/2020

of 1 December 2020

on the agreement of a paediatric investigation plan and on the granting of a deferral for bispecific antibody binding to clotting factor IX and X (Mim8) (EMEA-002762-PIP02-20) 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 Community procedures for the authorisation and supervision of medicinal products for human and veterinary use and establishing a European Medicines Agency[2],

Having regard to the application submitted by Novo Nordisk A/S on 20 February 2020 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 16 October 2020, in accordance with Article 17 of Regulation (EC) No 1901/2006 and Article 21 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.

(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.

[1] OJ L 378, 27.12.2006, p.1.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

A paediatric investigation plan for bispecific antibody binding to clotting factor IX and X (Mim8), solution for injection, 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 bispecific antibody binding to clotting factor IX and X (Mim8), solution for injection, 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

This decision is addressed to Novo Nordisk A/S, Vandtårnsvej 108-110, 2860 – Søborg, Denmark.

EMA/PDCO/437236/2020
Amsterdam, 16 October 2020

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

EMEA-002762-PIP02-20

Scope of the application

Active substance(s):

Bispecific antibody binding to clotting factor IX and X (Mim8)

Condition(s):

Treatment of haemophilia A

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Novo Nordisk A/S

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Novo Nordisk A/S submitted for agreement to the European Medicines Agency on 20 February 2020 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation.

The procedure started on 31 March 2020.

Supplementary information was provided by the applicant on 10 July 2020. 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.

   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 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

Not applicable.

2. Paediatric investigation plan

2.1. Condition:

Treatment of haemophilia A

2.1.1. Indication(s) targeted by the PIP

Routine prophylaxis to prevent or reduce the frequency of bleeding episodes in patients with haemophilia A (congenital factor VIII deficiency) with or without factor VIII inhibitors.

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)

Solution for injection.

2.1.4. Measures

Area	Number of measures	Description
Quality-related studies	1	***Study 1*** Development of 1 mg/ml strength suitable for the paediatric population.
Non-clinical studies	0	Not applicable.
Clinical studies	3	***Study 2*** **(4513 MAD)** Open-label multiple ascending dose trial to evaluate safety, pharmacokinetics and pharmacodynamics of Mim8 in adolescents from 12 to less than 18 years of age (and adults) with haemophilia A. ***Study 3*** **(4514)** Multicentre open-label trial in adolescents from 12 to less than 18 years of age (and adults) with haemophilia A to investigate efficacy and safety of Mim8 prophylaxis compared to on-demand treatment and compared to standard-of-care prophylaxis. ***Study 4*** **(4516)** Multicentre, open-label trial to investigate safety, efficacy, and exposure of Mim8 prophylaxis in children from 1 to less than 12 years of age with haemophilia A.

Extrapolation, modelling and simulation studies	2	***Study 5*** Modelling and simulation paediatric dose finding study. ***Study 6*** Analysis of existing in-house data from children and adolescents from 2 to less than 18 years of age (and adults) and use of a literature maturation PK model of the Factor-VIII mimetic, emicizumab, to describe exposure in boys with haemophilia A from birth to less than 2 years of age.

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 October 2023
Deferral for one or more measures contained in the paediatric investigation plan:	Yes