EMA/16279/2025

European Medicines Agency decision

P/0403/2024

of 15 January 2025

on the agreement of a paediatric investigation plan and on the granting of a deferral for humanized IgG4 Monoclonal Antibody against FIXa and FX (NXT007), (EMEA-003550-PIP01-23) 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 Roche Registration GmbH on 8 December 2023 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 18 October 2024, 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, as amended.
[2] OJ L 136, 30.4.2004, p. 1, as amended.

Has adopted this decision:

Article 1

A paediatric investigation plan for humanized IgG4 Monoclonal Antibody against FIXa and FX (NXT007), 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 humanized IgG4 Monoclonal Antibody against FIXa and FX (NXT007), 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 Roche Registration GmbH, Emil-Barell-Strasse 1, 79639 - Grenzach-Wyhlen, Germany.

EMA/PDCO/342858/2024
Amsterdam, 18 October 2024

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

EMEA-003550-PIP01-23

Scope of the application

Active substance(s):

Humanized IgG4 Monoclonal Antibody against FIXa and FX (NXT007)

Invented name and authorisation status:

See Annex II

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:

Roche Registration GmbH

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Roche Registration GmbH submitted for agreement to the European Medicines Agency on 8 December 2023 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 22 January 2024.

Supplementary information was provided by the applicant on 1 July 2024. 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 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 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

Not applicable

2. Paediatric investigation plan

2.1. Condition:

Treatment of haemophilia A

2.1.1. Indication(s) targeted by the PIP

Treatment of haemophilia A

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	Description
Quality-related studies	Study 1 Development of a suitable administration device for the paediatric population less than 12 years of age. Study 2 Development of one or more autoinjector(s) for the paediatric population from 12 years to less than 18 years of age.
Non-clinical studies	Not applicable
Clinical studies	Study 3 Open-label, non-randomized, dose-finding study to evaluate safety, tolerability, pharmacokinetics, pharmacodynamics, immunogenicity, and efficacy of multiple doses of NXT007 in paediatric patients (from 2 years to less than 12 years of age) with severe or moderate haemophilia A with or without FVIII inhibitors. Study 4 Open-label, randomized, multicentre, global study to evaluate the efficacy, safety, and pharmacokinetics of NXT007 prophylaxis versus FVIII prophylaxis, in adolescent

	patients (at least 12 years of age and weighing at least 40 kg) (and adults) with severe or moderate congenital haemophilia A without FVIII inhibitors (emicizumab-naïve). Study 5 Open-label, randomized, multicentre global study to evaluate the bleed rate with NXT007 prophylaxis compared with emicizumab prophylaxis in adolescent patients (at least 12 years of age and weighing at least 40 kg) (and adults) with severe or moderate congenital haemophilia A without inhibitors, or congenital haemophilia A of any severity with persistent inhibitors, whether or not already treated with emicizumab. Study 6 Open-label, multicentre, single-arm study to evaluate the efficacy, safety, and pharmacokinetics of NXT007 prophylaxis in paediatric patients (less than 12 years of age, and from 12 years to less than 18 years of age who weigh less than 40 kg) with severe or moderate congenital haemophilia A without FVIII inhibitors, or congenital haemophilia A of any severity with persistent inhibitors (emicizumab-naïve and treated).
Modelling and simulation analyses	Study 7 Analysis of existing NXT007 data from healthy subjects, adolescent and adult patients with haemophilia A to select the dose(s) / dosing regimen(s) to be tested in adolescents in Study 4 and Study 5. Study 8 Analysis of existing NXT007 data from adolescents and adults, and use of a literature maturation PK model of emicizumab, to describe exposure in children with haemophilia A less than 12 years of age to derive the dosing regimen(s) to be tested in Study 3 and, subsequently, in Study 6.
Other studies	Not applicable
Extrapolation plan	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 September 2029

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:

The product is not authorised anywhere in the European Community.