EMA/658950/2019

European Medicines Agency decision

P/0014/2020

of 6 January 2020

on the acceptance of a modification of an agreed paediatric investigation plan for Human Cell Line recombinant human Factor VIII (human-cl rhFVIII) / Human Coagulation Factor VIII (rDNA) (Nuwiq and associated names), (EMEA-001024-PIP01-10-M02) 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 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 European Medicines Agency's decision P/92/2011 issued on 8 April 2011 and the decision P/0214/2012 issued on 28 September 2012,

Having regard to the application submitted by Octapharma Pharmazeutika Produktionsges.m.b.H on 6 August 2019 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 15 November 2019, 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.
[2] OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

Article 1

Changes to the agreed paediatric investigation plan for Human Cell Line recombinant human Factor VIII (human-cl rhFVIII) / Human Coagulation Factor VIII (rDNA) (Nuwiq and associated names), powder and solvent for solution for injection, intravenous use, 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 Octapharma Pharmazeutika Produktionsges.m.b.H, Oberlaaer Straße 235, 1100 – Vienna, Austria.

EMA/PDCO/479978/2019
Amsterdam, 15 November 2019

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

EMEA-001024-PIP01-10-M02

Scope of the application

Active substance(s):

Human Cell Line recombinant human Factor VIII (human-cl rhFVIII) / Human Coagulation Factor VIII (rDNA)

Invented name:

Nuwiq and associated names

Condition(s):

Treatment of haemophilia A (congenital Factor VIII deficiency)

Authorised indication(s):

See Annex II

Pharmaceutical form(s):

Powder and solvent for solution for injection

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Octapharma Pharmazeutika Produktionsges.m.b.H

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Octapharma Pharmazeutika Produktionsges.m.b.H submitted to the European Medicines Agency on 6 August 2019 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/92/2011 issued on 8 April 2011 and the decision P/0214/2012 issued on 28 September 2012.

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

The procedure started on 17 September 2019.

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 Norwegian Paediatric Committee member 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

Not applicable.

2. Paediatric Investigation Plan

2.1. Condition

Treatment of haemophilia A (congenital Factor VIII deficiency)

2.1.1. Indication(s) targeted by the PIP

Treatment and prophylaxis of bleeding in patients with 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)

Powder and solvent for solution for injection

2.1.4. Measures

Area	Number of studies	Description
Quality-related studies	0	Not applicable.
Non-clinical studies	0	Not applicable.
Clinical studies	4	**Study 1** Prospective, randomised, active-controlled, open-label, cross-over, multi-centre study in previously treated adult/adolescent patients at least 12 years of age. (GENA-01) **Study 2** Prospective, open-label, multi-centre study in previously treated patients from 2 to 12 years of age. (GENA-03) **Study 3** Prospective, open-label, multi-centre study in previously untreated patients (PUPs). (GENA-05) **Study 4** Prospective, open-label, multi-centre study in previously treated adult/adolescent patients at least 12 years of age. (GENA-08)

Area	Number of studies	Description
Extrapolation, modelling and simulation studies	0	Not applicable.
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:	Yes
Date of completion of the paediatric investigation plan:	By March 2020
Deferral for one or more studies contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment of haemophilia A (congenital Factor VIII deficiency)

Authorised indication(s):

- Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII)

Authorised pharmaceutical form(s):

Powder and solvent for solution for injection.

Authorised route(s) of administration:

Intravenous