EMA/427607/2021

European Medicines Agency decision

P/0302/2021

of 11 August 2021

on the acceptance of a modification of an agreed paediatric investigation plan for rurioctocog alfa pegol (ADYNOVI) (EMEA-001296-PIP01-12-M04) 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/0072/2013 issued on 26 March 2013, the decision P/0139/2014 issued on 11 June 2014, the decision P/0208/2015 issued on 11 September 2015 and the decision P/0001/2016 issued on 8 January 2016,

Having regard to the application submitted by Baxalta Innovations GmbH on 19 March 2021 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 25 June 2021, 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 rurioctocog alfa pegol (ADYNOVI), 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 Baxalta Innovations GmbH, Industriestrasse 67, 1221 - Vienna, Austria.

EMA/PDCO/212983/2021
Amsterdam, 25 June 2021

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

EMEA-001296-PIP01-12-M04

Scope of the application

Active substance(s):

Rurioctocog alfa pegol

Invented name:

ADYNOVI

Condition(s):

Treatment and prophylaxis of bleeding in patients with 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:

Baxalta Innovations GmbH

Information about the authorised medicinal product:

See Annex II

Basis for opinion

Pursuant to Article 22 of Regulation (EC) No 1901/2006 as amended, Baxalta Innovations GmbH submitted to the European Medicines Agency on 19 March 2021 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/0072/2013 issued on 26 March 2013, the decision P/0139/2014 issued on 11 June 2014, the decision P/0208/2015 issued on 11 September 2015 and the decision P/0001/2016 issued on 8 January 2016.

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

The procedure started on 27 April 2021.

Scope of the modification

Some measures and 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 congenital factor VIII deficiency

2.1.1. Indication(s) targeted by the PIP

Treatment and prophylaxis of bleeding in patients with congenital factor VIII deficiency (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	5	**Study 1:** Randomised, open-label, 2-arm study in adults and adolescents previously treated male patients (PTPs) with severe haemophilia A to evaluate efficacy, safety, and pharmacokinetic (PK) parameters of pegylated recombinant FVIII (BAX 855) for prophylaxis and treatment of bleeding. **Study 2:** Open-label, single-arm study to evaluate the efficacy and safety of BAX 855 in adults and paediatric male previously treated patients (PTPs) with severe haemophilia A undergoing elective major or minor emergency surgical, dental or other invasive procedures. **Study 3:**

		Open label, single-arm study in paediatric PTPs less than 12 years of age with severe haemophilia A to evaluate safety, immunogenicity, efficacy and PK parameters of BAX 855 for prophylaxis of bleeding. **Study 4:** Open label, randomised, single-arm study to evaluate safety including immunogenicity and efficacy of BAX 855 in previously untreated patients (PUPs) below 6 years of age with severe haemophilia A. **Study 5:** Prospective, open label, study to further evaluate safety, including long-term safety and efficacy of BAX 855 for prophylactic use including paediatric and adult PTPs with severe haemophilia A from other BAX 855 studies and BAX 855-naïve patients.

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 October 2024
Deferral for one or more measures contained in the paediatric investigation plan:	Yes

Annex II

Information about the authorised medicinal product

Condition(s) and authorised indication(s):

1. Treatment and prophylaxis of bleeding in patients with haemophilia A (congenital factor VIII deficiency)

Authorised indication(s):

- Treatment and prophylaxis of bleeding in patients 12 years and above with haemophilia A (congenital factor VIII deficiency).

Authorised pharmaceutical form(s):

Powder and solvent for solution for injection

Authorised route(s) of administration:

Intravenous use