

EMA/8664240/2015 **Corr**

European Medicines Agency decision

P/0021/2016

of 29 January 2016

on the acceptance of a modification of an agreed paediatric investigation plan for nonacog gamma (Rixubis), (EMEA-001139-PIP01-11-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 726/2004¹,

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 Agency²,

Having regard to the European Medicines Agency's decision P/293/2011 issued on 8 December 2011, and the decision P/0159/2012 issued on 25 July 2012,

Having regard to the application submitted by Baxalta Innovations GmbH on 21 September 2015 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 11 December 2015, 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 nonacog gamma (Rixubis), 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, A-1221 – Vienna, Austria.

Done at London, 29 January 2016

For the European Medicines Agency
Zaïde Frias
Head of Division
Human Medicines Research and Development Support
(Signature on file)



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/PDCO/644337/2015 **Corr**

London, 11 December 2015

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

EMA-001139-PIP01-11-M02

Scope of the application

Active substance(s):

Nonacog gamma

Invented name:

Rixubis

Condition(s):

Treatment of haemophilia B (congenital factor IX 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 21 September 2015 an application for modification of the agreed paediatric investigation plan with a deferral as set out in the European Medicines Agency's decision P/293/2011 issued on 8 December 2011, and the decision P/0159/2012 issued on 25 July 2012.

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

The procedure started on 13 October 2015.

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 Haemophilia B (congenital factor IX deficiency)

2.1.1. Indication(s) targeted by the PIP

Treatment and prophylaxis of bleeding in patients with haemophilia B (congenital factor IX 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 and solvent for solution for injection.

2.1.4. Studies

Area	Number of studies	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	4	Study 1: Prospective study in previously treated patients (PTPs) with severe or moderately severe haemophilia B to assess PK, efficacy, immunogenicity, and safety of nonacog gamma for the prophylaxis and on-demand treatment Study 2: Prospective, open-label, extension study of nonacog gamma in PTPs with severe or moderately severe haemophilia B who have completed Baxter clinical study protocols 250901 or 251101. Study 3: Prospective, open-label, uncontrolled study of nonacog gamma in PTPs with severe or moderately severe haemophilia B undergoing minor and major emergency and elective surgical, dental, or other invasive procedures. Study 4: Prospective, uncontrolled study to assess PK, efficacy, safety, and immunogenicity of nonacog gamma in paediatric subjects with severe or moderately severe haemophilia B.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety and efficacy issues in relation to paediatric use:	Yes.
Date of completion of the paediatric investigation plan:	By June 2017.
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 B (congenital factor IX deficiency)

Authorised indication(s):

- Treatment and prophylaxis of bleeding in patients with haemophilia B (congenital factor IX deficiency).

Authorised pharmaceutical form(s):

Powder and solvent for solution for injection

Authorised route(s) of administration:

Intravenous use