EMA/585777/2016

European Medicines Agency decision

P/0250/2016

of 9 September 2016

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for bivalent anti-human myostatin adnectin recombinant human IgG1-Fc fusion protein (BMS-986089) (EMEA-001793-PIP01-15) 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.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Contact EMA www.ema.europa.eu/contact

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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 application submitted by Bristol-Myers Squibb International Corporation on 22/10/2015 under Article 16(1) of Regulation (EC) No 1901/2006 also requesting a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 19 August 2016, in accordance with Article 18 of Regulation (EC) No 1901/2006 and Article 21 of said Regulation and Article 13 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 and on the granting of a waiver.

(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.

(4) It is therefore appropriate to adopt a decision granting a waiver.

[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 bivalent anti-human myostatin adnectin recombinant human IgG1-Fc fusion protein (BMS-986089), 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 bivalent anti-human myostatin adnectin recombinant human IgG1-Fc fusion protein (BMS-986089), 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

A waiver for bivalent anti-human myostatin adnectin recombinant human IgG1-Fc fusion protein (BMS-986089), 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 4

This decision is addressed to Bristol-Myers Squibb International Corporation, Parc de l'Alliance, Avenue de Finlande 4, 1420 - Braine-l'Alleud, Belgium.

EMA/PDCO/734815/2015
London, 19 August 2016

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

EMEA-001793-PIP01-15

Scope of the application

Active substance(s):

Bivalent anti-human myostatin adnectin recombinant human IgG1-Fc fusion protein (BMS-986089)

Condition(s):

Treatment of Duchenne Muscular Dystrophy

Pharmaceutical form(s):

Solution for injection

Route(s) of administration:

Subcutaneous use

Name/corporate name of the PIP applicant:

Bristol-Myers Squibb International Corporation

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Bristol-Myers Squibb International Corporation submitted for agreement to the European Medicines Agency on 22 October 2015 an application for a paediatric investigation plan for the above mentioned medicinal product and a deferral under Article 20 of said Regulation and a waiver under Article 13 of said Regulation.

The procedure started on 30 November 2015.

Supplementary information was provided by the applicant on 27 May 2016. 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 18 of said Regulation;
   - to grant a deferral in accordance with Article 21 of said Regulation;
   - to grant a waiver for one or more subsets of the paediatric population in accordance with Article 13 of said Regulation and concluded in accordance with Article 11(1)(c) of said Regulation, on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments for paediatric patients.

   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 and the subset(s) of the paediatric population and condition(s) covered by the waiver 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

1.1. Condition:

Treatment of Duchenne Muscular Dystrophy

The waiver applies to:

- the paediatric population from birth to less than 2 years;
- solution for injection, subcutaneous use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit over existing treatments.

2. Paediatric investigation plan

2.1. Condition:

Treatment of Duchenne Muscular Dystrophy

2.1.1. Indication(s) targeted by the PIP

Treatment of Duchenne Muscular Dystrophy

2.1.2. Subset(s) of the paediatric population concerned by the paediatric development

From 2 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 an age-appropriate dosage form
Non-clinical studies	1	Study 2: Juvenile toxicity study in rats (DN14071)
Clinical studies	4	Study 3: Double-blind, randomised, multiple ascending dose study with an open-label phase to assess the safety, tolerability, pharmacokinetics (PK), free-myostatin suppression and immunogenicity of multiple subcutaneous doses of BMS-986089 in ambulatory male children from 5 to less than 11 years of age with Duchenne Muscular Dystrophy (CN001006).

		Study 4: Randomised, double blind, placebo-controlled study to assess the efficacy, safety and tolerability of BMS-986089 in ambulatory male children from 5 to less than 11 years of age with Duchenne Muscular Dystrophy (CN001P14). Study 5: Randomised, double blind, placebo-controlled study to assess the efficacy, safety and tolerability of BMS-986089 in non-ambulatory male children from 11 to less than 18 years of age (and adults) with Duchenne Muscular Dystrophy (CN001P48). Study 6: Randomised, double blind, placebo-controlled study to assess the efficacy, safety and tolerability of BMS-986089 in male children from 2 to less than 5 years of age with Duchenne Muscular Dystrophy (CN001P46).
Extrapolation, modelling and simulation studies	1	Study 7: Dose finding modelling and simulation study.
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:	No
Date of completion of the paediatric investigation plan:	By April 2021
Deferral for one or more measures contained in the paediatric investigation plan:	Yes