

EMA/460045/2017

## European Medicines Agency decision

P/0224/2017

of 11 August 2017

on the acceptance of a modification of an agreed paediatric investigation plan for bivalent anti-human myostatin adnectin recombinant human IgG1-Fc fusion protein (BMS-986089) (EMA-001793-PIP01-15-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<sup>1</sup>,

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<sup>2</sup>,

Having regard to the European Medicines Agency's decision P/0250/2016 issued on 9 September 2016 and the decision P/0084/2017 issued on 16 March 2017,

Having regard to the application submitted by Bristol-Myers Squibb International Corporation on 30 March 2017 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a deferral and a waiver,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 23 June 2017, 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.
- (2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

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<sup>1</sup> OJ L 378, 27.12.2006, p.1.

<sup>2</sup> OJ L 136, 30.4.2004, p. 1.

Has adopted this decision:

**Article 1**

Changes to the agreed paediatric investigation plan for bivalent anti-human myostatin adnectin recombinant human IgG1-Fc fusion protein (BMS-986089), solution for injection, subcutaneous use, 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 Roche Registration Ltd, 6 Falcon Way, Shire Park, AL7 1TW - Welwyn Garden City, United Kingdom.

EMA/PDCO/226622/2017

London, 23 June 2017

## Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan EMA-001793-PIP01-15-M02

### 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 22 of Regulation (EC) No 1901/2006 as amended, Bristol-Myers Squibb International Corporation submitted to the European Medicines Agency on 30 March 2017 an application for modification of the agreed paediatric investigation plan with a deferral and a waiver as set out in the European Medicines Agency's decision P/0250/2016 issued on 9 September 2016 and the decision P/0084/2017 issued on 16 March 2017.

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

The procedure started on 25 April 2017.

### Scope of the modification

Some measures 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 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 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                  | <b>Study 1</b><br>Development of an age-appropriate dosage form.                                                                                                                                                                                                                                                                                                      |
| Non-clinical studies    | 1                  | <b>Study 2</b><br>Juvenile toxicity study in rats (DN14071).                                                                                                                                                                                                                                                                                                          |
| Clinical studies        | 4                  | <b>Study 3</b><br>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). |

|                                                 |   |                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                        |
|-------------------------------------------------|---|----------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|
|                                                 |   | <p><b>Study 4</b></p> <p>Randomised, double blind, placebo-controlled study to assess the efficacy, safety and tolerability of BMS-986089 in ambulatory male children from 6 to less than 12 years of age with Duchenne Muscular Dystrophy (CN001016).</p> <p><b>Study 5</b></p> <p>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 (CN001019).</p> <p><b>Study 6</b></p> <p>Randomised, double blind, placebo-controlled study to assess the efficacy, safety and tolerability of BMS-986089 in male children from 2 to less than 6 years of age with Duchenne Muscular Dystrophy (CN001P46).</p> |
| Extrapolation, modelling and simulation studies | 1 | <p><b>Study 7</b></p> <p>Dose finding modelling and simulation study.</p>                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                                              |
| 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           |