EMA/429450/2012

European Medicines Agency decision

P/0131/2012

of 4 July 2012

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for ponatinib (EMEA-001186-PIP01-11) 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.

7 Westferry Circus • Canary Wharf • London E14 4HB • United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8670
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

European Medicines Agency decision

P/0131/2012

of 4 July 2012

on the agreement of a paediatric investigation plan and on the granting of a deferral and on the granting of a waiver for ponatinib (EMEA-001186-PIP01-11) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council

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 ARIAD Pharma, Ltd. on 8 August 2011 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 16 May 2012, 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 ponatinib, coated tablet, capsule, hard, age-appropriate formulation, oral 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 ponatinib, coated tablet, capsule, hard, age-appropriate formulation, oral 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 ponatinib, coated tablet, capsule, hard, age-appropriate formulation, oral 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 ARIAD Pharma, Ltd., 1 Northumberland Avenue, London WC2N 5BW, United Kingdom.

Done at London, 4 July 2012

For the European Medicines Agency
Guido Rasi
Executive Director
(Signature on file)

EMA/PDCO/242649/2012

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

EMEA-001186-PIP01-11

Scope of the application

Active substance(s):

Ponatinib

Condition(s):

Treatment of chronic myeloid leukaemia

Treatment of acute lymphoblastic leukaemia

Pharmaceutical form(s):

Coated tablet

Capsule, hard

Age-appropriate formulation

Route(s) of administration:

Oral use

Name/corporate name of the PIP applicant:

ARIAD Pharma, Ltd.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, ARIAD Pharma, Ltd. submitted for agreement to the European Medicines Agency on 8 August 2011 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 14 September 2011.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8670
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

Supplementary information was provided by the applicant on 27 February 2012. The applicant proposed modifications to the paediatric investigation plan.

A meeting with the Paediatric Committee took place on 14 May 2012.

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)(b) of said Regulation, on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified subset(s) of the paediatric population and 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(es) and appendix.

London, 16 May 2012

On behalf of the Paediatric Committee
Dr Daniel Brasseur, Chairman
(Signature on file)

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

1. Waiver

1.1. Condition: Treatment of chronic myeloid leukaemia

The waiver applies to:

- Children from birth to less than 1 year of age;
- for coated tablet, capsule, hard, and age-appropriate formulation, for oral use;
- on the grounds that the disease or condition for which the specific medicinal product is intended does not occur in the specified paediatric subset(s).

1.2. Condition: Treatment of acute lymphoblastic leukaemia

The waiver applies to:

- Children from birth to less than 1 year of age;
- for coated tablet and capsule, hard, and age-appropriate formulation, for oral use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as the needs are already covered.

2. Paediatric Investigation Plan

2.1. Condition: Treatment of chronic myeloid leukaemia

2.1.1. Indication(s) targeted by the PIP

For the treatment of children with chronic (CP), accelerated (AP), or blast phase (BP) CML who are resistant or intolerant to prior tyrosine kinase inhibitor (TKI) therapy.

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

From 1 year to less than 18 years of age.

2.1.3. Pharmaceutical form(s)

Coated tablet.

Capsule, hard.

Age-appropriate formulation.

2.1.4. Studies

Area	Number of studies	
Quality	1	Study 1: Development of an age-appropriate formulation for oral use.
Non-clinical	1	Study 2: Toxicity study in juvenile rats.
Clinical	1	Study 3: Open-label, single-agent, dose-escalation, multi-centre trial to investigate tolerability, safety and activity of ponatinib in children from 1 year to less than 18 years of age with malignant disease for which no effective treatment is known, and with an expansion cohort of children with chronic phase chronic myeloid leukaemia.

2.2. Condition: Treatment of acute lymphoblastic leukaemia

2.2.1. Indication(s) targeted by the PIP

For the treatment of children with Ph+ ALL who are resistant or intolerant to prior TKI therapy.

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

From 1 year to less than 18 years of age.

2.2.3. Pharmaceutical form(s)

Coated tablet.

Capsule, hard.

Age-appropriate formulation.

2.2.4. Studies

Area	Number of studies	
Quality	1	Study 1: As for condition "Treatment of chronic myeloid leukaemia".
Non-clinical	1	Study 2: As for condition "Treatment of chronic myeloid leukaemia".
Clinical	2	Study 3: As for condition "Treatment of chronic myeloid leukaemia". Study 4: Randomised, multi-centre, dose-comparative, double-blind trial to investigate the safety and, activity and efficacy of ponatinib as add-on to standard therapy in children from 1 year to less than 18 years of age with relapsed or refractory BCR-ABL translocation-positive ALL.

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 December 2020
Deferral for one or more studies contained in the paediatric investigation plan:	Yes