EMA/587322/2012

European Medicines Agency decision

P/0237/2012

of 22 October 2012

on the agreement of a paediatric investigation plan and on the refusal of a deferral and on the granting of a waiver for human heterologous liver cells (EMEA-000067-PIP02-11) 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 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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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 Cytonet GmbH&Co. KG on 10 May 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 7 September 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 refusal 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 refusing 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 human heterologous liver cells, suspension for infusion, intravenous 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 human heterologous liver cells, suspension for infusion, intravenous 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 refused.

Article 3

A waiver for human heterologous liver cells, suspension for infusion, intravenous 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 Cytonet GmbH&Co. KG, Albert-Ludwig-Grimm Str. 20, 69469 – Weinheim, Germany.

Done at London, 22 October 2012

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

EMA/PDCO/449556/2012 Corr

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

EMEA-000067-PIP02-11

Scope of the application

Active substance(s):

Human heterologous liver cells

Condition(s):

Treatment of urea cycle disorders

Pharmaceutical form(s):

Suspension for infusion

Route(s) of administration:

Intravenous use

Name/corporate name of the PIP applicant:

Cytonet GmbH&Co. KG

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Cytonet GmbH&Co. KG submitted for agreement to the European Medicines Agency on 10 May 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 15 June 2011.

Supplementary information was provided by the applicant on 18 June 2012. The applicant proposed modifications to the paediatric investigation plan.

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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 refuse 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 Icelandic and the Norwegian Paediatric Committee members agree 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, 7 September 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 urea cycle disorders

The waiver applies to:

- The paediatric population from 6 to less than 18 years of age;
- for suspension for infusion for intravenous 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 urea cycle disorders

2.1.1. Indication(s) targeted by the PIP

Treatment of patients with urea cycle disorders.

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

From 0 to less than 6 years of age.

2.1.3. Pharmaceutical form(s)

Suspension for infusion

2.1.4. Studies

Area	Number of studies	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	2	**Study 1** Open-label, uncontrolled, multicentre, multiple dose trial to evaluate safety and efficacy of human heterologous liver cells in children from birth to less than 6 years of age with urea cycle disorders. **Study 2** Open-label, historical controlled, group-matched, multicentre, multiple dose trial to evaluate safety and efficacy of human heterologous liver cells in children from birth to less than 6 years of age with urea cycle disorders.

3. Follow-up, completion and deferral of PIP

Concerns on potential long term safety issues in relation to paediatric use:	Yes
Date of completion of the paediatric investigation plan:	By August 2014
Deferral for one or more studies contained in the paediatric investigation plan:	No

3.1. Refusal of deferral

The PDCO denied a deferral on the grounds that

The deferred study CCD06 was considered redundant. As the study will not be a subject of MAA the Paediatric Committee concluded it should be excluded from the PIP. Therefore, the deferral is no more applicable.