EMA/496850/2013

European Medicines Agency decision

P/0250/2013

of 21 October 2013

on the acceptance of a modification of an agreed paediatric investigation plan for human heterologous liver cells (EMEA-000067-PIP02-11-M01) 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

European Medicines Agency decision

P/0250/2013

of 21 October 2013

on the acceptance of a modification of an agreed paediatric investigation plan for human heterologous liver cells (EMEA-000067-PIP02-11-M01) 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 European Medicines Agency's decision P/0237/2012 issued on 22 October 2012,

Having regard to the application submitted by Cytonet GmbH & Co. KG on 20 May 2013 under Article 22 of Regulation (EC) No 1901/2006 proposing changes to the agreed paediatric investigation plan with a waiver and proposing a deferral,

Having regard to the opinion of the Paediatric Committee of the European Medicines Agency, issued on 11 October 2013, in accordance with Article 22 of Regulation (EC) No 1901/2006, and Article 21 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, following a re-examination procedure of the Paediatric Committee's opinion according to Article 25(3) Regulation (EC) No 1901/2006, an opinion on the acceptance of changes to the agreed paediatric investigation plan and on the granting of a deferral.

(2) It is therefore appropriate to adopt a decision on the acceptance of changes to the agreed paediatric investigation plan.

(3) It is therefore appropriate to adopt a decision on the granting of a 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 human heterologous liver cells, suspension for infusion, intravenous 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

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 the European Medicines Agency annexed hereto, together with its appendices, is hereby granted.

Article 3

This decision is addressed to Cytonet GmbH&Co. KG, Albert-Ludwig-Grimm Str. 20, 69469 – Weinheim, Germany.

Done at London, 21 October 2013

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

EMA/PDCO/565584/2013

Opinion of the Paediatric Committee on the acceptance of a modification of an agreed Paediatric Investigation Plan (after re-examination)

EMEA-000067-PIP02-11-M01

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 22 of Regulation (EC) No 1901/2006 as amended, Cytonet GmbH & Co. KG submitted to the European Medicines Agency on 20 May 2013 an application for modification of the agreed paediatric investigation plan with a waiver as set out in the European Medicines Agency's decision P/0237/2012 issued on 22 October 2012. The application for modification proposed changes to the agreed paediatric investigation plan and proposed a deferral.

An Opinion was adopted by the Paediatric Committee on 9 August 2013 for the above mentioned product. Cytonet GmbH & Co. KG received the Paediatric Committee Opinion on 15 August 2013.

On 11 September 2013 Cytonet GmbH & Co. KG submitted to the European Medicines Agency a written request including detailed grounds for a re-examination of the Opinion.

The re-examination procedure started on 12 September 2013.

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

Scope of the modification

Some measures and timelines of the Paediatric Investigation Plan have been modified. A deferral for the clinical measures has been granted.

Final Opinion

1. The Paediatric Committee, having assessed the detailed grounds for re-examination, in accordance with Article 25(3) of Regulation (EC) No 1901/2006 as amended, recommends as set out in the appended summary report:

1.1. to revise its opinion and

- to agree to the changes regarding the measures and the timelines of the paediatric investigation plan in the scope set out in the Annex I of this opinion;
- to grant a deferral;

1.2. following re-examination, to amend the scope of the modifications of the paediatric investigation plan and the deferral.

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.

London, 11 October 2013

On behalf of the Paediatric Committee
Dr Dirk Mentzer, 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. Measures

Area	Number of measures	Description
Quality	0	Not applicable.
Non-clinical	0	Not applicable.
Clinical	2	**Measure 1** Open-label, historical controlled, 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. **Measure 2** Open-label, historical controlled, multicentre, multiple dose trial to evaluate efficacy and safety 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 December 2014
Deferral for one or more measures contained in the paediatric investigation plan:	Yes