European Medicines Agency

Doc. Ref. EMA/63059/2010
P/20/2010

EUROPEAN MEDICINES AGENCY DECISION

of 19 February 2010

on the agreement of a Paediatric Investigation Plan and on the granting of a deferral and on the granting of a waiver for expanded human autologous mesenchymal adult stem cells extracted from adipose tissue (CX-401) (EMEA-000635-PIP01-09) in accordance with Regulation (EC) No 1901/2006 of the European Parliament and of the Council as amended

(ONLY THE ENGLISH TEXT IS AUTHENTIC)

DISCLAIMER: This Decision does not entitle to the rewards and incentives referred to in Title V of Regulation (EC) No 1901/2006, as amended.

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THE EUROPEAN MEDICINES AGENCY,

Having regard to the Treaty establishing the European Community,

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 as amended 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 Cellerix, S.A. on 19 June 2009 under Article 16(1) of Regulation (EC) No 1901/2006 as amended also requesting a waiver under Article 13 of said Regulation and a deferral under Article 20 of said Regulation,

Having regard to the Opinion of the Paediatric Committee of the European Medicines Agency, issued on 15 January 2010, in accordance with Article 18 of Regulation (EC) No 1901/2006 as amended, and Article 13 of said Regulation and Article 21 of said Regulation**,**

Having regard to Article 25 of Regulation (EC) No 1901/2006 as amended,

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 expanded human autologous mesenchymal adult stem cells extracted from adipose tissue (CX-401), suspension for injection, intralesional 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 expanded human autologous mesenchymal adult stem cells extracted from adipose tissue (CX-401), suspension for injection, intralesional 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 expanded human autologous mesenchymal adult stem cells extracted from adipose tissue (CX-401), suspension for injection, intralesional 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 Cellerix, S.A., Calle Marconi n1, Parque Tecnológico de Madrid, Tres Cantos, 28760 Madrid, Spain.

Done at London, 19 February 2010

For the European Medicines Agency
Thomas Lönngren
Executive Director

(Signature on file)

European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

Doc. Ref. EMA/PDCO/841841/2009
EMEA-000635-PIP01-09

OPINION OF THE PAEDIATRIC COMMITTEE ON THE AGREEMENT OF A PAEDIATRIC INVESTIGATION PLAN AND A DEFERRAL AND A WAIVER

Scope of the application

Active substance(s):
Expanded human autologous mesenchymal adult stem cells extracted from adipose tissue (CX-401)

Condition(s):
Anal fistula of cryptoglandular aetiology (Non-Crohn)
Anal fistula of Crohn´s disease associated aetiology
Non-inflammatory anal fistula

Pharmaceutical form(s):
Suspension for injection

Route(s) of administration:
Intralesional use

Name/corporate name of the PIP applicant:
Cellerix, S.A.

Basis for opinion

Pursuant to Article 16(1) of Regulation (EC) No 1901/2006 as amended, Cellerix, S.A. submitted for agreement to the EMEA on 19 June 2009 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 23 July 2009.

Supplementary information was provided by the applicant on 3 November 2009.

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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 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)(a) of said Regulation, on the grounds that the specific medicinal product is likely to be ineffective or unsafe in part or all of the paediatric population and 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 Agency, together with its annex(es) and appendix.

London, 15 January 2010

On behalf of the Paediatric Committee
Dr Daniel Brasseur, Chairman

(Signature on file)

ANNEX I

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

A. CONDITION(S)

1) Anal fistula of cryptoglandular aetiology (Non-Crohn)
2) Anal fistula of Crohn´s disease associated aetiology
3) Non-inflammatory anal fistula

B. WAIVER

- **Condition**

1) Anal fistula of cryptoglandular aetiology (Non-Crohn)
2) Anal fistula of Crohn´s disease associated aetiology

The waiver applies to
- All subsets of the paediatric population from birth to less than 4 years of age;
- for suspension for injection for intralesional use;
- on the grounds that the specific medicinal product does not represent a significant therapeutic benefit as clinical studies(s) are not feasible.

- **Condition**

3) Non-inflammatory anal fistula

The waiver applies to
- All subsets of the paediatric population;
- for suspension for injection for intralesional use;
- on the grounds that the specific medicinal product is likely to be ineffective.

C. PAEDIATRIC INVESTIGATION PLAN

- **Condition to be investigated**

1) Anal fistula of cryptoglandular aetiology (Non-Crohn)
2) Anal fistula of Crohn´s disease associated aetiology

- **Proposed PIP indication**

- **Subset(s) of the paediatric population concerned by the paediatric development**

  From 4 years to less than 18 years of age.

- **Formulation(s)**

  Suspension for injection for intralesional use

- **Studies**

Area	Number of studies	Description
Quality		Not applicable.
Non-clinical	1	Biodistribution study in rats.
Clinical	1	Open-label, long-term trial to assess the activity and safety of autologous expanded mesenchymal stem cells extracted from adipose tissue (CX-401) for treatment of complex anal fistula in paediatric patients with and without Crohn´s disease

Measures to address long term follow-up of potential safety issues in relation to paediatric use:	**Yes**
Date of completion of the paediatric investigation plan:	**By December 2020**
Deferral for some or all studies contained in the paediatric investigation plan:	**Yes**