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Patient Health Protection

Scientific recommendation on classification of advanced therapy medicinal products

Article 17 – Regulation (EC) No 1394/2007

Disclaimer: This document is a summary for public release of a scientific recommendation on classification of advanced therapy medicinal products. The original text adopted by the Committee for Advanced Therapies (CAT) has been redacted to delete commercially confidential information.

The present scientific recommendation refers exclusively to the case as presented to the European Medicines Agency (EMA) without prejudice to future evaluations by the Agency.

This scientific recommendation is not binding and is without prejudice to any decision taken by Member State competent authorities on matters falling within their own remits.

Short descriptor (or name when available) of the proposed active substance

Bone marrow-derived autologous CD34+ cells

Brief description of the proposed finished product

Suspension of non-substantially manipulated autologous CD34+ cells formulated in solution for intramyocardial delivery.

Proposed indication

Intended for improvement of heart function in patients with refractory angina and chronic myocardial ischemia.

EMA/CAT comment

Consideration of Article 1(2) of Directive 2001/83/EC (definition of medicinal product – see Annex A)

- The product consists of autologous bone marrow-derived CD34+ cells which can be considered a 'substance' in the meaning of the pharmaceutical legislation (in accordance with article 1(3)

7 Westferry Circus • Canary Wharf • London E14 4HB • United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7051
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

of Directive 2001/83/EC), administered to humans with a view to restoring physiological function(s) by exerting a metabolic and/or a pharmacological action.

- The product is presented as having properties for treating disease in human being.

- According to Article 1(2), the restoration, correction or modification of the physiological function is to be mediated by the substances that exert "a pharmacological, immunological or metabolic action". As the product consists of bone marrow-derived CD34+ cells, it can be agreed that the product acts via metabolic and/or a pharmacological means.

Fulfilment of Article 2(1) of Regulation (EC) No 1394/2007 (definition of advanced therapy medicinal product – see Annex A)

- The product consists of non-manipulated autologous bone marrow-derived CD34+ cells.

- The cells are considered to be 'engineered' as they are not intended to be used for the same essential function (haematological restoration) in the recipient as in the donor. It is rather assumed that they act by differentiating into endothelial cells, and through auto- and paracrine growth factor/cytokine effects.

- The product is administrated to human beings with a view to neovascularization. This neovascularization can be considered as regenerating, repairing or replacing a human tissue.

Based on the above considerations, it is considered that the product falls within the definition of an advanced therapy medicinal product, a tissue engineered product.

EMA/CAT conclusion

On the basis that:

- the product consists of autologous bone marrow-derived CD34+ cells;
- the bone marrow-derived CD34+ cells are not intended to be used for the same essential function (haematological restoration);
- the product is intended for the treatment of refractory angina via stem cell-induced angiogenesis in ischemic area of heart tissue by non-haematological differentiation of bone marrow-derived mononuclear cells into vascular cells and/or by paracrine effects of the stem cells;

the EMA/CAT considers that the product falls within the definition of a tissue engineered product as provided in Article 2(1)(a) of Regulation (EC) No 1394/2007.