EUROFANCOLEN

Project ID: 305421

Funded under: FP7-HEALTH

Phase I/II Gene Therapy Trial of Fanconi anemia patients with a new Orphan Drug consisting of a lentiviral vector carrying the FANCA gene: A Coordinated International Action

From 2013-01-01 to 2018-12-31, ongoing project

Project details

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<th>Total cost:</th>
<th>Topic(s):</th>
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<td>EUR 7 014 945,20</td>
<td>HEALTH.2012.2.4.4-1 - Preclinical and/or clinical development of substances with a clear potential as orphan drugs</td>
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<tr>
<th>EU contribution:</th>
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<tr>
<td>EUR 5 380 169,75</td>
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<th>Coordinated in:</th>
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<td>Spain</td>
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<th>Call for proposal:</th>
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<tr>
<td>FP7-HEALTH-2012-INNOVATION-1 See other projects for this call</td>
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<th>Funding scheme:</th>
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<td>CP-FP - Small or medium-scale focused research project</td>
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Objective

Fanconi anemia (FA) is a rare inherited syndrome characterized by the early development of bone marrow failure and increasing predisposition to cancer with age. Allogeneic hematopoietic cell transplantation (alloHCT) is the only curative therapy for hematopoietic manifestations of FA, although associated with complications arising from myeloablation, graft versus host disease and increased incidence of squamous cell carcinoma. The genetic correction of autologous hematopoietic stem cells (HSC) with lentiviral vectors constitutes a recent and safe alternative for the treatment of different genetic diseases affecting mature cells from different tissues and/or committed progenitors of the hematopoietic system. One of the key features of FA that make it a unique disease for gene therapy approaches rely on the characteristic proliferation defect that is already evident in the very primitive HSCs. Thus, a marked survival advantage would be expected from corrected HSCs, potentially allowing normalization of hematopoiesis in the absence or after mild conditioning. Difficulties in the collection of sufficient numbers of HSC from FA patients and the use of sub-optimal transduction protocols with gammaretroviral vectors limited the success of FA gene therapy trials conducted 10 years ago in the USA. Our innovative approach to develop for the first time an efficient and safe gene therapy of FA is based on two recent innovations: 1) Discovery of potent HSC mobilizers, such as plerixafor, and 2) Development of a new lentiviral vector by members of this Consortium, designed as Orphan Drug by the EC in December 2010. The principal objective of this Project is, therefore, the development of a multicentric Phase I/II gene therapy trial for FA-A patients, based on the genetic correction of plerixafor+G-CSF mobilized HSCs with the novel lentiviral vector, accompanied by comprehensive and groundbreaking safety and efficacy patient monitoring studies.

Related information

Report Summaries

Periodic Report Summary 1 - EUROFANCOLEN (Phase I/II Gene Therapy Trial of Fanconi anemia patients with a new Orphan Drug consisting of a lentiviral vector carrying the FANCA gene: A Coordinated International Action)

Periodic Report Summary 2 - EUROFANCOLEN (Phase I/II Gene Therapy Trial of Fanconi anemia patients with a new Orphan Drug consisting of a lentiviral vector carrying the FANCA gene: A Coordinated International Action)

Periodic Report Summary 3 - EUROFANCOLEN (Phase I/II Gene Therapy Trial of Fanconi anemia patients with a new Orphan Drug consisting of a lentiviral vector carrying the FANCA gene: A Coordinated International Action)
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**EU contribution:** EUR 1 055 227

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**Subjects**

Scientific Research

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