Cystinosis Research Foundation

*Lay Abstract Template for Awardees*

Spring 2013 Grants

Please complete this lay-oriented grant abstract form which will be published on the CRF web site and in the CRF Star Facts with announcement of your award. Please do not exceed 350 words total. Please submit this form to us as a Word file.

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**Principal Investigator (s)**: Stephanie Cherqui

**Project Title**: Mechanism of bone marrow stem cell-mediated therapy in the mouse model of cystinosis

**Objective/Rationale**: Please write a lay-oriented statement of the scientific rationale for this project. Approximately 75-85 words.

Adult bone marrow stem cells are promising tools for the development of innovative strategies for tissue repair and regenerative medicine. More specifically, hematopoietic stem cells (HSC) are the most attractive stem cells because of their easy access and their clinical use for over 30 years. However, the fate of the HSC after transplantation and the mechanisms by which they could lead to tissue repair is still unknown. The goal of this project is to reveal the mechanism(s) by which Ctns-expressing HSC can significantly improve cystinosis.

**Project Description**: Please write a brief, lay-oriented description of how you will carry out the project. Approximately 125-130 words.

We developed a new mouse model for cystinosis, the DsRed *Ctns-/-* mice expressing the DsRed reporter gene (appear red by microscope analysis) and transplanted with GFP-expressing HSC (appear green by microscope analysis). This model represents a new innovative tool to study bone marrow-derived cell tissue integration because of the abundant stem cell integration occurring in the *Ctns-/-* mice and because of the dual color that allow distinguishing the transplanted cells and the host cells. We are going to perform a phenotypic and genetic characterization of these different cell populations to understand the mechanism by which they lead to tissue cystine decrease and tissue preservation.

**Relevance to the Understanding and/or Treatment of Cystinosis**: Please explain how the project will impact cystinosis treatment or increase our understanding of cystinosis. Approximately 75 words.

This work is important for the future clinical application of stem cell therapy for cystinosis.

**Anticipated Outcome**: Please write a lay-oriented description of what you expect to learn/discover. Approximately 75 words.

We are expecting to reveal new insights in hematopoietic stem cell-mediated regenerative medicine that could extend their use to other diseases.