**NET4CGD**

**Project ID:** 305011  
**Funded under:** FP7-HEALTH

**Gene Therapy for X-linked Chronic Granulomatous Disease (CGD)**

**From** 2012-12-01 **to** 2018-05-31, closed project

### Project details

<table>
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<tr>
<th><strong>Total cost:</strong></th>
<th><strong>Topic(s):</strong></th>
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<td>EUR 8 302 977,60</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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<th><strong>EU contribution:</strong></th>
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<tr>
<td>EUR 5 999 607</td>
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<th><strong>Coordinated in:</strong></th>
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<td>France</td>
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<th><strong>Call for proposal:</strong></th>
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<td>FP7-HEALTH-2012-INNOVATION-1</td>
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<th><strong>Funding scheme:</strong></th>
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<td>CP-FP - Small or medium-scale focused research project</td>
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### Objective

This project is focused on the clinical development of a new orphan drug that can rapidly become a new treatment option for patients with the X-linked form of chronic granulomatous disease (X-CGD). This rare primary immune deficiency of phagocytes is caused by mutations in the gp91phox gene. Affected patients are highly-susceptible to infections and develop inflammatory granulomas. Several members of the Net4CGD consortium have already attempted hematopoietic gene correction of X-CGD using gp91 gammaretroviral gene transfer vectors. While functional correction and clinical benefit was initially achieved, problems arose, linked to insertional mutagenesis, vector silencing and lack of long-term engraftment. Net4CGD proposes future trials to achieve (i) effective transduction of hematopoietic cells, (ii) physiological expression of the transgene and (iii) long-term engraftment of gene modified cells. A new lentiviral vector (LV) was developed to express gp91phox in myeloid cells. Encouraging results obtained in preclinical studies and through the compassionate treatment of a patient, prompt us to test the LV in a multi-center study in several European centers expert in CGD, under the sponsorship of a rare disease specialist. The tasks include i) Manufacturing clinical grade vector to support clinical studies, ii) Conducting a multi-center phase I/II trial in eligible X-CGD patients, with LV gene-modified autologous hematopoietic stem cells to evaluate the safety and efficacy of the procedure iii) Ensuring high-quality and harmonization of products and procedures to facilitate future product registration iv) Obtaining state-of-the art information on biological efficacy and safety in patients by assessing immune restoration and large-scale integrome data. If positive, this study will be used to register the orphan drug. The treatment is expected to improve patients’ quality of life and will reduce the economical burden of CGD. Results should benefit other RD.

### Related information

**Result In Brief**  
A new gene therapy treatment for CGD

**Report Summaries**  
Final Report Summary - NET4CGD (Gene Therapy for X-linked Chronic Granulomatous Disease (CGD))

**News**  
Faster way to test new gene therapies
Coordinator

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