Persisting Transgenesis

From 2009-01-01 to 2013-06-30, closed project

Objective

For many disabling or fatal diseases, there is pre-clinical or clinical evidence of the potential therapeutic efficacy of gene therapy and, yet, the limitations of current gene transfer technologies have prevented success or even caused serious adverse events leading to termination of trials. PERSIST will explore the use of highly innovative gene-modifying and delivery technologies and capitalize on recent discoveries in gene expression control to develop radical solutions to the problem of precisely controlling the fate and expression of exogenous genetic information in gene therapy with applications in these and other deadly diseases. Our proposal combines 20 of Europe’s outstanding experts from 8 countries in the field of genetic engineering for persisting gene expression. Partners have pioneered the use of Zinc Finger Nucleases, engineered recombinases and transposases for gene targeting, synthetic promoters, epigenetic switches and micro-RNA for regulating gene expression, developed state-of-the-art gene delivery platforms based on lentiviral, AAV and gutless adenoviral vectors, conducted front-line clinical trials, and productively collaborated in previous FP projects. PERSIST includes 16 work packages of which 6 focus on vector innovations (part A: emerging technologies), 6 on applications & evaluation (part B), 1 on process development and the remaining 2 on training/dissemination and project management including IPR issues. Targeting deadly diseases, such as inherited immunodeficiencies, storage disorders and haemophilias, PERSIST is in line with the Strategic Research Agenda (SRA) of the Innovative Medicines Initiative (IMI) and will result in: faster discovery and development of better medicines; more attractive professional environment for scientists; better European expertise and know-how to attract biomedical R&D investment in Europe; and a stronger competitive advantage for SMEs, spin-offs and start-ups to enhance Europe’s economy.

Related information

Result In Brief
At the cutting edge of gene surgery

Report Summaries
Final Report Summary - PERSIST (Persisting Transgenesis)

News
Ground-breaking work to improve gene therapy
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Medicine and Health

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