Ground-breaking work to improve gene therapy

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Credit: Ketut Subiyanto from Pexels

For many disabling or fatal diseases, there is pre-clinical or clinical evidence of the potential therapeutic benefits of gene therapy. Unfortunately, the limitations of current gene transfer technologies have

prevented successful trials or even led to serious adverse effects during trials.

The EU-funded project PERSIST ('Persisting transgenesis') was concerned with the development of new gene therapy tools and technologies for clinical application.

Project researchers, led by Milan's Vita-Salute San Raffaele University, explored highly innovative gene targeting, editing and delivery technologies, capitalising on recent discoveries in gene expression control.

Specifically, they worked to solve the problem of precisely directing the fate and expression of genetic information in gene therapy, with applications for severe diseases such as inherited immunodeficiencies, storage disorders and haemophilias.

These diseases represent crucial paradigms for novel therapies that can be explored by the genetic modification of several cell types. These cell types include lymphocytes, hepatocytes, and different types of stem cells, all of which were targeted by PERSIST.

The project was successful on a number of levels. First, partners developed new strategies for long-term transgene expression while lowering the risk of induced immune responses, transgene toxicity and genotoxicity.

PERSIST thus overcomes the uncertainty related to dose-limiting side effects of earlier untargeted types of gene therapy. This work can now be applied towards the definition of safer and more effective treatment protocols for human diseases.

The results of the PERSIST project will contribute to improving

treatment for the specific diseases the researchers studied. The research could also be applied to several other inherited diseases and sets the stage for applications in acquired diseases such as cancer and for infectious diseases.

Thanks to the collaborative nature of the project, strong and lasting research affiliations were created across Europe, and this is likely to result in the more rapid discovery and development of other new and important therapeutic techniques.

PERSIST received EUR 11.2 million in EU funding and brought together high-level research teams in eight EU countries and Switzerland. The researchers completed their work in June 2013.

More information: PERSIST www.persist-project.eu/

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