RNA interference for human therapy

September 20 2012

Credit: Thinkstock

Leading scientists in the field investigated the potential of RNA interference (RNAi) technology as a therapeutic intervention for down-regulating the expression of disease-associated genes. Project deliverables hold significant exploitation ground in research and medicine.

RNAi has emerged as a powerful technology for down-regulating gene expression, culminating in the Nobel prize award for Medicine in 2006. Research has shown that it could be effectively used in a variety of tissues to knock down target genes – especially those involved in disease – thus demonstrating the potential of RNAi in human therapy.

The EU 'RNA interference technology as human therapeutic tool' (RIGHT) initiative aimed to explore the technology of RNAi for therapy by delineating its underlying mechanisms. To achieve that, project partners had to overcome key technological barriers such as undesired interferon response and insufficient delivery, stability and targeting of RNAi inhibitors to the appropriate cells.

Selective reagents were generated for efficient delivery of chemically synthesised or vector-expressed siRNAs to cells and tissues of diseased organisms. Cell biology and disease models were used to assess the function and effectiveness of RNAi for the treatment of selected disorders. In particular, scientists showed the so-far unexplored potential of RNAi against influenza, arthritis, HBV and acute myeloid leukaemia (AML) in pre-clinical models.

Furthermore, the improved understanding of the molecular processes associated with RNAi and the naturally occurring microRNAs (miRNAs) is expected to have applications in healthcare, such as for the therapy of acute myeloid leukemia.

The RIGHT consortium demonstrated the efficacy of the RNAi technology as a therapeutic tool for many diseases. The knowledge generated will not only impact future research in the field but RNAi-based solutions are expected to get translated into clinical practice.

Provided by CORDIS

Citation: RNA interference for human therapy (2012, September 20) retrieved 3 May 2024 from https://medicalxpress.com/news/2012-09-rna-human-therapy.html

This document is subject to copyright. Apart from any fair dealing for the purpose of private study or research, no part may be reproduced without the written permission. The content is

provided for information purposes only.