

RNA Interference (RNAi)

The phenomenon of RNAi was first observed in plants and described as Post Transcriptional Gene Silencing (PTGS). The discovery in animals and the breakthrough in its understanding came from studies with the worm *Caenorhabditis elegans* in 1998 by Andrew Fire and Craig Mello who were awarded the Nobel prize for their discovery in 2006. RNAi is a natural process that occurs in the majority of organisms ranging from fungi and plants to mammals. Within the cell the catalytic process of RNAi is triggered by Small Interfering RNAs (siRNAs) which are short double-stranded RNA molecules. A sequence-specific target mRNA-recognition by the siRNA leads to the cleavage and subsequent degradation of the mRNA and thus prevents the production of the encoded protein.

Potential for new class of therapeutic proteins

In the same manner as natural siRNAs also synthetic siRNAs can trigger RNAi. Shortly after the discovery of RNAi, researchers all over the world heralded RNAi as a revolution in biology and rapidly recognized the usefulness of synthetic siRNAs as a research tool, e.g. for elucidating biological pathways. In particular in the pharmaceutical industry RNAi has been widely used for the identification and validation of new therapeutic targets. Moreover, siRNAs have the potential to become a whole new class of innovative therapeutics.

RNAi Therapeutics Platform

Scientists immediately recognized the potential for a major new therapeutics platform that lies in RNAi. In principle, using RNAi it is possible to silence any given gene in the genome, including as yet "undruggable" target genes. Many diseases are the result of the expression of undesired genes (viral infections), or mutated and overexpressed genes (many oncological diseases). By harnessing RNAi those target genes can be silenced before their corresponding harmful proteins are produced.

Roche and RNAi Technology

On July 9 2007, Roche and Alnylam formed a major alliance in the field of RNAi therapeutics with Alnylam's site in Bavaria, Germany becoming the Center of Excellence for RNAi therapeutics Roche Kulmbach.