Understanding RNAi

What is RNAi?

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Deoxyribonucleic acid (DNA) holds the instructions to make all proteins, which do the work of the cell. To create a protein, DNA is copied to ribonucleic acid (RNA). RNA is the messenger that carries the instructions to assemble proteins. RNA interference (RNAi) is a cellular mechanism that disables specific RNA messages, preventing proteins from being made. RNAi is a naturally occurring process in plants, animals and humans that is involved in immunity and the regulation of gene expression.

How does RNAi work?

Small pieces of RNA, like small interfering RNA (siRNA) or microRNA (miRNA), connect to and activate a group of proteins called the RNA induced silencing complex (RISC). They guide the complex to target and degrade a specific RNA message, or messenger RNA (mRNA). This prevents the corresponding protein from being made.

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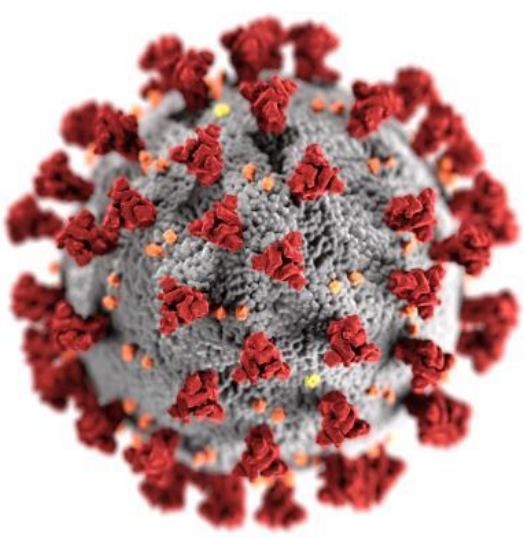
Fighting disease with RNAi

RNAi is a promising tool for disease intervention. Scientists have learned how to manipulate this naturally occurring process, designing synthetic interfering RNAs that can silence specific genes that cause disease. RNAi may be useful for treating cancer, autoimmune diseases, genetic disorders, and viral infections--potentially every human disease caused by abnormal gene activity can be targeted by RNAi. Eliminating the genetic message that creates a harmful protein is a novel upstream approach that addresses the root genetic cause of disease rather than the symptoms.

The Future of RNAi

RNAi-based drugs comprise an entirely new class of human therapeutics. New drug delivery techniques are being investigated and engineered to promote consistent targeted gene silencing. This Nobel Prize winning science has immense capabilities and could improve the lives of patients with limited treatment options.

COVID-19 and RNAi



Investigation of RNAi technology as a prospective treatment strategy for COVID-19 is well underway. Scientists have identified potential targets of RNAi by studying a virus that has genomic similarities with the virus that causes COVID-19. RNAi can be harnessed to inhibit the expression of viral proteins that facilitate infection. An RNAi antiviral for COVID-19 that can be inhaled has demonstrated potency and efficacy in the lab and is on track to begin human testing by the end of the year.

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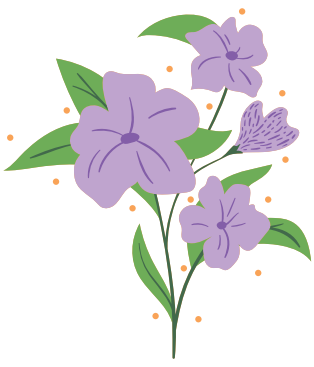
A BRIEF HISTORY OF RNAi

RNAi is a form of gene silencing in which small strands of RNA selectively intercept and destroy the information needed to build proteins.

1977

Scientists learn that RNA is divided into regions that make proteins (**exons**) and regions that do not (**introns**). The exons can be joined together by a process called RNA splicing.



1990

Botanical researchers discover a natural form of gene silencing (**cosuppression**) in petunias

1993

RNA splicing wins Nobel Prize for Physiology or Medicine

1998

Scientists coin the term **RNAi** after discovering that small pieces of RNA can trigger gene-specific silencing naturally in worms.

2001

Scientists successfully reduce gene expression using **synthetic** siRNA

2002

RNAi shown to inhibit **animal viruses** such as HIV and Influenza

2004

First time siRNA is used in **clinical trials** for age related macular degeneration

2006

RNAi wins **Nobel Prize** for Physiology or Medicine

2008

Compelling evidence for RNAi to be used as a therapeutic in **humans**

2016

46 registered clinical trials that involve **RNAi therapeutics**

2018

FDA approves first RNAi based drug for patients with peripheral nerve disease caused by hereditary transthyretin-mediated **amyloidosis** (hATTR)

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