Targeting the genetic code to cure diseases

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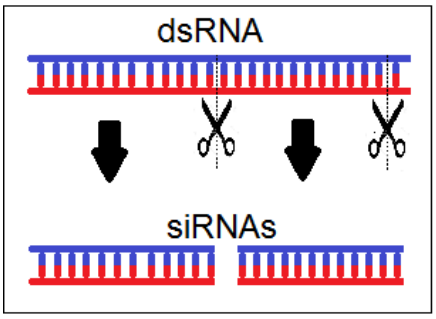
It is small, it is short and it can cure diseases. DNA's cousin can be used in gene therapy to treat a variety of genetic diseases and cancer, as well as fight against bacterial and viral infections. The cousin, small interfering RNA (siRNA), are so called because of their small size and their natural ability to interfere with protein synthesis. This ability is taken advantage of to silence bacterial, viral or native pathogenic genes to essentially cure diseases.

RNA

RNA is composed of ***r***ibose sugars instead of ***d***eoxyribose like DNA, and it is usually single-stranded in contrast to the double-stranded (ds) DNA molecule. RNA is a widely-known molecule as a coding strand for protein synthesis in all forms of life. However, there are other roles of RNA and they too can exist in a double-stranded nature like their cousin, the DNA.



Long dsRNAs are cut in cells into smaller fragments by enzymatic scissors called Dicers. These generated fragments are called small interfering RNA (siRNA) as they interfere and inhibit protein synthesis. SiRNA is integrated into the RNA-induced silencing complex (RISC) which function to silence genes expression either by degradation or translational inhibition.

Inhibition of protein synthesis

DsRNA in cells are cleaved into small fragments and then incorporated into a protein complex, which binds to coding RNA. Once bound, the complex acts to silence protein synthesis by either degrading the coding RNA or preventing the protein synthesising machinery from accessing the code.

Cellular vehicles

For siRNA to be used in clinical settings it is essential to encapsulate them in nanosized particles for efficient delivery. These nanoparticles act as vehicles that carry siRNAs to target cells where they can inhibit gene expression, and consequently, protein synthesis in the targeted cells. The efficiency of siRNA and these carriers have been demonstrated in laboratories to varying degrees. Research is currently underway to develop efficient carriers in hopes of one day using siRNA to treat disease-causing cells, from genetic to acquired diseases such as Huntington's or cancer.

For more information

Görgülü, A. 2016. Systemiska och cellulära begränsningar med *in vivo* RNAi behandling och utveckling av siRNA vektorer i terapeutiskt syfte.