

EDITORIAL

Non-coding RNAs and the advent of new directions in therapeutics

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A huge explosion in the discovery of non-coding RNAs and their tremendous contributions to control of gene expression have truly revolutionized our thinking about biological processes and the origin of disease. MicroRNAs, for example, form a network of fine controls for genes that affect multiple cellular activities and single miRNAs can have multiple targets. MicroRNAs are now known to have a regulatory role in virtually every aspect of cell and organ function. The molecular biology underlying the cellular production of miRNAs, including synthesis, processing, targeting control over cellular gene expression and protein synthesis, has been one of the most rapidly developing fields in recent years. Still there is much to learn about the types of ncRNAs and their various roles in determining the epigenetic status of chromosomes and their post-mRNA function in processes as broadly ranging and diverse as embryonic development and cell fate determination, the acquisition of immunity, brain biology and growth. In addition to normal processes, they are significantly involved in diseases that are heritable, acquired or involve infectious agents. Because aberrant or compromised expression of these regulatory elements appears to have pivotal roles in health, longevity and responses to medical intervention, miRNAs are likely to become important therapeutic targets and gene therapies in their own right.

Small-interfering RNAs (siRNAs) are RNA duplexes that utilize the miRNA machinery for sequence-specific inhibition of gene expression via targeted cleavage of complementary mRNAs. The design of siRNAs for a particular target is straightforward, and they can be produced by chemical or biochemical methods and delivered exogenously or expressed endogenously in cells and tissues. The use of siRNAs and RNAi, for example, can be used to alter expression of the disease-causing or -enabling gene products to achieve a therapeutic outcome. The chapters that follow offer particular insights into the biochemistry of miRNAs of various types, methods for their gene targeting and delivery, as well as possible therapeutic targets.

CONFLICT OF INTEREST

The author declares no conflict of interest.

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