

Adeno-associated virus

Adeno-associated virus (AAV) is a nonpathogenic human virus that belongs to the Parvoviridae family. Replication-defective AAV, which is devoid of all viral genes, is utilized as a gene transfer vector. AAV vectors do not integrate into the host genome. AAV vectors have several desirable properties for in vivo gene transfer, including low immunogenicity, lack of inflammatory response following in vivo administration, and the ability to deliver genes into postmitotic cells (eg, cardiac myocytes) with high efficiency.

Antagomir

An antagomir is a chemically modified antisense oligonucleotide. Typically, a locked nucleic acid modification is utilized to restrict the ribose moiety of the nucleotide into a conformation ideal for complementary Watson-Crick binding. This modification confers long-lasting stability to the antagomir. Antagomirs bind and deplete microRNAs from cells, thereby preventing microRNA-induced messenger RNA degradation and translational inhibition.

Dicer

Dicer is a large multidomain protein possessing RNase III activity that is involved in processing larger RNA precursors into smaller RNA species. Dicer is responsible for cleaving pre-microRNAs into mature microRNAs, and double-stranded RNAs into small interfering RNAs.

Episome

Episome refers to an extrachromosomal genetic element (eg, DNA) that is capable of replication when supplied with the necessary factors. Therefore, episomal expression vectors allow for gene transfer and subsequent transgene expression in the absence of chromosomal integration.

Exosome

Exosomes are small vesicles derived from late endosomes. Exosomes participate in secreting damaged proteins and RNA from the cell.

Nanoparticle tracking analysis

Nanoparticle tracking analysis allows for the visualization and analysis of particles in liquids by relating the rate of Brownian motion to particle size. This method is able to determine the size distribution of small particles in liquid suspension with a diameter of approximately 10 to 1000 nm.

Pluripotency

Pluripotency refers to a stem cell with the ability to differentiate into any of the three germ layers (endoderm, mesoderm, and ectoderm). Furthermore, pluripotent stem cells can be further divided into completely pluripotent (ability to form every cell of the embryo proper) or partially pluripotent (ability to form cells of all three germ layers, but does not exhibit all the characteristics of a completely pluripotent stem cell).

RNA-induced silencing complex

The RNA-induced silencing complex (RISC) is a multiprotein complex that incorporates one strand of small interfering RNA or microRNA and uses it as a template to recognize complementary messenger RNA. Upon recognition of the complementary strand, the RISC subsequently activates a protein within its multiprotein complex known as argonaute, which cleaves the RNA.

Myocardin

Myocardin is a serum response factor transcriptional coactivator found specifically in cardiac and smooth muscle, which belongs to a family of transcription factors and plays a key role in inducing smooth muscle differentiation.