Medicinal use of ribozymes in the treatment of various diseases.

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Description

Ribozymes, similar to protein enzymes, catalyze chemical reactions using their three-dimensional structure. However, ribozymes are made of RNA nucleotides, not amino acids, like protein enzymes. Many chemical processes, such as cleavage, ligation, and isomerization events, can be catalyzed by ribozymes. RNA molecules exhibiting catalytic activity are known as ribozymes. Since its discovery in the early 1980s, RNA chemical catalysis has been linked to a number of crucial biological activities, including peptide bond formation during translation, RNA processing, RNA splicing, and RNA processing. With the exception of the ribosome, all naturally existing ribozymes catalyze the breaking or ligation of the RNA phosphodiester backbone. Unlike other ribonucleases that are now known, ribozymes catalyze highly sequence-specific reactions that are controlled by interactions between the ribozyme and its substrate molecules' RNA. The RNA molecule holds the substrate molecule's recognition, binding, and cleavage reaction. Ribozymes are very interesting molecular tools and potential gene suppressors with crucial uses due to their ability to precisely inactivate other RNAs.

Antiviral properities of ribozymes

Several areas of study, including cancer and genetic illnesses, have looked into the potential of ribozymes as therapeutic agents. Through the cleavage of mRNA transcripts, ribozymes control the expression of the target gene(s) in a negative manner. If a gene's expression might contribute to disease, ribozymes might be used as a therapeutic strategy to reduce that gene's activity. Several crucial genes involved in viral replication have been chosen as potential targets in earlier research. The use of ribozymes will unavoidably treat the viral infection by reducing viral replication.

The Human Immunodeficiency Virus (HIV), Herpes Simplex Virus (HSV), and human cytomegalovirus have all been used as targets in antiviral ribozyme research. It was shown that certain ribozymes have the potential to be employed as therapeutic agents in both *in vitro* and *in vivo* settings. The effectiveness of antiviral ribozymes can be studied using a variety of methods. The ribozyme can be rationally built if a target gene is chosen and the cleavage site is identified. If the cleavage location is unknown, the prospective target cleavage site might be screened to find any region that is exposed to the ribozyme for simple bindings shown that certain ribozymes have the potential to be employed as therapeutic agents in both *in vitro* and *in vivo* settings. The effectiveness of antiviral ribozymes can be studied using a variety of methods. The ribozyme can be rationally built if a target gene is chosen and the cleavage site is identified. If the cleavage location is unknown, the prospective target cleavage site might be screened to find any region that is exposed to the ribozyme for simple binding. Another approach is to search a library of ribozymes for any ribozymes that have a high affinity for or ability to cleave the target virus.

Since the viral genome is only available as RNA in hepatitis C virus infections, ribozyme-mediated gene therapy is particularly well adapted to treating these conditions. The primary targets have been the nucleocapsid coding sequence and the highly conserved 5′ untranslated region (UTR). Certain hammerhead ribozymes have significantly decreased the amount of HCV RNA in patients' hepatocytes with chronic HCV infection. For patients with chronic infections, a viral load reduction of this magnitude is crucial to their quality of life. Furthermore, it has been demonstrated that nuclease-resistant hammerhead ribozymes greatly reduce the replication of an HCV-poliovirus chimaera by concentrating on several locations in the 5′-UTR. It has been shown in other tests using hairpin catalytic motifs that ribozymes are in charge of cellular resistance to infection by retroviral particles carrying HCV target sequences. Modified hammerhead ribozymes are being tested in a clinical trial to combat HCV RNAs.

Conclusion

Ribozymes are the unique class of RNA molecules with biotechnology and medicine applications. Their catalytic activity has been extensively studied, and they have the potential to be used as tools for gene knockdown, gene therapy, and biocatalysis. They are a promising tool for a variety of applications due to their specificity, high catalytic activity, versatility, and potential therapeutic applications.

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