"Clicking" Gene Therapeutics: A Successful Union of Chemistry and Biomedicine for New Solutions

The use of nucleic acid, DNA and RNA, based strategies to disrupt gene expression as a therapeutic is quickly emerging. Indeed, synthetic oligonucleotides represent a major component of modern gene therapeutics. However, the efficiency and specificity of intracellular uptake for nonmodified oligonucleotides is rather poor. Utilizing RNA based oligonucleotides as therapeutics is even more challenging to deliver, due to extremely fast enzymatic degradation of the RNAs. RNAs get rapidly degraded in vivo and demonstrate large off-target binding events when they can reach and enter the desired target cells. One approach that holds much promise is the utilization of "click chemistry" to conjugate receptor or cell specific targeting molecules directly to the effector oligonucleotides. We discuss here the applications of the breakthrough technology of CuAAC click chemistry and the immense potential in utilizing "click chemistry" in the development of new age targeted oligonucleotide therapeutics.

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