Jonathan Perreault, Transcriptomics 2016, 4:2 (Suppl)
http://dx.doi.org/10.4172/2329-8936.C1.006

Design and selection of hammerhead ribozymes

Jonathan Perreault
INRS - Institut Armand-Frappier, Canada

Since the 1990s, hammerhead ribozymes have been studied in regards to gene therapy because of their simple RNA-cleaving catalytic property and the relative ease with which they could be designed to target mRNAs through sequence modifications permitting base complementarity with the RNA to be cleaved. Since then, the discovery of RNA interference and CRISPR have relegated ribozymes behind, while spurring a renewed interest in noncoding RNA-mediated gene therapy. However, meanwhile advances in design and major discoveries on hammerhead ribozymes, such as better activity when stem I and II interact, have opened new avenues. We have demonstrated the high efficiency of hammerhead ribozymes by using combinations that target the same mRNA. Moreover, the automated design software, RiboSoft, streamlines the use of ribozymes for gene knockdowns. Results of RNA targeting against PABPN1, a gene involved in hereditary diseases and other RNAs will be shown. Ribozymes have many advantages over RNAi and CRISPR: they are inherently active and do not rely on any accessory protein or component, making them easily portable to any organism; since they do not require processing and rely on structure for their activity, other modules can be added for their function and they can be assayed *in vitro*. In short, the revived interest in using RNA for gene therapy is likely to also help ribozymes make a come back by stimulating research for general problems such as gene delivery.

Biography

Jonathan Perreault has completed his PhD at the University of Sherbrooke in Canada and Post-doctoral studies at Yale University in the laboratory of Ronald Breaker. He joined INRS – Institut Armand-Frappier in 2011. His work on functional nucleic acids has been published in reputed journals such as *Nucleic Acids Research* and *Nature*. It encompasses bioinformatics, biochemistry, molecular biology and microbiology approaches aiming at discovering and elucidating ncRNAs as well as developing applications.

jonathan.perreault@iaf.inrs.ca

Notes: