RNA Aptamer

RNA Aptamers refer to RNA oligonucleotides that are capable of binding to specific targets with high affinity and specificity. Through a process called Systematic Evolution of Ligands by EXponential enrichment (SELEX), a number of RNA aptamers have been identified against various targets including organic compounds, nucleotides, proteins and even whole cells and organisms. RNA aptamers have proven to be of high therapeutic and diagnostic value with recent FDA approval of the first aptamer drug and additional ones in the clinical pipelines. It has also been found to be a particularly useful tool for cell-type specific delivery of other RNA therapeutics like siRNA. All these establish RNA aptamers as one of the pivotal tools of the emerging RNA nanotechnology field in the fight against human diseases including cancer, viral infections, and other diseases [1].

The modest benefit of conventional therapies is due to the presence of glioblastoma stem cells (GSCs) that cause tumor relapse and chemoresistance and, therefore, that play a key role in GBM aggressiveness and glioblastoma recurrence. So far, strategies to identify and target GSCs have been unsuccessful. Thus, the development of an approach for GSC detection and targeting would be fundamental for improving the survival of GBM patients.

Affinito et al. using the cell-systematic evolution of ligand by exponential (SELEX) methodology on human primary GSCs, they generated and characterized RNA aptamers that selectively bind GSCs versus undifferentiated GBM cells. They found that the shortened version of the aptamer 40L, which we have called A40s, costained with CD133-labeled cells in human GBM tissue, suggestive of an ability to specifically recognize GSCs in fixed human tissues. Of note, both 40L and A40s were rapidly internalized by cells, allowing for the delivery of the microRNA miR-34c and the anti-microRNA anti-miR-10b, demonstrating that these aptamers can serve as selective vehicles for therapeutics. In conclusion, the aptamers 40L and A40s can selectively target GSCs. Given the crucial role of GSCs in GBM recurrence and therapy resistance, these aptamers represent innovative drug delivery candidates with great potential in the treatment of GBM [2].

References

1)
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2)
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