

Engineering Next-Generation snRNAs to Leverage Their Splicing Capabilities for Targeted Therapeutics

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Abstract

Many diseases, particularly those involving neurodegeneration, are likely due to adverse splicing pathology. By engineering snRNAs, a key element of the spliceosome complex, targeted therapeutics can be made to induce the desired splicing outcome. This project used engineering principles to engineer U1 snRNA molecules and tested them using a novel reporter system. Many effective, stable snRNAs outperformed their wildtype counterparts, opening the door for future investigations regarding their therapeutic potential.