Degradation of Toxic RNA in Type 1 Myotonic Dystrophy (DM1)

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Gain-of-function of expanded CUG repeats (r(CUG)^{exp}) is the predominant cause of DM1. The toxic r(CUG)^{exp} sequesters muscleblind 1 (MBNL1) and upregulates CUG binding protein 1 (CUGBP1), which collectively leads to splicing misregulation responsible for key features observed in DM1-inflicted patients. Herein, we describe methods to degrade r(CUG)^{exp} which leads to the correction of splicing defects of two well-studied pre-mRNAs.

Synthesis and Development of Materials for Shock Wave Energy Dissipation (SWED)

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Dissipating shock wave energy by mechanochemically active compounds is a promising approach for protecting personnel and machinery from detonation. It has been reported that polyurea absorbs *low energy* shock waves via disruption of hydrogen bonds. However, there are no reports of dissipating *high energy* shock waves using covalent bond changes. We hypothesized that endothermic chemical reactions with breakage of covalent bonds and with volume collapse in the molecules allow for dissipation of *high energy* shock waves. The diradical intermediate in the Bergman cyclization (BC) meets both of the desired qualities for SWED, but the intermediate must be kinetically stabilized to dissipate shock energy. This work focuses on the synthesis of aromatic enediynes that kinetically stabilize the diradical intermediate formed in BC. The energy dissipating properties of the enediynes tested by laser-induced stress wave experiment will also be discussed.