



## SPLICE-SWITCH ANTISENSE OLIGONUCLEOTIDES CONTROLLING TITIN ISOFORM EXPRESSION AND USES THEREOF

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### The Invention

UW-Madison researchers have developed new splice-switching antisense oligonucleotides (SSOs) to shift titin protein isoform production from the smaller isoform to the larger isoform, which could alleviate diastolic dysfunction in patients with heart failure with preserved ejection fraction (HFpEF). To promote the larger titin isoform, the inventors designed SSOs aimed at inhibiting RBM20 binding on titin mRNA and selected the two most effective SSOs for testing in mice. Their results demonstrate that these SSOs significantly promote the expression of the larger titin isoform, to a greater extent than the previous generation of SSOs (in WARF P230412). The inventors believe these SSOs hold promise as gene therapy candidates for treating diastolic dysfunction in HFpEF patients.

#### Tech Fields

- [Therapeutics & Vaccines : Cardiovascular](#)

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