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Toward an optimized RNAi construct for silencing the Z alpha-1-antitrypsin variant

Alpha-1-antitrypsin deficiency is a common hereditary disease that most frequently results from a Glu342Lys substitution in the serpin protease inhibitor, alpha-1-antitrypsin (AAT). This mutation causes AAT to aggregate and progressively form inclusion bodies within hepatocytes, which leads to cirrhosis. We seek to prevent liver disease in AAT deficiency patients by developing a highly potent and specific RNA interference (RNAi) construct to block the production of mutant AAT in hepatocytes. We have successfully produced six plasmid-based RNAi constructs that are ready for testing.



Poster Number:

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