RNA Aptamers Targeting a coagulation Cascade Cofactor

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Value Proposition

Thrombosis (blood clots within blood vessels) is a major underlying factor in many cardiovascular diseases and a major source for complications following hospital stays and surgeries. Venous thrombosis occurs in approximately 1 per 1000 annually in adult populations. Deep vein thrombosis has over 200,000 US cases a year while pulmonary embolism occurs in more than 200,000 US cases per year. A genetic disorder, FV leiden, is an inherited blood-clotting disorder that occurs due to a mutation of the blood's factor V (FV) protein. There are more than 3 million cases a year in the US. Currently, anticoagulants (blood thinners) are used to treat these disorders. However, anticoagulants are associated with significant bleeding that increases patient morbidity and mortality. Heparin (an anticoagulant) is highly used despite these risks because it may be controlled by an antidote which allows for reversal of the detrimental effects.

Despite antidote control being an important part of drug development, to date there are few drug-antidote pairs available to treat blood clots. Thus, there is a need for specific therapies with available antidotes in order to reduce morbidity and mortality among these patients.

Technology

The genetic disorder FV Leiden occurs when there is a mutation in the FV gene. These patients are currently treated with anticoagulants, however before now none of the anticoagulants actually target the FV protein. This technology uses an RNA aptamer (oligonucleotide sequence) to bind to a specific co-factor (FVa) that is associated with blood clot formation. The specificity of this aptamer to its target is an advantage, moreover this aptamer is also reversible

Thus, this aptamer therapy is the first one of specifically inhibit FVa. This therapy could potentially reduce the overall number of FVa molecules that create a functional blood clotting complex in these patients, and could reduce their risk of blood clots.

Advantages

- This technology shows a specific aptamer that binds to specific components of the blood clotting complex (FVa)
- These aptamers are reversible, allowing for specific use spatially and temporally.