Aptamer Based Sickle Cell Anemia Therapeutic

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Technology description

INVENTION NOVELTY

This technology is a set of RNA aptamers capable of reducing sickle hemoglobin (HbS) polymerization, which has therapeutic potential in reducing vaso-occlusion and ischemia in sickle cell disease patients. Sickle cell anemia is a genetically inherited disease affecting 1 in 400 African American and up to 2% of the population in some areas of Africa. It results in malformed sickle-shaped red blood cells. HbS polymerize and adhere to the vascular endothelium, resulting in reduce blood flow and blokage. The symptoms and severity of the disease varies between patients and can include: severe pain, vaso-occlusion, anemia, retinopathy, organ damage, high risk of stroke and renal insufficiency. Current therapies (bone marrow transplantation, routine blood transfusion, or hydroxyurea medication) are expensive and/or ineffective. The use of RNA aptamers as a sickle cell disease therapy is advantageous.

TECHNICAL DETAILS

Johns Hopkins researchers have identified a number of aptamers capable of binding HbS cells with high affinity via a high-throughput selection. These HbS binding aptamers were subsequently screened for blocking HbS polymerization in vitro. To date, three RNA aptamers are capable of reducing HbS polymerization.

Advantages

Potential to be highly effective compared to current standards of care
Low cost compared to transplantation and transfusion
Potentially safer than hydroxyurea, a drug that indirectly prevent HbS polymerization
Compared to therapeutic antibodies, aptamers are cheaper, can be stored at ambient temperature

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