Biomedical

VCU TechTransfer and Ventures
Office of the Vice President for Research and Innovation

Novel ATP and 2,3-BPG Targeting Drug for Sickle Cell Disease Treatment

Lipid particle packaging of PLX small molecules providing localized delivery to treat lung cancers.

Sickle Cell Disease (SCD) is the most common inherited hematologic disorder, affecting over 100,000 people in the US, and millions worldwide. The disease occurs as a result of a single point mutation affecting hemoglobin (Hb) in red blood cells (RBCs). Under hypoxia, low oxygen conditions, or when HbS becomes deoxygenated (deoxy-HbS), it polymerizes causing sickling of RBCs, leading to several debilitating symptoms such as vaso-occlusion, oxidative stress, inflammation, hemolysis, painful crises, and multiple organ damage that ultimately leads to poor quality of life and decreased life expectancy. Current treatments for sickle cell disease are insufficient and do not work in some patients. To help overcome this issue, researchers at VCU have developed a noninvasive treatment using a small molecule activator of the glycolytic enzyme pyruvate kinase to decrease 2,3—BPG levels, as well as boost energy (ATP) production in RBCs, preventing sickling of RBC and hemolytic anemia; two key steps in the disease pathogenesis.

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The technology

Pyruvate kinase plays a key role in glycolysis; involved in 50% ATP production that drives many cellular functions, as well as 2,3-bisphosphoglycertae (2,3-BPG) production that serves a critical function in hemoglobin (Hb) tissue oxygenation. Reduced ATP level as a result of dysfunctional production of pyruvate kinase leads to hemolysis and chronic anemia responsible in part for SCD pathogenesis. Elevated 2,3-BPG in RBCs as a resulted of a compensatory mechanism to increase tissue oxygenation exacerbates sickling of RBCs. The novel drug targets pyruvate kinase to increase ATP production with concomitant decrease of 2,3-BPG that is expected to lead to a novel treatment for sickle cell disease.

Benefits

- Noninvasive
- Reduces SCD symptoms/complications
- High efficacy

Applications

- Sickle Cell Disease

Patent status:
Patent pending: U.S. and foreign rights are available.

License status:
This technology is available for licensing to industry for further development and commercialization.

Category:
Biomedical

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