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Researchers develop novel method for treatment of sickle cell disease

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Virginia Commonwealth University researchers have developed a unique anti-sickling agent that may one day be effective in treating sickle cell disease, a painful and debilitating genetic blood disorder that affects approximately 80,000 Americans.

The research team led by Donald Abraham, Ph.D., of Biological and Medicinal Chemistry, in the Department of Medicinal Chemistry in VCU's School of Pharmacy, has shown that 5-HMF, a pure compound developed by the team, has a high affinity for sickle cell hemoglobin and holds promise for the treatment of sickle cell disease.

"Our findings suggest that this anti-sickling agent may lead to new drug treatments and may one day help those suffering with sickle cell disease. This molecule, 5-HMF, is the most promising molecule to treat sickle cell anemia to come from our research group in more than 30 years," said Abraham, who is also the director of the Institute of Structural Biology and Drug Discovery.

The United States Patent and Trademark Office recently issued VCU a Notice of Allowance for a patent relating to a method of treating sickle cell disease with 5-HMF compound. A Notice of Allowance is a written notification that a patent application has cleared an internal review and it has been approved for issuance.

Sickle cell disease is caused by an abnormality in the hemoglobin molecule. Normal red blood cells carrying hemoglobin are smooth,

round and flexible and can travel easily throughout blood vessels. However, sickle cells are stiff, abnormally shaped, red blood cells that do not flow freely through blood vessels. The sickle cells also may clot together causing a blockage to form which results in pain and potentially dangerous complications that can compromise a patient's organs.

According to Abraham, the 5-membered, heterocyclic, anti-sickling agent binds to hemoglobin to increase the oxygen affinity of both normal and sickle hemoglobin. In a patient with sickle cell disease, the binding action of 5-HMF would allow sickle cells to move more smoothly throughout the blood vessels of the body and prevent blockages from forming.

Source: Virginia Commonwealth University