# Research leads to possible new treatment for sickle cell disease

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There's a new ray of hope for those battling sickle cell disease thanks to a compound that was discovered at Virginia Commonwealth University.

The compound, originally patented by VCU under the name 5-HMF, was developed by a team from the VCU Institute for Structural Biology and Drug Discovery, an interdisciplinary research center spanning the

School of Medicine and the School of Pharmacy.

Relabeled as Aes-103, the compound was licensed by VCU Tech Transfer to a startup company, AesRx LCC, which has completed preclinical and Phase 1 clinical trials.

AesRX has recently received regulatory approval for a Phase 2 clinical trial of the anti-sickling agent and plans to begin patient enrollment this month.

The double-blind, placebo-controlled study will evaluate the safety and efficacy of 28-day dosing of Aes-103. The impact of the drug on several sickle cell clinical symptoms also will be evaluated.

Aes-103, currently the only clinical-stage therapeutic that directly targets cell sickling, is one of the first molecules to enter the National Institutes of Health (NIH) Therapeutics for Rare and Neglected Diseases program (TRND) through its National Center for Advancing Translational Sciences (NCATS). The NIH, through a $5-plus million grant, is supporting the first three clinical trials of Aes-103.

"TRND researchers have worked collaboratively with AesRx to provide preclinical drug development expertise and clinical and regulatory resources necessary to move Aes-103 into human clinical trials," said John McKew, Ph.D., acting director of NCATS Division of Preclinical Innovation and director of TRND. "Through TRND, NCATS supports innovative methods and collaborative approaches to accelerate the discovery process while developing promising treatments for the patients who need them."

Sickle cell disease is a recessive disorder of the hemoglobin that can lead to a wide range of serious, sometimes life-threatening, conditions, including chronic hemolytic anemia, chronic pain and acute painful

crisis, stroke, acute chest syndrome and cumulative damage to tissues and organs. More than 100,000 people in the United States are afflicted with sickle cell disease.

"Francis Collins, M.D., Ph.D., the director of the National Institutes of Health, has held this project up as a prime example of translational medicine with academic medical centers working with startup companies in the research and development of life-changing drug therapies," said Francis Macrina, Ph.D., VCU vice president for research.

Provided by Virginia Commonwealth University