Another step toward sickle cell disease treatment

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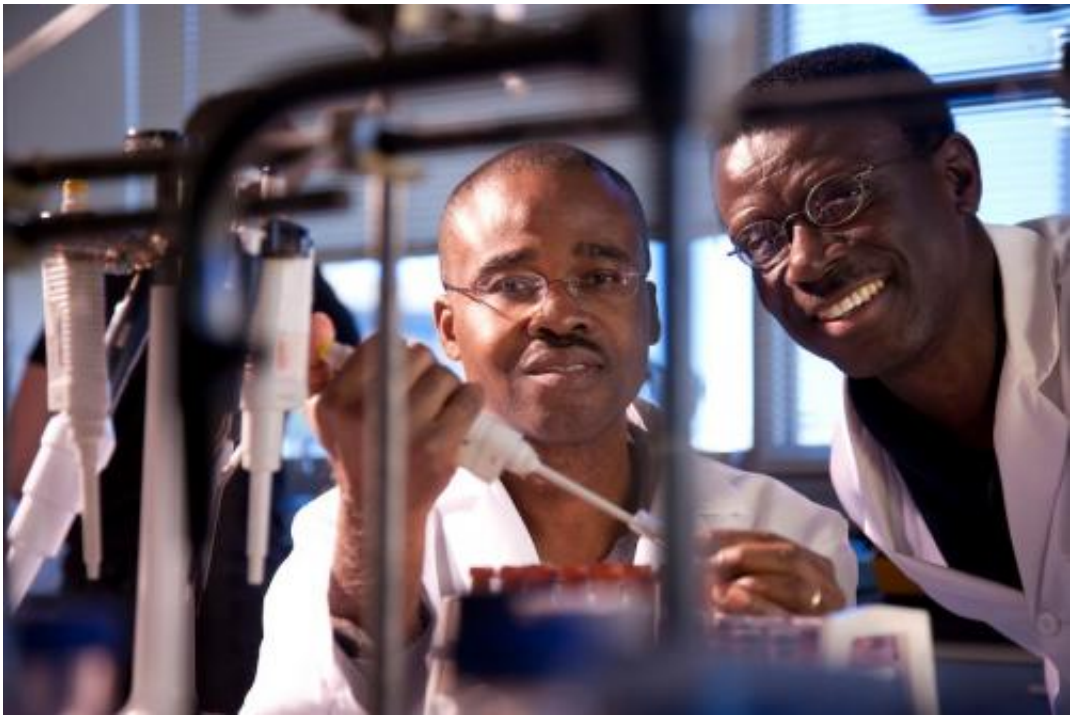

Martin Safo, Ph.D., and Richmond Danso-Danquah, Ph.D.

(Medical Xpress)—A compound discovered at Virginia Commonwealth University has taken yet another step closer to becoming the only approved drug in the world that is therapeutically effective in managing adult sickle cell disease by targeting hemoglobin.

Named Aes-103, the compound is the focus of the lead program at

AesRx, a biopharmaceutical company dedicated to the development of novel drugs. It was announced this week that AesRx was purchased by Baxter International, a global health care company with expertise in medical devices, pharmaceuticals and biotechnology.

"This is so important that I cannot underestimate its value," said Donald Abraham, Ph.D., Alfred and Frances P. Burger Emeritus Professor of Medicinal Chemistry and Biological Chemistry at VCU, who is an original developer of the compound. "Without this financial support, the advanced clinical trials would have taken many years longer."

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. The team included Martin Safo, Ph.D., associate professor of medicinal chemistry; Richmond Danso-Danquah, Ph.D., assistant professor of medicinal chemistry; and Abraham, emeritus director of the institute.

Relabeled as Aes-103, the compound was licensed by VCU Innovation Gateway to AesRx - a startup company at the time - which began clinical trials.

"We are very grateful for the role VCU has played in the development of Aes-103," said Steve Seiler, AesRx founder and CEO. "As a small company focusing on a big unmet medical need, AesRx needed to be pragmatic in its development strategy for Aes-103 and adjust as new data and financing strategies emerged. VCU worked collaboratively with us on this effort and was a true partner with AesRx all along the way."

VCU's liason to the company has been VCU Innovation Gateway, a university resource that facilitates commercialization of university inventions and supports university research through collaborative

agreements.

"Without Innovation Gateway at VCU, and the efforts of our former president Dr. Eugene Trani and his establishment of the Biotechnology Research Park, this invention would have never reached clinical trials," Abraham said. "I am very grateful to have had the opportunity for our basic research to be translated into a potentially useful compound for the treatment of sickle cell anemia."

The Aes-103 program is currently in a Phase 2 clinical trial as part of an ongoing collaboration with the NIH's National Center for Advancing Translational Sciences (NCATS) through its Therapeutics for Rare and Neglected Diseases (TRND) program.

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.

Aes-103 is a first-in-class, oral, small molecule compound. Early studies indicate the compound may work by binding to and stabilizing the relaxed state of hemoglobin and increasing oxygen affinity and stabilization, thereby reducing the sickling of red blood cells which, in turn, may reduce sickling-related outcomes such as vaso-occlusive crisis, pain, severe anemia and fatigue.

Provided by Virginia Commonwealth University