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Fighting sickle cell disease by looking back to infancy

Promising research targets key enzyme

MEDICAL UNIVERSITY OF SOUTH CAROLINA

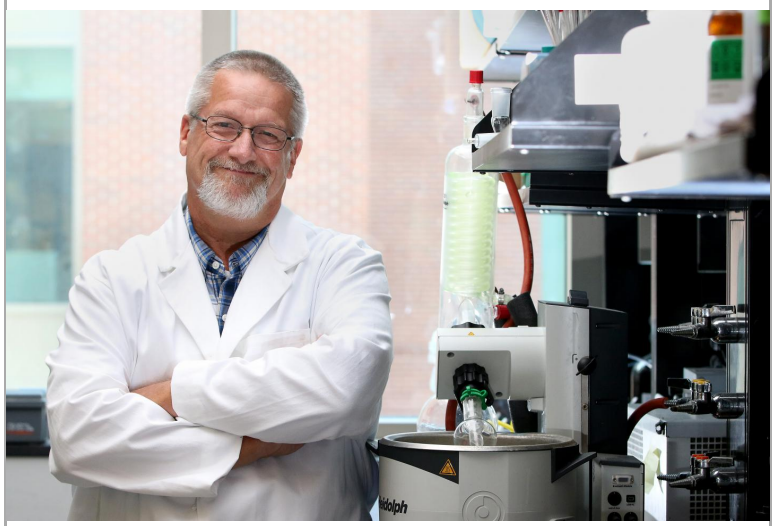

IMAGE: DR. PATRICK WOSTER'S RESEARCH RECENTLY GOT A BOOST FROM THE DORIS DUKE CHARITABLE FOUNDATION, WHICH SELECTED IT AS AN AWARDEE. **view more** ›

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Fast-track research focused on developing a new gene-modifying sickle cell disease treatment at the Medical University of South Carolina could lead to human clinical trials in as few as three years.

Patrick Woster, the SmartState endowed chair in medicinal chemistry at MUSC, said the project takes aim at the enzyme lysine-specific demethylase 1, or LSD 1. That enzyme is part of the biological process that makes a mutated hemoglobin, leading to sickle-shaped blood cells. They stick together, causing extremely painful and dangerous blood flow problems. Inhibiting LSD 1, making it less effective, could turn an important gene back on again and keep sickle-shaped cells from sticking together.

"This research could lead to a disease-modifying treatment," Woster said. "We have a good chance to get something on the market soon."

The Doris Duke Charitable Foundation chose Woster's research project, Epigenetic Modulators for the Treatment of Sickle Cell Disease, as an awardee in its Sickle Cell Disease/Advancing Cures funding competition. The foundation is contributing more than $750,000 to help fund his research.

Woster and his team will also benefit from the Food and Drug Administration orphan disease policy. It allows fast-track approval for new drugs to treat sickle cell disease, which affects an estimated 100,000 Americans.

Babies are born producing fetal hemoglobin, also called hemoglobin F. In most cases, by the time they're around six months old, that gene is turned off and their bodies begin making hemoglobin A, or adult hemoglobin.

But people with sickle cell disease make hemoglobin S instead. It causes their blood cells become sickle shaped, leading to lifelong doctor's appointments and hospitalizations, days missed from school and work, and ultimately, a shorter life.

He and Julie Kanter, a hematologist directs sickle cell research at MUSC, have been collaborating on developing the gene-modifying concept for the last three years. "We have an opportunity at MUSC for a true, translational project that can go from bench to bedside with both basic science and clinical components," Kanter said.

Woster said South Carolina's population makes it a natural fit for cutting-edge sickle cell research. "Sickle cell disease is a big problem in the U.S. among the African-American population," Woster said. About 27 percent of South Carolinians are African-American, more than double the national percentage. "I think proportionally we're doing more sickle cell research here than most other universities on a percentage basis."

About three million Americans carry the trait for the disease, and as Woster noted, African-Americans are especially hard hit. One in 13 has the trait, meaning they don't have the disease but can pass it on to their children, and one in 365 African-American babies will be born with the disease.

Woster said LSD 1 inhibitors, such as the ones his group is working on, could have a big impact. "This is a very exciting new avenue to explore. Our group is working on it, and I know of two other groups that are looking at this strategy. Whether we come up with something or those other groups do, I think this is one of the most promising avenues to pursue and holds the greatest hope for discovering a new drug for sickle cell disease."

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