

Proudford Foundation Honors DSU's OrphageniX for Sickle Cell Research

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OrphageniX, a biotechnology company based at Delaware State University, has been presented the Proudford Foundation Award for Research for its work in developing treatments for sickle cell disease.

Dr. Eric Kmiec, chair of the DSU Department of Chemistry and co-founder of OrphageniX, accepted the award on behalf of the company's research staff at the foundation's annual awards dinner on Sept. 20 in Baltimore, Md.



Dr. Nouredine Melikechi, DSU dean of Natural Sciences and Technology; Dr. Eric Kmiec, chair of the Dept. of Chemistry and co-founder of OrphageniX; and Alton Thayer, president of the Proudford Foundation
Provost Alton Thayer

The Proudford Foundation was established in the memory of DSU alumnus William E. Proudford, who passed away in 2004 at age 76 after a long and brave fight against sickle cell disease. Mr. Proudford graduated from then-Delaware State College with a bachelor's degree in accounting in 1974.

The scientific basis for OrphageniX was born in the Kmiec laboratory during the mid-1990s. The Kmiec group is well known for its work in the area of human gene therapy and molecular medicine. The company pioneered the concept of gene editing, a molecular process in which a synthetic piece of DNA is introduced into a human cell in order to direct the correction of a genetic mutation that causes an inherited disease. The repair of this inborn error in the chromosome can be thought of as a genetic "spell check" in which the misspelling of the word (or gene) is simply corrected and the disease state reversed.

During the time period between 2000-2006, the Kmiec lab studied and deciphered the molecular mechanism of action – how gene editing actually takes place inside the human cell. This effort was supported by multiple, peer-reviewed (R01) research grants from the National Institutes of Health, the highest level of scientific validation for any technology or research project. The Proudford Research Award has recognized this pioneering effort.

"The early years were challenging as the technology was matured, but now that gene editing has entered the mainstream of science, it was a worthwhile journey," Dr. Kmiec said.

OrphageniX is now focused on creating a genetic treatment for sickle cell disease, a disorder caused by a single base mutation (misspelling) in a single gene – that is, a misspelled word in the genetic code. Funding for OrphageniX came from a small number of Delaware angel investors; no venture capital money has been put into OrphageniX. The company is privately held and is now an

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attractive investment opportunity. OrphageniX is now working with DSU and A.I. DuPont/Nemours Children's Hospital to develop a unique partnership to apply gene editing to sickle cell disease focused in the Delaware Valley region.

This combination brings together strong basic scientists and highly respected clinicians with the central, interdisciplinary theme of translating this validated technology toward clinical application. DSU students, primarily African-American, are heavily involved in this project. OrphageniX is fundamentally committed to helping minority students pursue a career in gene therapy in support of inherited diseases such as sickle cell disease particularly at Historically Black Colleges and Universities.

“We are gratified that the Proudford Foundation has recognized the cutting-edge work that the DSU-based OrphageniX is doing in the area of sickle cell disease,” said Dr. Alton Thompson, DSU provost and vice president of Academic Affairs. “I am certain that more positive developments are to come from this innovative biotechnology company as it seeks and finds significant advances against this disease.”

The mission of the Baltimore-based Proudford Foundation is to support sickle cell awareness, education, state-of-the-art treatment and research, and to bring hope to families affected by the devastating disease.

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