

## **DSU Inherits Exciting Biotechnology Company**

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With the arrival this semester of Dr. Eric Kmiec as the Chairman of the Department of Chemistry, the University has also inherited an exciting biotechnology company that is focusing on the development of an innovative molecular medicine approval that can treat patients with Sickle Cell Disease (SCD)

The company – OrphageniX, Inc. – was begun by Dr. Kmiec and other scientists and a group of investors in 2000 when he taught and did research at the University of Delaware. In the beginning, OrphageniX worked to develop gene alteration treatments for several orphan diseases – maladies that affect a relatively low number of people. But now, the company has narrowed its focus to Sickle Cell Disease.

The technology is known as gene editing and centers on the concept that inborn genetic errors (mutation) which result in the manifestation of diseases like SCD can be reversed. Such correction can partially diminish the symptoms of the disease. The gene editing technique can be thought of as a spell-checker in which the misplaced letters of the mutant gene are corrected and the normal gene function is restored.

Kmiec and his lab pioneered this approach toward genetic diseases in the early 2000s and now the gene editing field is flourishing. “It is good to see this growth, as it is indicative of a healthy scientific approach,” said Kmiec, who added “Validation by others, even using other competitive technologies, is good any type of scientific investigation.”

During the course of the last five to seven years, Kmiec’s group and others have been defining the mechanism of action and regulation of gene editing, a crucial step in developing credibility for any new molecular medicine. “There have been a lot of starts and stops in other gene therapy investigations,” said Kmiec. “We wanted to understand as many parameters of this reaction as we could before thinking seriously about clinical application.”

During this period, Kmiec’s work was supported by the National Institute of Health with ROI awards, its most prestigious research grant; Kmiec’s lab has been funded for 16 years with several of the ROI awards during this period. In addition, OrphageniX used a “virtual company” strategy to file a series of strong patents and establish a strong intellectual property position for their technology in the business world. They spent most of their money on strengthening their patents and relied on Kmiec to advance the science using peer-reviewed grant support and publications.

Because it was developed at the University of Delaware, that institution holds the original patents on the technique. However, Dr. Kmiec said, “Now that the company is part of DSU, any new developments in the technique will likely be owned by DSU.

The choice of SCD as the primary target for OrphageniX was made about a year ago and the company is hard at work getting ready to begin its focused studies. SCD is a serious disorder in which the body makes sickle-shaped (crescent) red blood cells that contain abnormal sickle hemoglobin. Such sickle cells tend to block blood flow in the blood vessels of the limbs and organs, and can cause pain, serious infection and organ damage. Sickle Cell Anemia – low blood cell count– is the most common form of SCD. “Sickle Cell has the golden child of gene therapy,” Dr. Kmiec said. “Everyone wants to develop a therapy it. It seems so simple, but it’s not.”

Dr. Kmiec said he wants to make DSU a major part of the success and growth of the company and the research. “I see the company engaging some DSU students from the College of Business to learn, observe, and even help craft business strategies for the company,” he said. “This could be a great case study for the students of that college because the company functions as a real business, not just a hypothetical work experience. What we all do here at DSU will count”

In addition to its potential for engaging DSU’s business and natural science students, Dr. Kmiec said he sees some great possibilities for partnership with other institutions. He said discussions are currently ongoing among DSU the University of Pennsylvania and CHOP on collaborations. In this area of genetic engineering, a clinical partner is a critical component of the

success of any biotech company, corporate or academic, to translate the bench top science to their bedside patient practice.

The Department of Chemistry chair said that he is not looking for DSU to provide financial support for the company. Dr. Kmiec noted that DSU is not a venture fund and outside financial investments have solely supported the company in the past and new support is now needed to move the technology forward.

Michael Bowman, OrphageniX's business manager, called the company's scientific product "an enormous therapy" for which the potential to attract other significant partners is great and that could result in a great success story for the University and the State of Delaware. "It is our hope that the bandwidth of DSU can help us with this," said Mr. Bowman.

The Kmiec group's expertise, the company OrphageniX, and Delaware State University each bring something valuable to the table. "It is such an interesting and exciting opportunity to do something like this at DSU and in Dover, and perhaps initiate some more activity in the more southern part of the state," said Kmiec. "The DSU administration is setting a high bar and challenging us to develop an entrepreneurial environment even in these economic times," add Kmiec. "By utilizing the talent and enthusiasm based at DSU and challenging the investment community to support this effort here, we can create an exciting new environment in which to meet student needs and exceed expectations."

According to Centers for Disease Control Prevention:

- SCD affects 90,000 to 100,000 Americans.
- SCD occurs among about 1 out of every 500 Black or African American births.
- SCD occurs among about 1 out of every 36, Hispanic-American births.
- Sickle Cell Trait (SCT) occurs in people who inherit one sickle cell gene and one normal gene. Such people usually do not have SCD, but they can pass on the trait to their children.
- SCT occurs in about 1 in 12 Blacks or African-Americans.

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