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# UI strikes agreement with Pfizer Inc. to develop potential cystic fibrosis gene therapy

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The University of Iowa Research Foundation recently finalized a license and sponsored research agreement with Pfizer Inc. to support the development of potential gene therapies for cystic fibrosis (CF) by the laboratories of the University of Iowa (UI) professors John Engelhardt and Ziying Yan.

Engelhardt, Ph.D., is Professor and Chair of Anatomy and Cell Biology in the Carver College of Medicine, Roy J. Carver Chair in Molecular Medicine in the Carver College of Medicine, and Director of the UI Center for Gene Therapy. Yan, Ph.D., is a Research Associate Professor of Anatomy and Cell Biology. Engelhardt and Yan have worked in the gene therapy field for 25 years.

Pfizer, through its Genetic Medicines Institute that is headed by Michael Linden, will collaborate with the Engelhardt and Yan labs to develop a potential unique gene therapy for cystic fibrosis, an inherited disease that causes severe damage to the lungs and digestive system. Cystic fibrosis is caused by a defect in a single gene, making gene therapy an attractive approach for attempting to find potential a cure for CF patients.

Gene therapy works to treat genetic diseases by delivering a corrected copy of a defective gene into patient's cells. In this case, the vehicle used to deliver the corrected gene is a hybrid of the adeno-associated virus (AAV) and the human bocavirus. This new viral vector system–which was co-developed by Engelhardt, Yan, and bocavirus expert Professor Jianming Qiu of the University of Kansas–also has potential for vaccine applications against viral infections of the lung.

"Efficiency of delivery is key to any gene therapy approach," Engelhardt said. "The transport vehicle we have developed is an engineered virus that is highly efficient for entering human airway cells. Additionally, the 'truck' we use for transportation can carry a larger payload, which is especially important for cystic fibrosis since the diseased gene is very large."

The Engelhardt and Yan teams will focus on testing the efficacy of the vector system in cystic fibrosis model systems, while Pfizer will focus on packaging systems for the vector and optimization of the manufacturing process.

"This collaboration with Pfizer brings unique strengths that could enable the translation of this new vector system to clinical trials for CF lung disease and the discovery of potential new gene therapy applications for the vector system," Engelhardt states.

The UI Research Foundation is part of the Office of the Vice President for Research and Economic Development, which provides resources and support to researchers and scholars at the University of Iowa and to businesses across Iowa with the goal of forging new frontiers of discovery and innovation and promoting a culture of creativity that benefits the campus, the state, and the world. More at http://research.uiowa.edu, and on Twitter: @DaretoDiscover, @_UIRF.