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Gene Therapy Breakthrough: New Clinical Trial Treats Congenital Blindness

Elkins Park, Pa. - Alexander Dizhoor, PhD, Hafter Family Chair in Pharmacology and Pennsylvania College of Optometry (PCO) professor, participated in an important research study on gene therapy, and its effectiveness in treating a rare form of congenital GUCY2D blindness. The most recent part of the study was published in October in iScience, the National Library of Medicine within the National Center for Biotechnology Information and SciTechDaily.

The study, led by the late Dr. Samuel Jacobson of the University of Pennsylvania, involved researchers from several academic institutions including UPenn, Salus University, Thomas Jefferson University, University of Florida, in addition to Atsena Therapeutics, Inc.

Dr. Dizhoor participated in the study with the assistance of his Salus University colleagues, Igor Peshenko, PhD, assistant professor, and Dr. Elena Olshevskaya, PhD, instructor.

"Salus University's part in the project was to provide what our strength is, studying biochemistry and molecular biology and physiology of the retina, in particular - photoreceptors," said Dr. Dizhoor. "Especially the GUCY2D gene, mutations in which were the cause of the disease, is exactly what we are specializing in."

The results of the clinical trial showed, as a result of gene therapy, blind patients gained back vision within several weeks. Although their vision was not perfect, sensitivity to dim light increased nearly 1000-fold. Dr. Dizhoor emphasized what a substantial step this is for gene therapy in the rare form of congenital blindness at birth, GUCY2D Leber's congenital amaurosis.

"We have multiple lines of research that we are currently pursuing that will eventually, hopefully, also have some clinical implications," said Dr. Dizhoor. "Our place in the science is to provide the basic knowledge in biochemistry and molecular biology that can create a foundation for future research in gene therapy."

About Salus University

Salus University, founded as the Pennsylvania College of Optometry in 1919, today is a diversified, globally recognized professional academic center of learning that offers a wide range of degree programs in the professions of Optometry, Audiology, Physician Assistant, Blindness and Low Vision Studies, Biomedicine, Occupational Therapy, Speech-Language Pathology and Orthotics and Prosthetics. Salus operates four clinical facilities in Philadelphia and Montgomery counties that provide highly specialized vision, hearing and balance, Speech-Language pathology and occupational therapy services. The University has over 1,200 students, and more than 14,000 alumni worldwide. For more information, please visit www.salus.edu.