Researchers developing molecular delivery vehicles for genetic therapies

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Researchers at New York University are working to develop molecular delivery vehicles that can be used to transport nucleic acids into diverse cell types, which may lead to eventual applications in genetic therapies. Their work is described as part of the cover story in the Nov. 13 issue of the American Chemical Society publication Chemical and Engineering News.

Scientists have been exploring RNA interference (RNAi) as a gene therapy technique to silence genes that are improperly produced. The "RNAi" approach requires the delivery into the cell of short pieces of the genetic material Ribonucleic Acid (RNA). These synthetic short RNA "oligos" can then pair with specific sites in the cell's own RNA, targeting the genetic messages for destruction and turning off expression of the corresponding genes. However, the widespread clinical use of this genetic therapy relies upon technical improvements, including new delivery vehicles such as the one Kent Kirshenbaum, an assistant professor in NYU's Department of Chemistry, and colleagues present in their work.

The NYU researchers use a modular linear molecule to deliver therapeutic RNA into cells. The molecule has a positively charged site that forms favorable stabilizing interactions with the negatively charged RNA, and a fatty component that interacts with cell membranes. The molecules and RNA form complexes, which protect the RNA from being degraded and deliver it to cells. As a result, the targeted deleterious genes are silenced.

Their research concentrates on making the transition from the lab into real-life smoother. Their linear molecule can be used to deliver small therapeutic RNAs into cell types that are much more representative of cellular targets that investigators are likely to encounter in clinical situations.

Kirshenbaum and his coworkers are now focused on understanding the physical chemical characteristics that give enhanced activity to their molecule, and then use the knowledge to generate a set of more sophisticated delivery reagents for siRNA.

"Our goal is to develop a platform that would allow us to create a library that could be used in different settings or for delivery to different cell types," he told Chemical and Engineering News.

Source: New York University