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GRPE elements to help cure HIV via gene therapy

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Singapore: A research team from Case Western Reserve University School of Medicine has discovered an approach that could make gene therapy dramatically more effective for patients.

Led by professor Dr. Eric Arts, the scientists discovered that the process of gene therapy is missing essential elements thereby reducing the effectiveness of this treatment. Re-introducing this element into their model system suggests that improvements for gene therapy horizon.

Advances in gene therapy offer promising treatment for genetic abnormalities, tumors and resistance to toxic chemotherapies. Such therapy has been used to treat cystic fibrosis, hemophilia, muscular dystrophy and sickle cell anemia.
But a failure to distribute enough modified genetic information to the patient's body has prohibited gene therapy from being more widely used.

Gene therapy relies mainly on viruses, which transport genomes inside the cells they infect-to deliver genetic material into a patient's cells. The virus-driven delivery tools are called "viral vectors."

Unfortunately, the success rate of viral vectors is uneven. For instance, adenoviruses, a cause for the common cold, and lentiviruses, such as HIV-1, are routinely converted into viral vectors. But adenovirus vectors don't last long, so therapy must be frequently re-administered. And lentiviral vectors, while stable, fail to deliver genetic material to enough defective human cells.

Dr. Arts, a professor of medicine in the Division of Infectious Diseases and HIV Medicine, learned that lentiviral carriers lack sufficient genetic material necessary for treatment.

"Using lentivirus for gene transfer appears to be a safe option," said Stanton L. Gerson, MD, director of the CaseComprehensive Cancer Center and the Asa and Patricia Shiverick- Jane Shiverick (Tripp) Professor of Hematological Oncology at Case Western Reserve School of Medicine and director of the Seidman Cancer Center at University HospitalsCase Medical Center, who is not involved in the study. "This discovery could greatly advance the recent successes

ongoing in cancer and childhood congenital diseases. Improvements in the technology of gene delivery identified by Arts and his colleagues could lead to many more effective studies that help patients with many different diseases, including cancer. Its impact could be felt in a few short years."

Ultimately, introducing GRPE elements into viral vectors could enhance the ease and effectiveness of gene therapy,which typically uses transplanted human stem cells. Enhanced gene therapy and other improvements in targeted cell delivery might eliminate the need for stem cells and allow cells to be administered directly into patients.