ASN LEADING THE FIGHT AGAINST KIDNEY DISEASE

1510 H Street NW • Suite 800 • Washington, DC 20005
p 202.640.4660 • f 202.637.9793 • www.asn-online.org

Press Release

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Contacts: **Tracy Hampton • (312) 339-9067 • thampton@nasw.org**
Christine Feheley • (202) 640-4638 • cfeheley@asn-online.org

RESEARCHERS DEVELOP GENE THERAPY METHOD TO TARGET KIDNEY CELLS

Findings could lead to improved treatments for kidney diseases.

Highlights

- A synthetic adeno-associated virus was highly efficient at delivering genetic material to different kidney cell types in mice and humans.
- The viral vector was also successfully used in gene therapy strategies to treat mice with kidney scarring.

Washington, DC (July 5, 2018) —Scientists have successfully used viral vectors to deliver genetic material to kidney cells. The findings, which appear in an upcoming issue of the *Journal of the American Society of Nephrology* (*JASN*), could have important implications for the treatment of chronic kidney disease (CKD).

Almost 50 years after it was first proposed, gene therapy is now entering into regular clinical use. The most common method uses adeno-associated virus (AAV) to deliver genetic material to target cells, but no AAV has been capable of doing so in the kidney.

Now a team led by Benjamin D. Humphreys MD, PhD (Washington University in St. Louis) reports the first examination of AAV subtypes for delivering and transferring (or "transducing") genetic material to the kidney. Of 6 AAVs tested, a synthetic AAV called Anc80 showed specific and high efficiency for transducing different kidney cell types in both mice and humans. Anc880 was also successfully used in gene therapy strategies to treat mice with kidney scarring.

The findings may be useful in therapeutic approaches to treat CKD, a condition that affects10% of the population worldwide. "CKD is an enormous problem and there has been little innovation and no significant therapies in decades. Our discovery shows that genetic material can be delivered to kidney cells that play a key role in CKD," said Dr. Humphreys. "Future gene therapy approaches could deliver genes that slow or reverse the growth of these cells in CKD."

Study co-authors include Yoichiro Ikeda, MD, PhD; Zhao Sun, PhD; Xiao Ru, MD; and Luk Vandenberghe, PhD.

Disclosures Dr. Vandenberghe is an inventor on several technologies licensed to pharmaceutical and biotechnology companies including Anc80 technology, which was licensed to Akouos, Vivet Therapeutics, Lonza Houston, and Selecta Biosciences. He receives research funding from and consults for Lonza Houston, Solid and Selecta Biosciences. He is also cofounder and equity holder of GenSight Biologics and Akouos, and he is a member of the Scientific Advisory Board of GenSight Biologics, Akouos, and NightStarX. For all other authors, no competing financial interests exist.

The article, entitled “Efficient Gene Transfer to Kidney Mesenchymal Cells Using a Synthetic Adeno-Associated Viral Vector,” will appear online at http://jasn.asnjournals.org/ on July 5, 2018, doi: 10.2215/ASN.2018004026.

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Tweet: Researchers develop gene therapy method to target kidney cells.
Twitter: @HumphreysLab

Facebook: Scientists have successfully used viral vectors to deliver genetic material to kidney cells. The findings, which appear in the *Journal of the American Society of Nephrology*, could have important implications for the treatment of chronic kidney disease.

Image: The synthetic AAV Anc80 caused expression of green fluorescent protein in interstitial pericytes and perivascular fibroblasts (which are red in this photo). Photo Credit: Yoichiro Ikeda MD, PhD

Media:
Caroline Arbanas, Elizabethe Durando:

arbanasc@wustl.edu
elizabethe.durando@wustl.edu
also
Suzanne_Day@MEEI.HARVARD.EDU