[Billing Code 4140-01-P]

DEPARTMENT OF HEALTH AND HUMAN SERVICES

National Institutes of Health

Government-Owned Invention; Availability for Licensing

AGENCY: National Institutes of Health

ACTION: Notice

SUMMARY: The invention listed below is owned by an agency of the U.S. Government and is available for licensing in the U.S. in accordance with 35 U.S.C. 209 and 37 CFR Part 404 to achieve expeditious commercialization of results of federally-funded research and development.

FOR FURTHER INFORMATION CONTACT: Licensing information may be obtained by emailing the indicated licensing contact at the National Heart, Lung, and Blood, Office of Technology Transfer and Development Office of Technology Transfer, 31 Center Drive Room 4A29, MSC 2479, Bethesda, MD 20892-2479; telephone: 301-402-5579. A signed Confidential Disclosure Agreement may be required to receive any unpublished information.

SUPPLEMENTARY INFORMATION: Technology description follows.

Capsid-Free AAV Vectors for Gene Delivery and Their Use for Gene Therapy

Description of Technology: The invention concerns novel capsid-free AAV vectors that can be used for gene delivery and gene therapy applications. The invention provides for a linear nucleic acid molecule comprising in this order: a first adeno-associated virus (AAV) inverted terminal repeat (ITR), a nucleotide sequence of interest, and a second AAV ITR, wherein said nucleic acid molecule is devoid of AAV capsid protein coding sequences. The said nucleic acid molecule can be applied to a host at repetition without eliciting an immune response. Methods of producing and purifying this nucleic acid molecule, as well as its use for gene transfer and gene therapy are also described.

Potential Commercial Applications: The commercial applications of the technology relate to the field of gene therapy. It may offer significant advantages compared to existing methods of gene delivery and gene therapy.

Competitive Advantages:

• The AAV vectors described in the invention devoid the AAV capsid proteins and thus are not exposed to the adverse effects caused by immunogenicity.

• In contrast to the use of plasmid DNA for gene delivery, the AAV DNA of the invention seems to confer greater stability in cell nuclei, allowing prolonged expression compared to plasmid DNA.

• The vector DNA of the invention is not limited in size to the packageable size genome.

• The production of the AAV DNA vector is economical, simple and provides high yields.

Development Stage: Early-stage; In vitro data available

Inventors: Drs. Luis Garcia, Cyriaque Beley, and Thomas Voit (INSERM Paris); Drs. Robert M. Kotin and Lina Li (NHLBI)

Publication: Li L, Dimitriadis EK, Yang Y, Li J, Yuan Z, Qiao C, Beley C, Smith RH, Garcia L, Kotin RM. Production and characterization of novel recombinant adeno-associated virus replicative-form genomes: a eukaryotic source of DNA for gene transfer. PLoS One. 2013 Aug 1;8(8):e69879. doi: 10.1371/journal.pone.0069879.

Intellectual Property: NIH Reference No. E-241-2010/0 – US Patent Application No. 14/004,379 (Publication No. 2014-0107186 A), and its foreign counterparts in Europe (11 157986.8; 12 708035.6), Canada (2,829,518), Australia (2012228376), Brazil (BR 1 1 2013 023185 8), China (201280022523.5), Israel (228328), India (8000/DELNP/2013), Japan (2013-557138), and South Korea (10-2013-7026982)

Licensing Contact: Uri Reichman, Ph.D., M.BA.; Phone: 301-435-4616; E-mail: uri.reichman@nih.gov

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Uri Reichman, Ph.D., M.B.A.
Senior Advisor for Licensing
Office of Technology Transfer and Development
National Heart, Lung, and Blood Institute
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