BILLING CODE: 4140-01-P

DEPARTMENT: DEPARTMENT OF HEALTH AND HUMAN SERVICES

National Institutes of Health

Government-Owned Inventions; 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 and/or co-development 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. Foreign patent applications are filed on selected inventions to extend market coverage for companies and may also be available for licensing and/or co-development.

ADDRESSES: Invention Development and Marketing Unit, Technology Transfer Center, National Cancer Institute, 9609 Medical Center Drive, Mail Stop 9702, Rockville, MD, 20850-9702.

FOR FURTHER INFORMATION CONTACT: Information on licensing and co-development research collaborations, and copies of the U.S. patent applications listed below may be obtained by contacting: Attn. Invention Development and Marketing Unit, Technology Transfer Center, National Cancer Institute, 9609 Medical Center Drive, Mail Stop 9702, Rockville, MD, 20850-9702, Tel. 240-276-5515 or email ncitechtransfer@mail.nih.gov. A signed Confidential Disclosure Agreement may be required to receive copies of the patent applications.

SUPPLEMENTARY INFORMATION: Technology description follows.

Title of invention:

AAV-based Vectors for the Therapeutic Management of Menkes Disease and Related Copper Transport Disorders

Description of Technology:

The only currently available treatment for Menkes disease, subcutaneous copper histidinate injections, is successful only in patients with ATP7A gene mutations that do not completely corrupt ATP7A copper transport function (estimated 20-25% of affected patients) and when started at a very early age (first month of life). The combination of viral gene therapy with copper injections provides working copies of the ATP7A copper transporter into the brain, together with a source of the substrate (copper) needed for proper brain growth and clinical neurodevelopment.

Codon-optimized nucleic acids encoding a reduced-size ATP7A protein and compositions of AAV vectors were discovered by NICHD researchers along with methods of administering this therapy. Human P-type ATPase copper-transporting ATPase 1 (ATP7A) transports copper from enterocytes (where it is taken up from dietary copper) into the blood. ATP7A also mediates passage of copper across the blood-cerebrospinal fluid (CSF) barrier and the blood-brain barrier. In Menkes disease and occipital horn syndrome (OHS), copper accumulates in intestinal cells and less copper is absorbed into the blood,

resulting in restricted copper supply to other tissues, particularly the brain. Death in infancy or early childhood is a common consequence. Therapeutic delivery of the copper transport protein via an AAV vector, combined with subcutaneous copper histidinate treatment will relieve the copper deficiency to the brain and permit normal neurological development and function.

Potential Commercial Applications:

- Treatment of Menkes Disease, Occipital Horn Syndrome, and of ATP7A-related distal motor neuropathy

Value Proposition:

- Provides working copies of the ATP7A copper transporter into the brain, together with a source of the substrate (copper) needed for proper brain growth and clinical neurodevelopment.

Development Stage:

Pre-clinical (in vivo validation)

Inventor(s):

Stephen G. Kaler, M.D. (NICHD)

Intellectual Property:

HHS Reference No. E-062-2015/0

US Provisional Application No. 62/244,594 filed 21 October 2015

Licensing Opportunity: Researchers at the NICHD seek licensing and/or co-development research collaborations for the therapeutic management of Menkes Disease and related copper transport disorders..

Contact Information:

Requests for copies of the patent application or inquiries about licensing, research collaborations, and co-development opportunities should be sent to John D. Hewes, Ph.D., email: john.hewes@nih.gov.

Dated: February 8, 2016.

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John D. Hewes

Technology Transfer Specialist, Technology Transfer Center, National Cancer Institute

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