

## DEPARTMENT OF HEALTH AND HUMAN SERVICES

### National Institutes of Health

#### Center for Scientific Review; Notice of Closed Meetings

Pursuant to section 10(d) of the Federal Advisory Committee Act, as amended (5 U.S.C. App.), notice is hereby given of the following meetings.

The meetings will be closed to the public in accordance with the provisions set forth in sections 552b(c)(4) and 552b(c)(6), Title 5 U.S.C., as amended. The grant applications and the discussions could disclose confidential trade secrets or commercial property such as patentable material, and personal information concerning individuals associated with the grant applications, the disclosure of which would constitute a clearly unwarranted invasion of personal privacy.

*Name of Committee:* AIDS and Related Research Integrated Review Group, AIDS Discovery and Development of Therapeutics Study Section.

*Date:* March 8, 2016.

*Time:* 8:00 a.m. to 6:00 p.m.

*Agenda:* To review and evaluate grant applications.

*Place:* Residence Inn Bethesda, 7335 Wisconsin Avenue, Bethesda, MD 20814.

*Contact Person:* Shiv A Prasad, Ph.D., Scientific Review Officer, Center for Scientific Review, National Institutes of Health, 6701 Rockledge Drive, Room 5220, MSC 7852, Bethesda, MD 20892, 301-443-5779, [prasads@csr.nih.gov](mailto:prasads@csr.nih.gov).

*Name of Committee:* Center for Scientific Review Special Emphasis Panel, Infectious Diseases and Microbiology.

*Date:* March 8, 2016.

*Time:* 9:30 a.m. to 6:00 p.m.

*Agenda:* To review and evaluate grant applications.

*Place:* National Institutes of Health, 6701 Rockledge Drive, Bethesda, MD 20892 (Virtual Meeting).

*Contact Person:* Guangyong Ji, Ph.D., Scientific Review Officer, Center for Scientific Review, National Institutes of Health, 6701 Rockledge Drive, Room 3211, MSC 7808, Bethesda, MD 20892, 301-435-1146, [jig@csr.nih.gov](mailto:jig@csr.nih.gov).

*Name of Committee:* Center for Scientific Review Special Emphasis Panel, AREA application in Infectious Diseases and Microbiology.

*Date:* March 8, 2016.

*Time:* 9:30 a.m. to 6:00 p.m.

*Agenda:* To review and evaluate grant applications.

*Place:* National Institutes of Health, 6701 Rockledge Drive, Bethesda, MD 20892, (Virtual Meeting).

*Contact Person:* Liangbiao Zheng, Ph.D., Scientific Review Officer, Center for Scientific Review, National Institutes of Health, 6701 Rockledge Drive, Room 3202, MSC 7808, Bethesda, MD 20892, 301-996-5819, [zhengli@csr.nih.gov](mailto:zhengli@csr.nih.gov).

(Catalogue of Federal Domestic Assistance Program Nos. 93.306, Comparative Medicine; 93.333, Clinical Research, 93.306, 93.333, 93.337, 93.393-93.396, 93.837-93.844, 93.846-93.878, 93.892, 93.893, National Institutes of Health, HHS)

Dated: February 9, 2016.

**Sylvia Neal,**

*Program Analyst, Office of Federal Advisory Committee Policy.*

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## DEPARTMENT OF HEALTH AND HUMAN SERVICES

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#### Government-Owned Inventions; Availability for Licensing

**AGENCY:** National Institutes of Health, HHS.

**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](mailto: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  
U.S. Provisional Application No. 62/244,594 filed 21 October 2015