

licensing 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. Technology description follows.

### **Lentiviral Protein Delivery System for RNA-Guided Genome Editing**

**Description of Technology:** This invention provides an HIV-1-based lentiviral vector system for gene correction strategies involving a homologous recombination with a variation of the CRISPR/Cas9 system. Such systems are being explored as potential therapies for certain hereditary diseases. This system comprises (a) a lentivirus vector particle comprising a lentiviral genome which encodes at least one guide RNA sequence that is complementary to a first DNA sequence in a host cell genome, (b) a Cas9 protein, and optionally (c) a donor nucleic acid molecule comprising a second DNA sequence. In addition, the invention provides a host cell comprising the foregoing system, as well as a method of altering a DNA sequence in a host cell comprising contacting a host cell with the foregoing system. Alternatively, the invention also provides a fusion protein comprising a Cas9 protein and a cyclophilin A (CypA) protein, wherein the fusion protein binds to the lentivirus vector particle, as well as a lentiviral vector particle comprising such a fusion protein. Other such lentivirus-based vectors encode a guide RNA, which contains a specific sequence that recognizes a target gene, and a Cas9 endonuclease, which cuts at the specific site. However, such systems present some problems due to constitutive expression of Cas9 endonuclease in lentiviral vector-transduced cells and the large size of the Cas9 gene. The variation of this invention delivers the Cas9 endonuclease directly, instead of the gene encoding the protein.

**Potential Commercial Applications:** Clinical trials for hereditary diseases such as sickle-cell disease and beta-thalassemia are good market opportunities. Gene correction using the disclosed lentiviral vector system are being tested with respect to the beta-globin gene and the BCL11A gene to treat sickle-cell disease and will be used for induced pluripotent stem cell (iPS) generation.

**Development Stage:** Early-stage. In vitro data in cell-line models available.

**Inventors:** Naoya Uchida, Juan J. Haro Mora and John F. Tisdale (NHLBI).

**Intellectual Property:** US Application No. 62/236,223, filed October 2, 2015 and PCT/US2016/054759, filed

September 30, 2016, (NIH Reference No. E-165-2015/0.1).

**Publications:** Lentiviral protein delivery system for RNA-guided genome editing, PCT Publication No. WO/2017/059241, published April 6, 2017.

**Licensing Contact:** Cristina Thalhammer-Reyero, Ph.D., M.B.A.; 301-435-4507; [thalhamc@mail.nih.gov](mailto:thalhamc@mail.nih.gov).

**Collaborative Research Opportunity:** The National Heart, Lung and Blood Institute is seeking statements of capability or interest from parties interested in collaborative research to further develop, evaluate or commercialize this technology. For collaboration opportunities, please contact Denise Crooks at [crooksd@mail.nih.gov](mailto:crooksd@mail.nih.gov).

Dated: March 22, 2018.

**Cristina Thalhammer-Reyero**, Senior Licensing and Patenting Manager, Office of Technology Transfer and Development, National Heart, Lung, and Blood Institute.

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**BILLING CODE 4140-01-P**

## **DEPARTMENT OF HEALTH AND HUMAN SERVICES**

### **National Institutes of Health**

#### **Eunice Kennedy Shriver National Institute of Child Health and Human Development; Notice of Meeting**

Pursuant to section 10(d) of the Federal Advisory Committee Act, as amended, notice is hereby given of a meeting of the Board of Scientific Counselors, NICHD.

The meeting will be open to the public as indicated below, with the attendance limited to space available. Individuals who plan to attend and need special assistance, such as sign language interpretation or other reasonable accommodations, should notify the Contact Person listed below in advance of the meeting.

The meeting will be closed to the public as indicated below in accordance with the provisions set forth in section 552b(c)(6), Title 5 U.S.C., as amended for the review, discussion, and evaluation of individual intramural programs and projects conducted by the Eunice Kennedy Shriver National Institute Of Child Health And Human Development, including consideration of personnel qualifications and performance, and the competence of individual investigators, the disclosure of which would constitute a clearly unwarranted invasion of personal privacy.

**Name of Committee:** Board of Scientific Counselors, NICHD.

**Date:** June 1, 2018.

**Open:** 8:00 a.m. to 11:45 a.m.

**Agenda:** A report by the Scientific Director, NICHD, on the status of the NICHD Division of Intramural Research; talks by various intramural scientists, and current organizational structure.

**Place:** National Institutes of Health, Building 31A, Conference Room 2A48, 31 Center Drive, Bethesda, MD 20892.

**Closed:** 11:45 a.m. to 4:00 p.m.

**Agenda:** To review and evaluate personal qualifications and performance, and competence of individual investigators.

**Place:** National Institutes of Health, Building 31A, Conference Room 2A48, 31 Center Drive, Bethesda, MD 20892.

**Contact Person:** Constantine A. Stratakis, MD, D(med)Sci, Scientific Director, Eunice Kennedy Shriver National Institute of Child Health and Human Development, NIH, Building 31A, Room 2A46, 31 Center Drive, Bethesda, MD 20892, 301-594-5984, [stratak@mail.nih.gov](mailto:stratak@mail.nih.gov).

Information is also available on the Institute's/Center's home page: <https://www.nichd.nih.gov/about/meetings/Pages/index.aspx>, where an agenda and any additional information for the meeting will be posted when available.

(Catalogue of Federal Domestic Assistance Program Nos.93.864, Population Research; 93.865, Research for Mothers and Children; 93.929, Center for Medical Rehabilitation Research; 93.209, Contraception and Infertility Loan Repayment Program, National Institutes of Health, HHS)

Dated: March 23, 2018.

**Michelle Trout**,

Program Analyst, Office of Federal Advisory Committee Policy.

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## **DEPARTMENT OF HEALTH AND HUMAN SERVICES**

### **National Institutes of Health**

#### **Eunice Kennedy Shriver National Institute of Child Health and Human Development; Notice of Meeting**

Pursuant to section 10(a) of the Federal Advisory Committee Act, as amended (5 U.S.C. App.), notice is hereby given of a meeting of the National Advisory Board on Medical Rehabilitation Research. The meeting will be open to the public, with attendance limited to space available. Individuals who plan to attend and need special assistance, such as sign language interpretation or other reasonable accommodations, should notify the Contact Person listed below in advance of the meeting.

**Name of Committee:** National Advisory Board on Medical Rehabilitation Research.