



Billing Code 4140-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

National Institutes of Health

Prospective Grant of an Exclusive Patent License: Gene Therapy for Treatment or Prevention of Niemann-Pick Disease Type C1, Subject to Existing Three Non-Exclusive Licenses

AGENCY: National Institutes of Health, Health and Human Services (HHS).

ACTION: Notice.

SUMMARY: The National Human Genome Research Institute is contemplating the grant of an Exclusive Patent License to practice the inventions embodied in the United States, European and Canadian Applications listed in the **SUPPLEMENTARY INFORMATION** section of this notice to AveXis, Inc., located in Bannockburn, Illinois, USA.

DATES: Only written comments and/or applications for a license which are received by the National Human Genome Research Institute's Technology Transfer Office on or before [INSERT DATE 15 DAYS AFTER DATE OF PUBLICATION IN THE FEDERAL REGISTER] will be considered.

ADDRESSES: Requests for copies of the patent application, inquiries, and comments relating to the contemplated Exclusive Patent License should be directed to: Anna Solowiej, Ph.D., J.D., Senior Licensing and Patenting Manager, NHGRI Technology Transfer Office, 6700B Rockledge Drive, Suite 3100, Bethesda, MD 20817 (for business mail); Telephone (301)435-7791; E-mail: anna.solowiej@nih.gov.

SUPPLEMENTARY INFORMATION:

Intellectual Property

Group A, HHS Reference No.: E-185-2014-0: “Viral Gene Therapy as Treatment for Cholesterol Storage Disease or Disorder”

- I. U.S. Provisional Application 62/144,702, filed April 8, 2015, expired (HHS Reference No.: E-185-2014-0-US-01).
- II. PCT Application PCT/US2016/026524, filed April 7, 2016, expired (HHS Reference No.: E-185-2014-0-PCT-02).
- III. U.S. Application 15/565,065, filed October 6, 2017 (HHS Reference No.: E-185-2014-0-US-04).
- IV. Canadian Application 2,982,129, filed October 6, 2017 (HHS Reference No.: E-185-2014-0-CA-05).
- V. European Application 16717228.7 filed November 8, 2017, issued as EP 3280451, validated in Italy, Spain, France, UK, and Germany (HHS Reference No.: E-185-2014-0-EP-06).

Group B, HHS Reference No.: E-100-2017-0: “Codon-Optimized Human NPC1 Genes for the Treatment of Niemann-Pick Type C1 Deficiency and Related Conditions”

- I. U.S. Provisional Application U.S. 62/522,677, filed June 20, 2017, expired (HHS Reference No.: E-100-2017-0-US-01).
- II. PCT Application PCT/US2018/038584, filed June 20, 2018, expired (HHS

Reference No.: E-100-2017-0-PCT-02).

III. U.S. Application U.S. 16/623,863 filed December 19, 2019 (HHS Reference No.: E-100-2017-0-US-05).

IV. Canadian Application 3,068,010, filed December 19, 2019 (E-100-2017-0-CA-03).

V. European Application 18740403.3 filed January 20, 2020 (HHS Reference No.: E-100-2017-0-EP-04).

The patent rights in these inventions have been assigned or exclusively licensed to the Government of the United States of America.

The prospective exclusive license territory may be worldwide and in fields of use that may be limited to manufacture and commercialization of pharmaceutical products for the treatment and/or prevention of Niemann Pick disease, Type C1 (NPC1) using gene therapy in humans that incorporate the Licensed Product(s), in combination with AAV9, subject to three existing non-exclusive licenses for this technology.

Above listed patent portfolio cover inventions directed to gene therapy and specifically, expression vectors and therapeutic methods of using such vectors in the treatment of Niemann-Pick Disease Type C1.

This notice is made in accordance with 35 U.S.C. 209 and 37 CFR part 404. The prospective exclusive license will be royalty bearing. The prospective exclusive license may be granted unless within fifteen (15) days from the date of this published notice, the National Human Genome Research Institute receives written evidence and argument that establishes that the grant of the license would not be consistent with the requirements of 35 U.S.C. 209 and 37 CFR part 404.

In response to this notice, the public may file comments or objections. Comments and objections, other than those in the form of a license application, will not be treated confidentially, and may be made publicly available.

License applications submitted in response to this notice will be presumed to contain business confidential information and any release of information in these license applications will be made only as required and upon a request under the Freedom of Information Act, 5 U.S.C. 552.

Dated: July 7, 2020

Claire T. Driscoll,

Director,

Technology Transfer Office,

National Human Genome Research Institute.

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