[Billing Code 4140-01-P]

DEPARTMENT OF HEALTH AND HUMAN SERVICES

National Institutes of Health

Prospective Grant of Exclusive Patent License: Development of Adeno-Associated Virus Vectors for the Treatment of Glycogen Storage Disease Type Ia.

AGENCY: National Institutes of Health, HHS.

ACTION: Notice.

SUMMARY: This notice, in accordance with 35 U.S.C. 209(c)(1) and 37 CFR 404.7(a)(1)(i), that the National Institutes of Health, Department of Health and Human Services (HHS), is contemplating the grant of an exclusive license to practice the inventions embodied in the following Patent Applications to Dimension Therapeutics, Inc. ("Dimension") located in Cambridge, Massachusetts, USA:

Intellectual Property

United States Provisional Patent Application No. 61/908,861, filed November 26, 2013, titled "Adeno-Associated Virus Vectors for the Treatment of Glycogen Storage Disease" [HHS Reference No. E-552-2013/0-US-01]; International Patent Application No. PCT/US2014/067415 filed November 25, 2014 titled "Adeno-Associated Virus Vectors for the Treatment of Glycogen Storage Disease" [HHS Reference No. E-552-2013/0-

PCT-02] and continuation applications, divisional applications and foreign counterpart applications claiming priority to the US provisional application No. 61/908,861.

With respect to persons who have an obligation to assign their right, title and interest to the Government of the United States of America, the patent rights in these inventions have been assigned to the Government of the United States of America.

The prospective exclusive licensed territory may be worldwide and the field of use may be limited to: "Development and commercialization of gene therapy using adeno-associated viral vectors for the treatment of Glycogen Storage Disease Type Ia."

DATES: Only written comments and/or applications for a license which are received by the NIH Office of Technology Transfer on or before [INSERT DATE 15 DAYS FROM DATE OF PUBLICATION OF NOTICE IN THE FEDERAL REGISTER] will be considered.

ADDRESSES: Requests for copies of the patent application, inquiries, comments, and other materials relating to the contemplated exclusive license should be directed to: Surekha Vathyam, Ph.D., Senior Licensing and Patenting Manager, National Cancer Institute Technology Transfer Center, 9609 Medical Center Drive, Rm 1E-530 MSC9702, Rockville, MD 20850-9702, E-mail: vathyams@mail.nih.gov.

SUPPLEMENTARY INFORMATION: The subject technologies disclose novel adeno-associated virus (AAV) vectors expressing human glucose-6-phosphatase-alpha (G6Pase-alpha or G6PC) for the treatment of glycogen storage disease, particularly glycogen storage disease type Ia (GSD-Ia). GSD-Ia is an inherited disorder of metabolism associated with life-threatening hypoglycemia, hepatic malignancy, and renal failure caused by the deficiency of G6Pase-alpha, a key enzyme in maintaining blood glucose homeostasis between meals. The two novel gene therapy vectors of the invention, rAAV-GPE-G6PC and rAAV-GPE-co-G6PC are recombinant AAV vectors expressing wild-type G6Pase-alpha and codon-optimized (co) G6Pase-alpha, respectively. G6Pase-alpha in both vectors is directed by nucleotides -2864 to -1 of the G6PC gene 5'-flanking promoter/enhancer region (GPE). The vectors also contain an intron and stuffer sequences. The rAAV-GPE-G6PC vector not only corrects metabolic abnormalities in murine GSD-Ia (G6pc-/- mice) but also prevents long-term risk of hepatocellular adenoma. The results also showed that the enhancer elements upstream the human G6PC minimal promoter at nucleotides -382 to -1 contained within the rAAV-GPE-G6PC vector are responsible for the increased efficacy in treating GSD-Ia mice.

The prospective exclusive license will be royalty bearing and will comply with the terms and conditions of 35 U.S.C. 209 and 37 CFR 404.7. The prospective exclusive license may be granted unless within fifteen (15) days from the date of this published notice, the NIH 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 404.7.

Complete applications for a license in the prospective field of use that are filed in response to this notice will be treated as objections to the grant of the contemplated Exclusive Patent License Agreement. Comments and objections submitted to this notice will not be made available for public inspection and, to the extent permitted by law, will not be released under the *Freedom of Information Act*, 5 U.S.C. 552.

Dated: May 17, 2016.

Richard U. Rodriguez

Associate Director, NCI

National Institutes of Health

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