



[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 part 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. 62/096,400, filed December 23, 2014, titled “Adeno-Associated Virus Vectors Encoding G6PC and Uses Thereof” [HHS Reference No. E-039-2015/0-US-01]; International Patent Application No. PCT/US2015/067338 filed December 22, 2015 titled “Adeno-Associated Virus Vectors Encoding G6PC and Uses Thereof” [HHS Reference No. E-039-2015/0-PCT-02]; and all

continuation applications, divisional applications and foreign counterpart applications claiming priority to the US provisional application No. 62/096,400.

The patent rights in these inventions have been assigned and/or exclusively licensed 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.

ADDRESS: 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 technology discloses novel adeno-associated virus (AAV) vectors expressing human G6Pase-alpha (or G6PC) for the

treatment of glycogen storage disease, particularly 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. These new recombinant AAV vectors that express human G6Pase-alpha directed by the tissue-specific human G6PC promoter/enhancer at nucleotides -2864 to -1 incorporate the following improvements: 1) one expresses a variant of G6Pase-alpha with enhanced enzymatic activity; 2) the other expresses a codon-optimized variant of G6Pase-alpha with higher enzyme expression levels and enhanced enzymatic activity.

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 part 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 part 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.

Date: May 19, 2016.

Richard U. Rodriguez

Associate Director, NCI

National Institutes of Health

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