



[Billing Code 4140-01-P]

DEPARTMENT OF HEALTH AND HUMAN SERVICES

National Institutes of Health

Prospective Grant of Exclusive Patent License: Mutant IDH1 Inhibitors Useful for Treating Cancer

AGENCY: National Institutes of Health

ACTION: Notice

SUMMARY: National Center for Advancing Translational Sciences, an institute of the National Institutes of Health, Department of Health and Human Services, is contemplating the grant of an exclusive patent license to practice the inventions embodied in the Patent Applications listed in the Summary Information section of this notice to GeneXion Oncology, Inc., located in New York, NY.

DATES: Only written comments and/or applications for a license which are received by the National Center for Advancing Translational Sciences on or before [INSERT DATE 30 DAYS FROM DATE OF PUBLICATION OF NOTICE IN THE FEDERAL REGISTER] will be considered.

ADDRESSES: Requests for copies of the patent application, inquiries, and comments relating to the contemplated exclusive license should be directed to: Sury Vepa, Ph.D., J.D., Senior Licensing and Patenting Manager, National Center for Advancing Translational Sciences, NIH, 9800 Medical Center Drive, Rockville, MD 20850, Phone: 301-217-9197, Fax: 301-217-5736, or email sury.vepa@nih.gov. A signed Confidential Disclosure Agreement may be required to receive copies of the patent applications.

LICENSING AVAILABILITY: The Development of mutant Isocitrate Dehydrogenase 1 (mIDH1) inhibitors for the Treatment of Human Cancers

CATEGORY: Routine

ACTION NEEDED BY: There is no specific date that this needs to be approved by, but the sooner the document is approved, the sooner NIH can make a potential therapeutic available to the public.

SUMMARY: Administration of an inhibitor of mIDH1 can potentially treat cancers resulting from or characterized by the presence of mIDH1. Industrial partners are being sought for licensing and to help further develop this technology for use in humans. There are currently few effective therapeutics to treat resulting from aberrant activity of mIDH1, such as acute myeloid leukemia.

JUSTIFICATION: Although there is no specific date requirement, rapid approval is requested in order to make a potential therapeutic available to the public quickly.

SUPPLEMENTARY INFORMATION:

Intellectual Property

1. International Application No. PCT/US15/067406 filed on 12/22/2015 which is entitled “Mutant IDH1 Inhibitors Useful for Treating Cancer” (HHS Ref. No: E-243-2014/0-PCT-02), and
2. U.S. Provisional Application No. 62/353298 filed on 06/22/2016 which is entitled “Mutant IDH1 Inhibitors Useful for Treating Cancer” (HHS Ref. No. E-189-2016/0-US-01)

The patent rights in these inventions have been assigned and/or exclusively licensed to the government of the United States of America and the University of North Carolina at Chapel Hill.

The prospective exclusive license territory may be worldwide and the field of use may be limited to the use of Licensed Patent Rights for the following: “Therapeutics for cancers in humans which result from or characterized by the presence of mutant IDH1.”

The inventions relate to a series of novel compounds that potently and selectively inhibit mIDH1. These compounds reduce 2-HG levels in cell lines in vitro as well as in human cancer cells grown in mouse xenografts in vivo. These compounds show greater than 250-fold selectivity for the mutant enzyme over the wild-type, show favorable in vitro stability (in mouse, rat, dog and human hepatocyte exposure studies), are AMES negative, and exhibit no significant metabolic CYP liabilities. These compounds possess very favorable in vivo rodent pharmacokinetics and bioavailability and are well tolerated in rodents, even when dosed at high levels.

Thus, the compounds of the subject inventions can be used individually or in combination to develop new therapies to treat diseases which result from mutant IDH1 activity. The diseases caused by mutant IDH1 activity include cancer (e.g., acute myeloid leukemia, glioma, cholangiocarcinoma and potentially other solid tumors) and selected rare diseases, such as Ollier Disease.

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, and the prospective exclusive license may be granted unless within thirty (30) days from the date of this published notice, the National Center for Advancing Translational Sciences 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.

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 25, 2017

Pamela McInnes, D.D.S.

Deputy Director

Office of the Director

National Center for Advancing Translational Sciences

[FR Doc. 2017-11241 Filed: 5/31/2017 8:45 am; Publication Date: 6/1/2017]